NAVIGATING GLOBAL INEQUALITIES IN ACCESS TO ESSENTIAL MEDICINES: THE CASE OF ZOLGENSMA IN TURKEY

İNCİ GİZEM GÜMÜŞ

BOĞAZİÇİ UNIVERSITY

2025

NAVIGATING GLOBAL INEQUALITIES IN ACCESS TO ESSENTIAL MEDICINES: THE CASE OF ZOLGENSMA IN TURKEY

Thesis submitted to the

Institute for Graduate Studies in Social Sciences

in partial fulfillment of the requirements for the degree of

Master of Arts

in

Political Science and International Relations

by

İnci Gizem Gümüş

Boğaziçi University

2025

Navigating Global Inequalities in Access to Essential Medicines:

The Case of Zolgensma in Turkey

The thesis of İnci Gizem Gümüş

has been approved by:

Assoc. Prof. Zühre Aksoy Terzibaşoğlu _____
(Thesis Advisor)

Assoc. Prof. Irmak Ertör _____
(Thesis Co-Advisor)

Prof. Begüm Özkaynak Ortaköylüoğlu _____

Assoc. Prof. Gaye İlhan Demiryol _____

Assoc. Prof. Ayça Akarçay _____
(External Member)

May 2025

DECLARATION OF ORIGINALITY

I, İnci Gizem Gümüş, certify that

- I am the sole author of this thesis and that I have fully acknowledged and documented in my thesis all sources of ideas and words, including digital resources, which have been produced or published by another person or institution;
- this thesis contains no material that has been submitted or accepted for a degree or diploma in any other educational institution;
- this is a true copy of the thesis approved by my advisor and thesis committee at Boğaziçi University, including final revisions required by them.

Signature.....

Date.....

ABSTRACT

Navigating Global Inequalities in Access to Essential Medicines:

The Case of Zolgensma in Turkey

Despite the accelerated pace of pharmaceutical innovation, access to life-saving therapies remains deeply stratified, with availability shaped less by clinical urgency than by the imperatives of market profitability. This study analyzes the inaccessibility of the gene therapy Zolgensma for spinal muscular atrophy (SMA) patients in Turkey as a situated expression of global disparities in pharmaceutical access. It is based on 14 in-depth semi-structured interviews with caregivers, patients, clinicians, legal experts, and NGO representatives, complemented by analysis of secondary literature, policy documents, institutional reports, and public communications. Drawing on world-systems theory and Marxist-feminist critiques of care labor, it examines how exclusion is reproduced across global, national, and intimate scales, and how unpaid caregiving emerges as a structurally essential response to institutional abandonment—a form of "survival labor." By situating Turkey's semi-peripheral position within the global political economy, the study shows how monopolistic pricing, TRIPS-aligned patent protections, and fragmented healthcare provision collectively displace the burden of therapeutic procurement onto families, who are forced to either launch high-stakes crowdfunding campaigns or seek entry into the commercial life-cycle of the therapy via rare early access programs and clinical trials. Together, these findings illustrate how the global distribution of pharmaceuticals within the contemporary capitalist world-system continues to reflect and reinforce the geographies of inequality it claims to overcome.

ÖZET

Yaşamsal İlaçlara Erişimde Küresel Eşitsizlikleri Anlamak: Türkiye'de Zolgensma örneği

Farmasötik yenilikler hızla ilerlerken, hayat kurtarıcı terapilere erişim, klinik aciliyetin ötesinde, piyasa kârlılığı gibi ekonomik gereklilikler tarafından şekillendirilmekte ve bu durum, mevcut eşitsizliklerin sürmesine yol açmaktadır. Bu çalışma, spinal müsküler atrofi (SMA) hastaları için üretilmiş Zolgensma gen terapisinin Türkiye'deki erişilemezliğini, yaşamsal ilaçlara erişimdeki küresel eşitsizliklerin bir yansıması olarak incelemektedir. Çalışma, 14 derinlemesine yarı yapılandırılmış görüşmeye ve ikincil literatür, kurumsal raporlar, ve kamuya yönelik bildiriler gibi kaynakların taranmasına dayanmaktadır. Görüşmeler, SMA hastalarının ebeveynleri ve diğer bakım sağlayıcıları, yetişkin hastalar, klinik uzmanlar, hukuk uzmanları ve STK temsilcileri ile gerçekleştirilmiştir. Dünya-sistemleri teorisi ve Marksist-feminist bakım emeği eleştirilerinden yararlanarak, dışlanmanın küresel, ulusal ve bireysel düzeylerde nasıl yeniden üretildiği ve bu bağlamda ücretsiz bakımın, kurumsal terk edilmelere karşı yapısal olarak gerekli bir "hayatta kalma emeği" biçiminde nasıl ortaya çıktığı analiz edilmiştir. Türkiye'nin küresel siyasi ekonomi içindeki yarı-çevresel konumunu merkeze alan bu çalışma, tekelci fiyatlandırma, TRIPS ile uyumlu patent yasaları ve yetersiz sağlık hizmeti arzının, tedavi tedariki yükünü ailelerin üzerine yüklediğine işaret etmektedir. Bu durum, aileleri ya kitlesel para toplama kampanyaları başlatıp zamanla yarışmaya ya da hastalarını tedavinin ticari yaşam döngüsüne sokmayı göze alarak erken erişim programları ve klinik deneylerde yer aramaya zorlamaktadır. Bu bulgular, çağdaş

kapitalist dünya-sisteminde ilaçların küresel dağılımının, aşmayı iddia ettiği eşitsizlik coğrafyalarını nasıl yansıttığını ve pekiştirdiğini gözler önüne sermektedir.

DEDICATION

To my family,
Every page carries the imprint of your patience, belief, resilience, and the curiosity you nurtured long before I had words for it.

To Utku Önder,
for carving chaos into momentum, rhythm, and light.
I stand ready to do the same for you.

TABLE OF CONTENTS

PREFACE

NOTE ON SENSITIVE CONTENT

This thesis engages with sensitive and emotionally difficult material, including narratives of severe illness, caregiving burdens, and experiences of loss and vulnerability within the SMA community. While every effort has been made to handle these topics with care and respect, some readers may find certain sections distressing or triggering. Readers are advised to approach the material with discretion, particularly where personal or professional experiences intersect with the themes discussed.

CHAPTER 1

INTRODUCTION

In a 2000 speech, Ugandan physician Dr. Peter Mugyenyi presented a compelling critique of the global distribution of HIV medications, articulating the rhetorical question, “Where are the drugs? The drugs are where the disease is not. And where is the disease? The disease is where the drugs are not.” (as cited in The Lancet Global Health, 2021). Twenty-five years later, Dr. Mugyenyi's critique remains profoundly relevant, not only for critical diseases such as HIV/AIDS, tuberculosis (TB), and malaria, which continue to disproportionately affect impoverished communities in the Global South but also increasingly for rare diseases treated with ultra-expensive medications classified as "orphan" within the post-TRIPS intellectual property (IP) landscape. Today, the TRIPS-based intellectual property regime is widely viewed as among the major determinants of access to medicines (Gopakumar, 2015, pp. 370-371), and this inverse geography of health and medicine remains a defining feature of the global pharmaceutical order. Few domains illustrate this disjuncture more starkly than the emerging field of gene therapy, where medical innovation consistently outpaces the political, economic, and regulatory mechanisms necessary to ensure equitable access to its end-products. Thus, despite rapid advancements in genomic science, the benefits of these breakthroughs are unequally distributed, shaped by structural hierarchies that govern who lives, who waits, and who is left behind (‘t Hoen, 2009).

Indeed, since the advent of the first gene and cell therapies in the 1990s, the field of life sciences has witnessed significant advancements, with key milestones such as the completion of the Human Genome Project, the emergence of next-

generation sequencing (NGS), the refinement of precise gene-editing tools such as CRISPR-Cas9, breakthroughs in viral and non-viral vector design, and innovations in cell therapy techniques. These developments rejuvenated what previously had been a stagnant research and development (R&D) sector (Li et al., 2020; Özkan, 2022). Remarkably, as of July 2024, 31 gene therapies have received global approval for clinical use. Over 2,000 therapies are also in various stages of development, with over half in preclinical stages and five nearing preregistration for regulatory approval (American Society of Gene and Cell Therapy, 2024). This substantial expansion in clinical trials and development pipeline is widely seen as just the initial phase of a broader growth trajectory, with projections indicating that the global gene therapy market is expected to grow at a compound annual growth rate of 30.1% between 2023 and 2028 (Market Data Forecast, 2023).

However, progress has yet to effectively translate into broad and equitable patient access. In fact, most gene therapies become the most expensive medication in the world at a certain point in their life cycle, only to be overthrown by the next one. For instance, Yescarta (axicabtagene ciloleucel), a CAR T cell therapy for large B cell lymphoma[1], costs over $400,000 per dose, while Libmeldy (atidarsagene autotemcel), a gene therapy for children with metachromatic leukodystrophy[2], exceeds $4 million per dose. Similarly, Zynteglo (betibeglogene autotemcel), a gene therapy for β-thalassemia[3], is priced at $1.8 million, and Hemgenix (etranacogene dezaparvovec), a gene therapy for hemophilia[4], is estimated at $3.5 million (Doxzen et al., 2024).

1 Highly aggressive subtype of non-Hodgkin lymphoma with rapid proliferation of malignant B cells.
2 Rare genetic disorder causing progressive nervous system degeneration due to sulfatide accumulation.
3 Inherited blood disorder causing reduced hemoglobin production and chronic anemia.
4 Genetic bleeding disorder marked by deficient blood clotting.

These dynamics manifest with particular clarity in the case of Zolgensma, a gene therapy developed for spinal muscular atrophy (SMA)[5], a severe genetic neuromuscular disorder, the most critical forms of which present in early childhood. Developed by Novartis (formerly AveXis) and approved in 2020 by the European Medicines Agency, Zolgensma is among the most expensive medications in the world (Nuijten, 2022), with a 2024 listed price of USD 2.95 million per dose (Novartis, 2024, p. 7). While the therapy has generated substantial clinical interest and public attention for its potential to significantly alter the disease course when administered early, its price tag has rendered it largely inaccessible beyond socioeconomically priviledged populations in advanced economies. In Turkey, where the prevalence, incidence, and carrier frequency of SMA are comparatively high (Özdemir et al., 2022; Ünver et al., 2024). Zolgensma remains neither publicly reimbursed nor formally approved for use, with government officials and reports consistently citing insufficient clinical evidence to support the efficacy and safety of the therapy (Republic of Turkey Ministry of Health, 2023b). As a result, affected families are often forced to navigate precarious, time-intensive, and resource-demanding informal pathways, including public fundraising campaigns, social media mobilization, and competition for limited opportunities to access the therapy abroad through clinical trials and early access programs. These routes unfold against the backdrop of a severe progressive condition, where each delay compounds the emotional, financial, and clinical stakes involved. These conditions raise urgent questions not only about the availability of life-saving therapies, but also about the structural configurations through which access is allocated, restricted, and contested within the uneven and hierarchical architectures of the contemporary capitalist

5 Genetic neuromuscular disorder causing progressive motor neuron loss and severe muscle weakness.

world-system. Against this backdrop, this thesis asks: How do the barriers to accessing Zolgensma in Turkey manifest in the lived experiences of the SMA community, and in what ways do they reflect broader structural disparities in pharmaceutical access shaped by global economic hierarchies and the TRIPS-based intellectual property regime?

This question is approached through an integrated, multi-scalar analysis that traces how and why structural inequities in pharmaceutical access are produced at the global level through regimes of capital and intellectual property, mediated at the national level through Turkey's health and regulatory infrastructures, and ultimately negotiated at the intimate level within the embodied, relational, and gendered dynamics of everyday care work. To this end, this thesis employs a dual theoretical framework that integrates Immanuel Wallerstein's world-systems theory with Marxist-feminist critiques of care labor. World-systems theory provides a macro-structural lens through which I will analyze Turkey's semi-peripheral position within the global capitalist economy and the broader geopolitical and economic hierarchies that condition pharmaceutical production, distribution, and access. Meanwhile, Marxist-feminist scholarship on care labor offers an analytic lens for examining how systemic deficits in public health provisioning are absorbed, negotiated, and contested at the household level, particularly through the feminized and frequently undervalued labor of caregiving. Methodologically, this thesis adopts a single-case study design focusing on Turkey, selected for its semi-peripheral status within the global capitalist system, its IP-maximalist intellectual property stance, its comparatively high incidence and prevalence of SMA, and the ongoing controversy surrounding access to Zolgensma. The empirical foundation of the study is built on 14 in-depth semi-structured interviews with caregivers, clinicians, NGO

representatives, patient advocates, and legal experts, and strenghtened by a review of relevant secondary literature, public communications, and policy documents. In line with the research question, this qualitative approach enables a situated, multi-scalar analysis that captures how barriers to access are produced, experienced, and contested across global, national, and intimate domains.

Persistent barriers to pharmaceutical access have fueled sustained debate over whether the pricing of Zolgensma and similar gene therapies accurately reflects the true costs of their development or the therapeutic value they claim to deliver (Nuijten, 2022; Sabatini et al., 2022). Industry narratives often justify these high prices by citing the extensive financial investments, long development timelines, and significant risks associated with research and development (R&D), manufacturing, regulatory approval, clinical administration, and long-term safety monitoring (Doxzen et al., 2024). However, the limited size of patient populations, the curative or durable nature of such therapies, and the relatively higher disease prevalence in middle- and lower-income countries have further reduced commercial incentives, as the potential for substantial financial returns is constrained and perceived risks are amplified. As a result, rare diseases like spinal muscular atrophy (SMA) are increasingly subjected to "orphaning" within the industry, as companies attempt to recoup costs through inflated price points and speculative valuations (Doxzen et al., 2024). The consequences of this dynamic are far-reaching, affecting an estimated 300 million individuals worldwide who live with one of the approximately 8,000 known rare diseases (Fehr & Prütz, 2023; Nguengang Wakap et al., 2020).

Today, the term "rare diseases" refers to medical conditions that affect small and often dispersed populations, while "orphan diseases" denotes a subset that has been particularly marginalized due to historically limited commercial incentives for

pharmaceutical investment. “Orphan drugs”, in turn, are pharmaceutical products developed to meet these unmet needs, their viability often enabled through regulatory and policy mechanisms that aim to address market failures and foster therapeutic innovation in neglected domains. Orphan drug designation, a regulatory status that offers incentives to promote the development of treatments for rare diseases, is typically conferred upon therapies targeting life-threatening, chronically debilitating, or otherwise severe conditions that are deemed commercially nonviable under the imperatives of capitalist market logics. In response to this perceived market failure, regulatory agencies in high-income countries have introduced a suite of incentive structures designed to stimulate innovation in this field. These include expedited regulatory pathways such as conditional marketing authorization, breakthrough therapy designation, and accelerated review procedures, as well as economic inducements such as tax credits, direct research grants, and exemptions from regulatory fees (Gammie et al., 2015; Luzzatto et al., 2018). Building upon the protections established by the Agreement on Trade-Related Aspects of Intellectual Property Rights (TRIPS), many jurisdictions have also implemented stringent intellectual property provisions for orphan-designated drugs, including extended periods of market exclusivity and patent term extensions, in order to ensure that pharmaceutical companies can extract maximum commercial value from their products over an extended timeframe.

Despite the introduction of novel incentives, the expanding prevalence of gene therapies, and the growing reliance on patient data and biological materials to monitor their safety and efficacy, access to these treatments remains profoundly limited for patients in developing and less developed countries, in resource-constrained settings within advanced economies, and among the global poor (Özkan,

2022, p. 2). Indeed, of the 31 approved gene therapies worldwide, only 5 were granted approval in countries defined as low- or middle-income by the World Bank (Market Data Forecast, 2023). Even when approved, these medications cannot be afforded without complete or substantial reimbursement by public or private insurance providers, which places significant financial burdens on the health systems of less developed and developing countries, often leaving communities with urgent needs for these medications pressing for access for years (Adachi et al., 2023; Lopez Gousset & Bolz-Johnson, 2021).

While clinical trials offer an opportunity for access for some patients in countries without approved products, patient access to clinical trials remains concentrated in high-income countries. In fact, none of the 1,116 gene therapy clinical trials that were open in April 2024 involved participants from low-income countries, and only 2.9% were recruiting participants in middle-income countries, except China (American Society of Gene and Cell Therapy, 2024). Moreover, even when patients in middle- and low-income countries are able to participate in clinical trials, their participation does not necessarily result in positive outcomes for the approval of the medication in their country. Indeed, a study found that, despite conducting clinical trials involving participants from these countries, 34 new drugs sponsored by large companies received approval in only 2 out of 22 upper-middle-income countries and 2 out of 9 lower-middle-income countries (Miller et al., 2021). Likewise, the global distribution of developers of gene therapies is heavily skewed towards high-income countries, with only 3.4% of all worldwide developers of gene therapy, unmodified cell therapy, and tissue-engineered products being located elsewhere in early 2023 (Alliance for Regenerative Medicine, 2023).

Notwithstanding the overwhelming preponderance of gene therapy research,

development, availability, affordability, and access in high-income countries, 90% of the global disease burden is currently shouldered by lower-income and lower-middle-income countries (Coates et al., 2021). Altogether, a brief overview of the current landscape of access to gene therapies and orphan drugs reveals that access to these life-saving medicines in the structurally marginalized regions of the world-system—often collectively described as the Global South—is intricately linked to, and constrained by, the structural positions assigned to individuals within the global life-cycle of therapeutic innovation. Despite having a higher disease burden, the current access pathways prove either unavailable or insufficient for communities in peripheral and semi-peripheral political-economic contexts, leaving patients with almost no alternative but to participate in clinical trials with very limited quotas to access treatments with uncertain safety and efficacy levels. Moreover, even when clinical data provided by these patients constitutes a critical input for the development and approval of these treatments, their participation rarely translates into tangible benefits for their communities. In centering the case of Zolgensma in Turkey, this thesis examines how access to life-saving therapies is not merely a function of scientific progress or policy design, but is produced, experienced, and contested across intersecting global, national, and intimate levels, reflecting the positionalities of states, institutions, and individuals within an uneven global pharmaceutical landscape. Ultimately, this thesis introduces the concept of *nested peripheries* to conceptualize how structural exclusions in pharmaceutical access in Turkey are reproduced across global, national, and intimate scales, manifested in the country's semi-peripheral position within the world-system, the marginalization of rural provinces, the gendered and undercompensated labor of caregiving, alongside

the biomedical sidelining of rare disease patients as interlocking layers of peripherality.

This thesis is organized into seven chapters. Following this introductory chapter, Chapter 2 sets out the theoretical framework, situating world-systems theory among competing approaches and integrating Marxist-feminist analyses of care labor to frame the analysis of access barriers as shaped by global socioeconomic configurations and negotiated through the situated practices of caregiving within affected households. Chapter 3 provides a comprehensive review of relevant literature, encompassing debates around the TRIPS-based intellectual property regime, the relationship between patents, innovation, and economic development, and the political economy of orphan drugs, culminating in a focused overview of the policy and clinical landscape of SMA care in Turkey. Chapter 4 outlines the research design and methodological approach, detailing the rationale for the single-case study, data collection procedures, and analytic strategy. Chapter 5 delivers the empirical findings, analyzing how barriers to Zolgensma access are produced, navigated, and contested across multiple domains. Chapter 6 engages these findings through the theoretical lens developed earlier, offering a critical discussion of how structural inequalities are refracted through lived experience in a semi-peripheral context. Chapter 7 concludes the thesis by synthesizing the key insights, reflecting on their broader relevance for debates on pharmaceutical access and global inequality, acknowledging the limitations of the study, and suggesting avenues for future research.

CHAPTER 2

THEORETICAL BACKGROUND

2.1 From liberal economic thought to critical perspectives: Theoretical foundations of pharmaceutical inequality

Conventional economic theories of growth and development often portray global capitalism as a natural, inevitable, and essentially irrevocable stage in human history, positioning it as the only viable and the best possible socioeconomic system capable of enduring in the long run (Peet & Hartwick, 2009, p. 21). Prioritizing economic growth over development and often equating the two, such theories commonly posit that the accumulation of wealth by a select few serves as an incentive for entrepreneurship and innovation, ultimately driving economic growth for the benefit of the majority. Socioeconomic inequality, on the other hand, is framed as an unfortunate yet inevitable cost of economic growth, one that can only be mitigated through charitable efforts until the benefits of economic growth eventually "trickle down" to the wider society (Peet & Hartwick, 2009, pp. 2, 21). Today, our production, valuation, distribution, access, and consumption processes derive heavily from the underlying principles of global capitalism and the dominant economic paradigms that prioritize economic growth over development. Among the most evident cases is the political economy of health, where pharmaceutical production, pricing strategies, intellectual property regimes, disease distribution, and access to treatment are shaped and reproduced by mechanisms grounded in purely market-driven economic principles and structural inequalities inherent in the capitalist system.

Concepts derived from conventional economic paradigms continue to dominate the imagination and lexicon of the society, shaping the efforts to critically engage with the current sociopolitical landscape and to conceptualize viable alternatives. Given that mainstream economics is characterized by a highly sophisticated, abstract, scientifically conceived, and politically entrenched theoretical structure, conventional theories of development have been able to assert a claim to virtually neutral and universal truth that has largely remained out of reach for fundamental critiques and democratic input. However, if one traces the trajectory of these theories from the political philosophy of the early modern capitalism, through liberal classical economics, to present-day neoliberalism, critical theories of development such as Marxism, dependency theory, world-systems theory, postcolonial approaches, feminist economics, post-development theory, and indigenous economics emerge as significant counter-narratives against the premises, assumptions, and conclusions of conventional economic paradigms. Such critical approaches stand out by scrutinizing unequal access to essential resources and opportunities to participate in decision-making processes that determine their distribution, as well as the construction of economic knowledge itself (Peet & Hartwick, 2009). It is within this critical framework that world-system theory by Wallerstein (1974/2011, 1974), as adopted in this thesis, finds its relevance in elucidating the structural inequalities inherent in global access to orphan drugs.

The intellectual framework of Enlightenment philosophers and political economists in the 17th- and 18th-century Britain emerged from the confluence of Western scientific rationalism, the evolving material conditions of early capitalism, and the moral imperatives of the Protestant ethic. This transformation marked a departure from the Augustinian Christian views of feudalism, which regarded labor

as punishment for original sin, toward the Protestant belief that labor was a virtuous "calling," the exercise of which is a sign of self-discipline, a legitimate source of wealth, and communal service. With the advent of Protestantism, human acquisitiveness and labor-derived wealth, once regarded as sinful, became redefined as signs adherence to providential order and individual virtue, thereby establishing their valorization as central tenets of societal progress and moral duty underpinning the foundations of modern economics and conventional development theories (Andreski, 2013; Peet & Hartwick, 2009, pp. 24-25; Weber, 1905/2013; Weber, 1978). Moreover, modern economics emerged as a rejection of the ideas, institutions, and policies that upheld the absolutist state and the dominance of monarchical and aristocratic classes in early capitalism, with Enlightenment philosophers, while retaining rationalism, theorizing to empower the rising bourgeoisie against feudalism, the absolutist state, and landed aristocracy in their struggle for control over the economy and the state (Peet & Hartwick, 2009, pp. 25-26).

Disassociating value creation from divine and absolutist influences, Enlightenment philosophers and political economists in 17th- and 18th-century Britain sought to rationalize ideas of competitive individualism, private property, and capital accumulation, aiming to reconcile them with the common good by ensuring the freedom of the self-interested, rational individual to pursue their interests within modern social institutions, notably the markets. Within this context, the political philosophies of Hobbes and Locke helped shape a capitalist ethos that justifies the pursuit of self-interest by a theoretically autonomous, rational, and self-seeking individual. This conception of subjectivity, grounded in fear, avarice, or the instinct for self-preservation (Hobbes, 1651/2008; Smith, 1759/2010), emerged alongside Western rationalism, Protestant ethics, and the material conditions of early

capitalism. By grounding the entitlement of this individual to private property in a principle of appropriation and extending this entitlement to the appropriation of the product of the sellers of labor, the Lockean notion of labor creating property justified not only private property but also unlimited individual property appropriation, unequal property relations, and employer–employee wage relations (Locke, 1689/2005; Macpherson, 1962, p. 221).

The liberal assumption of the self-seeking human nature legitimized not only the pursuit of rational self-interest as the moral code of the new capitalist system, but also the operation of its socioeconomic institutions free from intervention. Indeed, Smith (1776/1937) extends the Lockean concept of natural liberties to include "competition, free movement of workers, free shifts of capital, and freedom from government intervention," operating on the premise that humans are *homo economicus*, rational self-interested agents endowed with a natural "propensity to truck, barter, and exchange" (p. 7), and argues that channeling and actualizing this innate drive within self-regulating markets is the primary mechanism for promoting both economic growth and public interest in modern economic systems. According to Smith (1776/1937), the invisible hand, as an automatic mechanism that organizes competitive trade in self-regulating markets, serves as a dual catalyst for economic growth and relative societal benefit across classes in the long run by (1) optimally allocating productive resources to maximize productivity, profitability, and innovation, while (2) maintaining prices within “socially just” levels, establishing a sustainable socioeconomic balance between wages, rents, prices, and profits, and preventing market monopolies through competitive pressure. Building on Smith (1776/1937), Ricardo's theory of free trade based on competitive advantage posits that the division of labor at the national level should parallel international

specialization, where each country utilizes its productive resources according to its natural or artificial advantages; and through free trade, this specialization would generate universal benefits by optimizing resource distribution, stimulating industry, fostering innovation, and uniting nations around a common interest, with the complexity of specialization, market structures, and trade networks ultimately determining the creation and distribution of wealth both within nations and globally (Ricardo, 1817/2010; Smith, 1776/1937).

Classical economics represents the foundational phase of a loosely coherent liberal ideological continuum, with its more contemporary iterations manifesting in the forms of neoclassical economics, neoliberalism, and modernization theory to the present day. In parallel with the 19th-century scientific revolution, the emergence of neoclassical economic theory marked a shift from "political economy" to "economic science," asserting that within the constraints of consumer preferences, productive technologies, and the mobility of productive factors, the interaction of rational, utility-maximizing producers and consumers optimizes resource allocation, ensuring that all participants receive incomes proportionate to their contributions and justifying capitalism as the most efficient and fair economic system through an objective, neutral, and abstract mathematical framework (Peet & Harwick, 2009, p. 47). The resurgence of liberalism in the late 20th century, marked by the neoliberal ideas advanced by Mises, Hayek, and later Friedman and proponents of the Chicago school, represents the contemporary iteration of the liberal economic continuum, predicated on the argument that, if unfettered by state intervention, self-regulating market forces would equilibrate the interests and expectations of consumers, entrepreneurs, managers, and workers, by making full use of the knowledge and skills of each member of the society, thereby preventing inefficiencies and wastage,

and ensuring the compensation of each “factor of production” according to its input (Hayek, 1966; Mises, 1912/2010; Palley, 2005). State interventionism, beyond managing interest rates and ensuring the conditions for self-regulating markets, is seen as a "distortion that leads to misallocation and inefficiencies" (Tabb, 2004, pp. 335–336), fostering unemployment, underdevelopment, and economic crises at domestic and global scales (Friedman, 1995; Hayek, 1966), whereas the advancement of underdeveloped nations was argued to depend solely on the establishment of a free-market economy, facilitated by the adoption of neoliberal policies designed to promote competitive markets and entrepreneurial innovation (Friedman, 1995). Thus, Sachs (1992) characterizes neoliberalism as aligning with classical liberalism's advocacy for limited state intervention and market dependence, 20th-century liberalism's empathy for the marginalized, yet distinctively "neo" in its rationalization of suffering as an unavoidable byproduct of economic reform and efficiency (Peet & Hartwick, 2009).

In its attempt to reformulate purely economic theories of growth, modernization theory expands its focus to encompass social, cultural, political, psychological, and behavioral dimensions, highlighting factors such as (a) human capacity, occupational and structural specialization, urbanization, mobility, adaptability, democratization, political participation, civic culture, and institutionalization (Almond & Verba, 1963; Parsons, 1951/2005); (b) the cultivation of “the modern persona” embodying qualities such as enlightenment, ambition, social mobility, rationality, adaptability, curiosity, optimism, secularism, entrepreneurship, and innovation (Inkeles & Smith, 1974; Lerner, 1958; McClelland, 1961/1976); and (c) production methods, productivity levels, investments, privatization, deregulation, the division of labor, industrialization, consumption

patterns, market expansion, and capital accumulation (Peet & Harwick, 2009). While ostensibly countering the reductionist and naturalistic assumptions about human attitudes, behaviors, and motivations in classical, neoclassical, and neoliberal economics, modernization theory was established upon two core teleological assumptions, namely that modernity is a single, universal, and homogenizing evolutionary process, and that the Euro-America is the definitive standard against which development is measured, thereby creating a global system divided into centers of modern progress and peripheries of traditional backwardness, with the former bearing the burden of guiding the peripheries towards their anticipated future and the latter having to welcome multinational corporations, encourage imported technology, withdraw state-led developmental efforts, and allow the market to discipline their economies to develop (Peet & Hartwick, 2009).

This theoretical lineage not only highlights the connections among classical economics, neoclassical economics, modernization theory, and neoliberal economics but also elucidates the ways through which this coherent ideological continuum operates under largely shared assumptions and defense mechanisms. On one hand, the proponents of this ideological continuum base their conceptualizations on the assumptions that (a) humans are inherently rational, economically motivated, and utility-maximizing agents; (b) self-regulating markets are spontaneous and neutral mechanisms facilitating optimal resource allocation and collective prosperity; (c) socioeconomic inequality is secondary to and a byproduct of economic growth, which can only be overcome through a spillover effect; and (d) state interventionism is a counterproductive strategy that should be minimized to maintaining the essential conditions for the effective operation of a self-regulating market. On the other hand, they commonly defend their stances through (a) naturalizing human behavior and

institutions in their accounts; (b) claiming class-neutrality through obscuring its ties with the bourgeoisie, colonialism, and influence over the state apparatus, (c) a claim to universal good through linking collective prosperity to trickle-down economics and individual effort; (d) a claim to scientific "truth" through mathematical abstraction; (e) externalizing any inefficiencies attributable to the operation of the market forces to interventionism; and (f) the denigration of alternative institutions or modes of existence as either "backward" or "impossible".

This ideological continuum reinforces the prioritization of individual utility-maximization over collective social responsibility and community welfare, a clear manifestation of which is found in the implementation and influence of mechanisms like TRIPS and other global governance frameworks, which regulate the innovation, finance, and trade of intellectual property, particularly in sectors of critical human concern, such as pharmaceuticals. Through the tightening of intellectual property regimes, whether in the form of the TRIPS implementation, TRIPS-plus provisions, or further trade agreements, knowledge has shifted from existing as a "public good" to becoming a commodity that is produced, valued, sold, and owned by private enterprises, aligning with the imperatives of an increasingly globalized market. Accordingly, the prioritization, production, and distribution of knowledge-intensive products and processes have become subject to the exercise of monopolizing power.

Notably, although the commodification of knowledge is fundamentally informed by neoliberal principles in the contemporary capitalist world-system, its ideological rationalization often also relies on utilitarian principles. Here, the most prominent ideological justification of intellectual property emerges from the extension of Lockean logic of labor creating property, where intellectual work is characterized as a form of labor that, in the absence of adequate reward, individuals

would be inherently disinclined to undertake, much as they would from other types of labor. Intellectual property, emerging from the mixing of intellectual labor with ideas construed as immaterial "commons" in a neo-Platonic sense, is presumed to compensate intellectual laborers for the value they create, secure the incentives needed to sustain innovation, and avert a decline in overall social welfare, thereby justifying its legal protection under a liberal state alongside rights of life and liberty (Hughes, 1988). In fact, their intangible and non-rivalrous nature has been argued to render ideas ideal candidates for commodification through immaterial enclosures, as it fulfills the two "Lockean provisos" perfectly, that is, the limitations of sufficiency and spoilage per se, on the grounds that one individual's use of an idea leaves "enough, and as good" for others and as ideas do not diminish in legitimacy or utility when amassed, given that there is no finite threshold at which one's capacity to employ them effectively becomes inadequate (Drahos, 1996; Hughes, 1988; Perelman, 1998; Richards, 2015/2004). Within this framework, the central contention is that intellectual entrepreneurs lack sufficient incentives to invest their intellectual and material resources in knowledge-producing activities that underpin knowledge-intensive goods and processes, absent the material returns and market control secured by a robust intellectual property regime, thereby prompting them to prioritize alternative sectors they perceive as more secure and lucrative (Drahos, 1996; Gervais, 2015; Richards, 2015/2004). The resulting reallocation of resources undermines the research and development of knowledge-intensive goods and processes, ultimately resulting in a Pareto-inferior outcome[6] for society at large. Even when accounting for the potential welfare losses associated with the monopolization of knowledge-intensive goods and processes, this perspective

6 A situation in which at least one party could be made better off without making anyone else worse off, indicating a suboptimal allocation of resources.

maintains that mitigating these presumed negative effects ultimately yields net benefits for all stakeholders involved (Richards, 2015/2004).

Given their inherently ideological nature, designed to rationalize the political power of advancing groups, the assumptions and propositions of this framework demand critical scrutiny and empirical substantiation to assess their theoretical and practical validity, as (a) the characterization of knowledge producers as "intellectual capitalists" overlooks the fact that exploitation typically occurs post-production (Richards, 2015/2004); (b) profit motivation may stem from structural capitalist realities rather than intrinsic human nature; (c) intellectual property regimes often benefit exploitative entities, not creators; (d) the sufficiency proviso fails to account for how socioeconomic, political, and technological contexts constrain the application of ideas (Drahos, 1996); (e) the spoilage proviso neglects the stifling effects of over-commercialization and monopolization, diminishing the collective benefits of ideas, and (f) such justifications overlook the fact that intellectual outputs are seldom the result of isolated individual efforts. (Richards, 2015/2004).

Indeed, undermining the intangible and non-rivalrous nature of ideas, it is through the deliberate creation of artificial scarcities via intellectual property rights that legally sanctioned profits and monopoly power are derived from the commodification of knowledge. Accordingly, within the contemporary capitalist world-system, it is primarily intellectual property regimes that govern the distribution and value of knowledge, determining who controls it, who has access to it, whose consent is necessary for its use, the duration of such control, and how much value is assigned to it based on market imperatives. In fact, by limiting the dissemination of knowledge, it has been argued that intellectual property rights can give rise to what is known as the “tragedy of the anti-commons”, wherein a resource is underutilized

because multiple owners of "upstream knowledge" possess the right to exclude others from effective "downstream" access and use (Heller, 1998), thereby impeding the broader societal and innovative potential that could emerge from more open access (Richards, 2015/2004). Given that access to a single idea can catalyze further intellectual creation in an almost exponential manner as new ideas build upon one another, prolonged and stringent restrictions on access and use can lead to a profound dichotomy, wherein monopolies over both intellectual and physical capital emerge on one side, while national and global inequalities intensify on the other. Moreover, it should not be taken for granted that providing legal protections to private, profit-driven enterprises, with the intention of incentivizing the production and commercialization of intellectual property and its derivatives, automatically leads to outcomes that address broader human needs, particularly when such needs are inadequately represented in the market due to a lack of purchasing and political power necessary to influence the decisions of these entities (Richards, 2015/2004).

These issues are particularly pronounced in the pharmaceutical sector, where the contemporary magnitude, pace, and diversity of scientific breakthroughs, particularly those related to pharmaceutical innovation and healthcare technologies, are increasingly accompanied by a fervent pursuit to capture their real, potential, and speculative profits, often secured through the strategic extension of intellectual property protections (Richards, 2015/2004). Although the market-based justifications advocate that strict intellectual property rights serve the public interest by increasing the number and scope of commercially available goods, they have been argued to create at least three additional market failures of fundamental importance to human life and well-being in the context of pharmaceutical access, that is, the issues of availability, accessibility, and spending efficiency (Sonderholm, 2010). The issue of

"availability" pertains to the neglect of diseases disproportionately affecting populations in developing countries, such as Chagas disease, malaria, tuberculosis (TB) leishmaniasis, schistosomiasis, and narcolepsy, with the insufficient purchasing power of patients and governments leaving little incentive for global R&D investments in these diseases (Sonderholm, 2010, p. 5; Velásquez, 2010/2013, p. 3). Orphan diseases, including spinal muscular atrophy (SMA), which affect only a small number of people globally, face comparable challenges, as their small market size offers limited financial incentive for pharmaceutical companies to invest in the research, development, and production of pharmaceuticals targeting these conditions. For instance, Pedrique et al. (2013) found that, from 2000 to 2011, a mere four out of 336 new chemical entities (NCEs) were developed for neglected diseases, with only 2,016 out of 148,445 clinical trials registered by December 2011 being dedicated to these diseases.

The issue of "accessibility," on the other hand, arises when the inflated prices of patent-protected drugs exclude a significant portion of potential buyers, even in cases where they would be able to purchase the medicines if priced closer to marginal costs (Sonderholm, 2010, p. 5). In the context of infectious diseases, Pogge (2023) even argues that patents exacerbate the pricing of medicines, deliberately placing them out of reach for less affluent patients, which in turn sustains the prevalence of the targeted diseases and guarantees continued demand for these treatments (p. 200). Indeed, throughout the first five years of the TRIPS Agreement itself, thousands of people died in the developing countries due to the exorbitant price of the antiretroviral (ARV) medicines for the treatment of HIV/AIDS despite their availability in the market, until popular mobilisation and the effective use of TRIPS flexibilities by major developing country governments in the late 1990s

introduced generic versions of ARVs, reducing the cost of treatment from USD 10,000–12,000 per person per year to USD 350 per person per year (Gopakumar, 2015, p. 372). Collectively, these factors illustrate a persistent and critical issue, whereby essential pharmaceuticals, vital for the health and well-being of patients in developing and less-developed countries, as well as the global poor, are systematically withheld, often for prolonged periods, leading to what Moon et al. (2012) call "neglected populations" (p. 1). Consequently, even in instances where intellectual property protections may be effective in addressing the market failure of undersupply, it becomes imperative, as Sell (2007) contends, to question: "Which publics are served?" (p. 43).

Given the complex ethical, economic, and social challenges associated with drug development, access, and pricing emerging at the intersection of public health, private profit, and scientific innovation, the debate surrounding pharmaceutical patents has been shaped by a multitude of alternative and, to various degrees, competing frameworks that have informed policy decisions concerning pharmaceutical access across different contexts. The mainstream utilitarian justifications explained above, which underline the maximization of the overall good, are opposed by frameworks based on the principle of non-abandonment, which instead prioritizes those in dire need. In a similar vein, while the principle of horizontal equity holds that individuals with similar needs should be treated equally, whereas vertical equity recognizes that fairness may demand giving precedence to those with rarer or more severe conditions in drug development, valuation, and pricing decisions. Finally, the debate surrounding pharmaceutical patents presents a tension between welfarism, which focuses on the attainment of individual well-being, and extra-welfarism, which integrates equity considerations into the pursuit of

maximizing well-being, emphasizing the need to balance individual interests with broader social goals (Postma, 2022, p. 2). Finally, alternative incentive mechanisms that "de-link" drug prices from R&D costs have been suggested to overcome the issue of access that emerge in the context of pharmaceutical access, including patent pools, push funding, prize funds that reward pharmaceutical innovation based on health impact, and patent buy-outs ('t Hoen, 2009, xix; Moon et al., 2012, p. 10).

2.2 Introducing World-Systems Theory

Critiques of this liberal orthodoxy have emerged from various intellectual traditions, including the German Historical School, which challenges its reductionist and economistic view of capitalism; Schumpeterian economics, which questions its essentialized postulates and continuous conceptualization of economic change; and Keynesian economics, which opposes its anti-state stance and the overreliance on market forces for interest equilibration. However, alternatives that draw on the perspectives of non-Western, subaltern, and excluded groups and reject the foundational premises of capitalist development itself, recognizing its inherent perpetuation of structural inequalities such as socioeconomic exploitation, cultural erasure, environmental degradation, and epistemological hegemony, have been articulated by Marxism, dependency theory, world-systems theory, and other critical approaches, including post-colonialism, feminism, post-structuralism, post-developmentalism, and ecological economics (Keynes, 1936; Peet & Hartwick, 2009; Schumpeter, 1911/1934). While this review foregrounds critical perspectives on mainstream economic thought, it should be noted that what is broadly referred to here as mainstream economics has, in recent years, evolved toward greater conceptual and methodological pluralism through incorporating internal critiques and

nuanced revisions in response to recognized limitations—developments that are not fully reflected in the scope of this review.

In examining an instance of systemic disparities in global pharmaceutical access, this thesis adopts radically critical theoretical frameworks to call into question the institutional infrastructure inherent to the capitalist global system. Thereby, it aims to reorient the focus towards socioeconomic justice by tackling its conventionally presumed relationship with economic growth, and to actively resist the deductive assumptions that present capitalism and its presumable byproduct, i.e. socioeconomic inequality, as an inevitable, natural, necessary, or redeemable phenomena in the long run. At its core, such adoption marks a conscious effort to simultaneously summon subaltern perspectives from the point of view of socioeconomically, culturally, legally, psycho-socially, and epistemologically oppressed groups, and to contribute to the foregrounding of their lived experiences and urgent needs as a counterpoint to the profit-driven imperatives embedded within the global pharmaceutical regime.

Marxism constitutes the philosophical and theoretical cornerstone for an array of neo-Marxist frameworks that synthesize historical materialism with diverse critical intellectual traditions, with world-systems theory and dependency theory emerging as particularly influential paradigms. World-systems theory and dependency theory share the foundational premise that development in the Global North was predicated on the active underdevelopment of the Global South (Peet & Hartwick, 2009). Indeed, dependency theory, world-systems theory, and their conceptual allies, including post-colonialist and post-structuralist frameworks, illustrate that Euro-American development was rooted in mechanisms of external exploitation, such as brutal conquest, colonial domination, and the systematic

extraction of human labor, natural resources, economic surpluses, and the large ecological transformations they required, from non-Western societies, rather than an internal innovation or a purportedly neutral European "rationality" (Moore, 2010). This process ultimately culminated in the formation of a global geography defined by "a spatial form of dependence", wherein the Euro-American "core" achieved self-sustaining economic growth, while the economies in "the periphery" were subjected to exploitation, rendered dependent, pushed towards meeting demands from the "core" rather than the needs of its own people, and experienced growth only as a derivative reflection of changes within the "core" (Dos Santos, 1970; Galeano, 1973).

Accordingly, Frank (1966) argues that underdevelopment in Third World societies cannot be understood as a primordial condition, nor as a residual effect of archaic institutions or an alleged irrationality intrinsic to these societies. Instead, he posits that underdevelopment was, and continues to be, actively produced through the very historical processes that simultaneously facilitated economic development in First World societies—specifically, "the development of capitalism itself", which transformed preexisting socioeconomic, political, social and cultural systems into mechanisms that served the advancement of its own interests (Frank, 1966, p. 18; 1969). The exploitative relationship between the core and the periphery is underpinned by a chain of surplus transfer, wherein resources are extracted from the exploited regions and classes to the dominant regions and groups across the global, national, and local capitalist systems, thereby generating development for the few and underdevelopment for the many at each point (Frank, 1969, pp. 7–8). Following integration into the spatial expropriation system of capitalism, the periphery has been actively underdeveloped by being prevented from producing to its own merit or to its

full potential, while its surplus was systematically extracted and funneled to the core to be invested in its development (Frank, 1969; 1979).

This historical process has continued in ever new forms, from the advent of the global capitalist system to the present with the center and periphery becoming increasingly polarized. The exact economic mechanisms of surplus extraction were further elucidated by "the theory of unequal exchange", which has grown to encompass the transfer of a wide variety of sources including knowledge, technology, environmental burdens, and culture, in the present day (Amin, 1973/1976; Emmanuel, 1972; Hickel et al. 2022). Dependency theory, world-systems theory, together with other work from the Third World emphasize in different levels the need for social revolution in countries experiencing the "development of underdevelopment", whereby they can withdraw from global capitalism and pursue self-determined economic sovereignty, equitable development, and a restructuring of their social and political systems to meet the needs of their own populations (Peet & Hartwick, 2009, p. 170). Building on the concept of the "development of underdevelopment" (Frank, 1966), Wallerstein (1974) defines underdevelopment as a structural consequence of peripheral integration into the capitalist world-system, rather than as an intrinsic characteristic of Third World economies or a preliminary stage in the transition to modernity. Likewise, world-systems theory adopts the concept of "unequal exchange" to articulate the central mechanism that operates simultaneously across groups, classes, and states, ultimately culminating in the appropriation of surplus from the entire world-economy by core areas.

According to Wallerstein (1974/2011), a world-system is a self-contained socioeconomic entity with boundaries, structures, member groups, and rules of legitimation, sustained through an extensive division of labor and the multiplicity of

cultural and political systems within its bounds. Its operation is similar to that of an organism and is held together by internal tensions between conflicting forces, as each group seeks to remold the system to its advantage, while the system itself evolves over time, exhibiting both stability and change in its structural characteristics at different points throughout its lifespan. The world-economy, a specific type of world-system characterized by a unified division of labor spanning multiple political and cultural systems, has endured as the only social system to survive into the present day in the form of capitalism (Wallerstein, 1974). In fact, its perseverance as a world-economy, rather than evolving into a world-empire, is facilitated by the coexistence of multiple political systems within its bounds, which prevents any single entity from exerting absolute control and instead allows economic forces to operate freely across a transnational arena, thereby structurally securing the flexibility necessary for continuous growth while perpetuating a systematically uneven distribution of its rewards.

According to Wallerstein, the emergence of the capitalist modern world-system in the sixteenth century marks the transition from a non-capitalist global order to a capitalist one, a process during which the configuration and expansion of world trade, characterized by a polarized global division of labor driven by unequal bulk trade linkages, economic cycles, and commodity chains, entrenched a zero-sum game of systemic inequality (Mielants, 2012; Wallerstein, 1974/2011). Expanding from Europe and the New World, this process incorporated an ever-increasing number of geographical zones, ultimately resulting in a world-economy that is capitalistic in its totality, with a systematically disparate distribution of rewards and burdens, and culminating in an increasingly obscured state of "global coloniality" that continues to reproduce what Korzeniewicz and Moran (2009) term a “high

inequality equilibrium" in novel yet enduring forms (Korzeniewicz & Moran, 2009; Mielants, 2012).

Here, Wallerstein introduces the core, periphery, and semi-periphery as three structural positions in which political entities are located according to their integration within the capitalist world-system at that moment in time, resulting in disparities across various dimensions, including life expectancy, standards of living, mechanisms of labor control, modes of production, the complexity of economic activities, the strength of state apparatuses, political regimes, cultural coherence, concentrations of military power, technological innovation, and the production, dissemination, and ownership of knowledge (Mielants, 2012; Wallerstein, 1974/2011; Wallerstein, 1974). Wallerstein (1974/2011) asserts that the initial eligibility for a particular role is often determined by an incidental advantage configured by historical and geographical contingencies that functions as a critical juncture. Once this critical juncture is realized, however, global market forces exacerbate and institutionalize these disparities, rendering them immutable in the short term.

One of the most prominent conceptual novelties made by the world-system theory for the purposes of this thesis is the introduction of the semi-periphery. Wallerstein (1974) theorizes the semi-periphery as a structurally indispensable component within the architecture of the capitalist world-system, positing it as a mediating stratum that occupies an intermediary position between the core and periphery across multiple dimensions, including but not limited to the complexity of economic activities, strength of the state machinery, cultural integrity, concentration of military strength, technological development, and production of knowledge. Operating as a "buffer zone," the semi-periphery serves the critical function of

partially deflecting political pressures that might otherwise be directly channeled from peripheral groups against core states and the institutions that articulate their geopolitical interests, thus contributing to an underlying stability within an otherwise highly polarized world-system. While it partakes in the exploitation of peripheral regions, it remains, in significant ways, subordinate to the influence and dominance of core states. Neither entirely impaired by international norms nor free to set them, the political objectives of semi-peripheral states and social groups are constrained by the fact that they operate outside the core's primary political circuits, where means to global decision-making is centered, thereby rendering them unable to effectively pursue alliances or engage in coalitions that would enable sufficient access to political agency to actualize their aspirations of transcending their marginalization within the capitalist world-system (Arnold & Naseemullah, 2024; Wallerstein, 1974; Wallerstein, 1974/2011). By emphasizing the structural inequalities inherent in the semi-peripheral position, Wallerstein's framework provides a valuable theoretical tool for understanding how countries and regions navigating this intermediary space encounter unique challenges within the contemporary capitalist world-system, which allows this thesis to critically assess how the structural inequalities inherent in the semi-peripheral status of Turkey shape the availability and affordability of as well as the ongoing struggle for access to the orphan drug Zolgensma.

The Wallersteinian introduction of the semi-periphery (Wallerstein, 1974/2011; 2023) is particularly pertinent to this thesis, as the concept has been widely employed to characterize Turkey's position within the capitalist world-system (Akçalı & Korkut, 2015; Alnasseri et al., 2011; Arnold & Naseemullah, 2024; Eren-Vural, 2007a; Moore & Dannreuther, 2009). The country has been often described as being "in the midst of a transitional process... from a peripheral to a core status",

reflecting its efforts to “emerge from a semi-peripheral status and graduate into the ranks of newly industrialized countries (NICs) or the core group of advanced industrialized countries” (Öniş, 1998, p. 473). According to Moore & Dannreuther (2009), the trajectory of the Turkish state, from the Ottoman Empire to the current position of the Turkish Republic in the contemporary capitalist world-system has often been understood as “a period of peripheralisation followed an impressive story of upward mobility culminating now in the civilising process of EU accession” (p. 138). From an economic standpoint, while the shifting political and economic strategies have influenced its pattern of production and trade across various points in its history, the commodity structure of trade in Turkey has commonly been argued to align with the characteristics of semi-peripherality, as it has typically exported core-like, capital-intensive goods to the periphery; and periphery-like, labor-intensive goods to the core (Özveren, 2000). Moreover, Turkey’s combined efforts to diversify its economic relations through engagement with the semi-peripheral and peripheral economies within its geopolitical near-abroad across the Balkans, Middle East, and Central Asia, as well as to sustain its economic ties with Euro-America has also been identified as an adaptive strategy adopted in response to the particular constraints and opportunities of the semi-peripheral positionality (Arnold & Naseemullah, 2024).

From a political standpoint, the Turkish state has been argued to exhibit a moderate degree of autonomy, neither fully subordinated to the interests of domestic socioeconomic classes nor entirely constrained by international rules and norms, yet not fully autonomous in shaping these contours either (Arnold & Naseemullah, 2024). In parallel with its semi-peripheral position, while the relative strength of the Turkish state machinery has been historically showcased by the state capacity to implement state-led developmentalist strategies and adopt import substitution

industrialization (ISI) model (Eren-Vural, 2007b, p. 346; Özveren, 2000), the structural constraints inherent in Turkey's position within the capitalist world-system including persistent reliance on foreign capital, technological dependency, exposure to external economic volatility, pressure from international organizations, and domestic political instability have significantly debilitated these efforts. These challenges ultimately prompted a shift towards market-oriented economic policies in the subsequent decades (Özveren, 2000). Another indicator of Turkey's political-economic semi-peripherality could be identified as the significant yet fluctuating degree to which the Turkish model of development has been adopted by the peripheral states within its sphere of influence, particularly in regions such as the Middle East, Central Asia, and the Balkans, where countries like Azerbaijan, Kazakhstan, and Bosnia and Herzegovina have looked to Turkish case to inform their own development trajectories. Finally, recent analyses of Turkey's position within the contemporary capitalist world-system have also argued that the country embodies "new semi-peripherality" on ideological grounds, highlighting its substantial subversive potential within the global order, underpinned by its ideological stance as the cultural "other," shaped by the adoption of "Ottoman Orientalism" to challenge Western hegemony (Moore & Dannreuther, 2009, pp. 138-139).

Still, it is imperative to challenge the tendency to uncritically assign Turkey a semi-peripheral status and to resist conceptualizing its trajectory as a natural linear progression from the periphery to the core. Furthermore, semi-peripherality cannot be expected to apply par excellence to every point in Turkey's historical trajectory, as such conceptual shortcuts obscure the multi-dimensional and historically contingent nature of Turkey's position, the shifting political, economic, and social processes that have redefined this position within the capitalist world-system at various junctures,

and the divergent experiences of various groups shaped by religion, ethnicity, socioeconomic class, gender, sexuality, geographic location, and the urban–rural divide. Nevertheless, this thesis asserts that, while Turkey's semi-peripheral status has not been linear or uniform throughout its history, it is now increasingly recognized as a relatively consolidated, albeit still dynamic, instance of semi-peripherality (Akçalı & Korkut, 2015; Alnasseri et al., 2011; Arnold & Nasemullah, 2024; Moore & Dannreuther, 2009).

Another particularly prominent dimension of the Wallersteinian world-systems theory for the prospects of this thesis is its emphasis on the agency and historical trajectory of groups and classes in relation to the capitalist world-economy. Indeed, Wallerstein (1974/2011) contends that, under the capitalist world-economy, the overriding imperative of production and innovation is the pursuit of maximum profit through market exchange, a dynamic that systematically subjugates use-value to exchange value, thereby eroding any incentive to address even the most fundamental human needs when such efforts fail to conform to the imperatives of market rationality or contribute to the expansion of profit margins. As such, the imagination of the profiteers of the world-system is actualized through the labor of the oppressed, whose surplus is systematically appropriated and reinvested into the production of resources to which they lack access, a process that simultaneously culminates in the reproduction of the capitalist world-system and counter-struggles from the oppressed, with these opposing forces being dialectically linked.

In this context, both the nation-state and social groups, regardless of whether they are class- or status-based, transform into mechanisms of coding, decoding, and re-coding the activity of agents, which are reduced into spaces or value-creative forces to be colonized. The capitalist world-system compels social groups to navigate

their economic interests within a unified world market, while simultaneously seeking to distort, challenge, or circumvent its structures for the benefit of the group through organized efforts to influence nation-states, which vary greatly in power yet none of which exert complete control over the world-market. Accordingly, social and political agents, as members of classes and status groups, are best understood as elements of the world-system where their emergence, consolidation, political roles, socioeconomic conditions, organizational activities, interests, potential, and imagination are intricately determined by their collective and dialectical relationship to the overarching world-economy. Thus, the geopolitical, political-economic, and spatial scope of the self-definition of a group or class in the capitalist world-economy emerges as a critical determinant, alongside its state of self-consciousness, economic function, organizational capacity, and its relationship to the means of production, all of which together shape its sociopolitical agency and historical trajectory (Wallerstein, 1974/2011). The emphasis placed on the agency and historical trajectory of class- and status-based groups within the capitalist world-economy positions world-systems theory as a valuable theoretical tool for examining the experiences, demands, interests, and struggles of these groups, which is vital for understanding disparities in global access to orphan drugs, as this issue is inherently linked to human lives and requires attention to both macro-structural dynamics and the lived realities of those affected.

Another valuable aspect of world-systems theory for this thesis is its insistence on conceptualizing the capitalist world-system as a historically specific totality, thereby providing a solid theoretical foundation to balance historical specificity and analytical universality (Wallerstein, 1974). To understand the capitalist world-system, world-systems theory emphasizes the necessity of adopting

the world-economy as the primary unit of analysis and examining inequality and social mobility on a global scale over extended periods, with particular focus on their relational dynamics. Against the ahistorical models of social change, Wallerstein (1974) argues that although the quest of examining social transformations over long historical time requires the logical division of the historical time into segments due to its simultaneous inclusion of continuities and transformations, "development" from one historical time to another have to be understood a posteriori, rather than assumed a priori.

Indeed, through its abstention from analyzing underdevelopment within non-systems such as states or cultures and its emphasis on historical specificity, world-systems theory resists parochial assumptions, including the notion that underdevelopment stems from internal deficiencies to be remedied through contact with the core. Combined with a conscious effort to theorize from the point of view of the subaltern, such commitment to a historical materialist theoretical framework equips world-systems theory with the theoretical means to effectively counter Eurocentric meta-theoretical narratives that have historically served colonial and imperialist projects under the guise of scientific objectivity (Mielants, 2012). Indeed, as noted by De Sousa Santos (2020), the exclusion, oppression, exploitation, and discrimination inherent in capitalism have expanded into and intensified within the epistemological and cultural spheres, where the imperial capitalist order of the Global North has imposed its epistemological and cultural foundations upon the diverse ways of being and knowledge systems across the Global South. As pharmaceutical production, testing, protection, and distribution are inherently global processes governed by the principles of a global market, analyzing disparities in access to orphan drugs requires adopting the world-system as the unit of analysis.

Examining such disparities solely at the national level risks reducing them to local deficiencies, thereby obscuring the impact of broader structural forces that may prioritize profit over human life, exchange value over use-value, and the needs of wealthier nations and social classes over those of the people in need.

Finally, much like other Marxist and Neo-Marxist frameworks, world-systems theory distinguishes itself from bourgeois thought not by "the primacy of economic factors" or a radically different level of analysis, but by its "point of view of totality", which interprets economic processes not as inevitable, neutral, optimal, self-regulating, and harmonizing phenomena; but as embedded within and constitutive of a global system of domination, exploitation, and dependency permeating all aspects of human life (Lukács, 1923/1972, p. 27). Despite remarkable advancements in medical research and the growth of pharmaceutical production, the true extent and persistence of global disparities in access to life-saving medications only become apparent through a reorientation of our analytical focus, revealing how these inequities contribute to the avoidable loss of life-years and hinder the capacity of the global community to effectively address diseases that disproportionately impact marginalized populations. By placing a much-needed emphasis on and providing the necessary tools for the analysis of socioeconomic inequality and justice on a global scale, world-systems theory offers a particularly robust framework for examining the structural inequalities embedded in global access to orphan drugs.

2.3 Understanding care labor in the capitalist world-system: A Marxist-Feminist perspective

"I am, because we are; and since we are, therefore I am."

(Mbiti, 1969, p. 141)

The instrumental and exploitative rationale of the individual, introduced by classical economic theory and epitomized in the contemporary neoliberal world-system as *homo economicus*, fundamentally contradicts the ethos of *homo curans*, actively undermining cooperative, nurturing, and non-exploitative ways of engaging with both fellow humans and the natural world (Tronto, 2017). Therefore, the imperative to meaningfully interpret the struggles, emotional landscapes, and lived experiences of families of children with SMA in their efforts to access treatment within the broader context of the capitalist world-system necessitates a critical framework that foregrounds human relationality, affective justice, and the labor of care. To this end, this thesis incorporates critical insights on gendered, affective, and relational labor from Marxist feminism to enrich the application of world-systems theory to the lived realities of families navigating the systemic barriers to accessing SMA gene therapy in Turkey. Indeed, insofar as these dimensions of the human experience remain unincorporated into the capitalist world-system, they persist as unnamed, unspoken, disregarded, and exploited, yet they equally hold the potential to become powerful sites of resistance if named, claimed, and mobilized to confront the hegemonic values of neoliberal capitalism (Federici, 2019; Lynch, 2021; Oksala, 2016). Given the scope and limitations of the present inquiry, this thesis refrains from attempting to reconcile the inherent tensions within these theoretical discourses. Instead, recognizing their fundamental complementarity, it adopts a critically reflective stance that acknowledges the critical ethos underpinning these frameworks while engaging selectively with their insights to illuminate the case at hand, without collapsing their distinct epistemological and methodological contributions into a reductive synthesis.

The production and reproduction of humans, non-human animals, and nature are inextricably intertwined with care labor, a fundamental practice that the capitalist

world-system depends on to sustain and nurture all life forms that are subjects of its exploitation and commodification (Federici, 2012; Patel & Moore, 2018). Nonetheless, under the "commodity-producing civilizational model" prescribed by the contemporary capitalist world-system, life in its human, non-human, and ecological forms is either reduced to units of unrealized capital oriented exclusively toward market imperatives or relegated as uncommodifiable externalities subject to neglect. In either case, life is construed within the capitalist logic as interchangeable and consequently also individually expendable, thereby not only its needs, sufferings, and relationality are systematically disregarded, but the very labor of caring for and reproducing life is "disassociated from value" (Ferrarese, 2017; Patel & Moore, 2018; Scholz, 2009, p. 127). Altogether, although it benefits extensively from the use value generated by care labor, capitalist world-system treats care as private, cultural, domestic, and ostensibly personal, thereby circumventing any responsibility for its provision, regulation, sustenance, and compensation (Federici 2012; Fraser, 2016; Müller, 2019).

This economic model demands adherence to an ethos of "entrepreneurial individualism," characterized by hyper-competitiveness, atomization, self-referentiality, and a detachment from relationality, presenting this ontology as both a natural and aspirational model of human behavior (Bröckling, 2015; Lynch, 2021; Mau, 2015). Such ontology disparages human dependency and interdependency, as well as the affiliations, affections, and commitments that motivate individuals to act contrary to self-interested calculation, framing such actions, driven by altruism, concern, friendship, loyalty, compassion, love, sympathy, gratitude, and generosity, as aberrations at worst or as apolitical, private, and personal matters at best (Held, 2005; Kittay, 2019; Lynch, 2021; Nussbaum, 1995; Okin, 1989). At the meso-level, it

translates into what Adorno (1963/2005) referred to as “bourgeois coldness”, that is, a community-level sense of suspicion, anger, resentment, apathy, and lack of responsibility towards others, particularly those who are misaligned, unusable, or vulnerable in the capitalistic logic (Lynch & Kalaitzake, 2018; MacDonald, 2011). This ethos finds reflection at the macro-level in the instrumentalization of state apparatuses and legal frameworks to serve market imperatives, leading to their reconfiguration into systems that are increasingly welfare-indifferent, care-oblivious, exploitative, hierarchical, and profit-oriented (Frericks, 2011; Gingrich & Häusermann, 2015; Mau, 2015). Together, the multi-scalar manifestations of “entrepreneurial individualism” call for not only the reclamation of a care-centric, co-created, relational concept of the individual person but also of the wider economic, socio-political and legal order for the facilitation of egalitarian and care-led socioeconomic change (Folbre, 1994; Tronto, 1993).

Moreover, the orientation of production towards profit-driven imperatives and the commodification of labor under the capitalist world-system creates a fundamental misalignment between the timescapes of care work and that of capitalist production. Regardless of whether it takes the form of childrearing, household management, educational care, environmental care, nursing, emotional support, caregiving for the elderly, disabled, sick, or chronically ill, or mobilizing politically for solidarity against related injustices, care labor inherently occupies biological, ecological, and lived time, often characterized by its open-ended, cyclical nature, and a profound demand for proximity, presence, and intimacy. According to Lynch (2021), the compression of time into a rigid construct of "clock-time” under the capitalist world-system relegates love, care, and solidarity work to the margins, where such activities are performed only with the residual time and energy that remain after fulfilling labor

obligations dictated by market imperatives. At the same time, the accelerated, dehumanized, and commodified production within the capitalist world-system is only made viable through the concurrent marginalization and exploitation of affectional, relational, reproductive, and solidarity work, which is pushed to peripheral or interstitial "timescapes" within domestic spaces, communal settings, social networks, educational contexts, and caregiving institutions, where it functions to reproduce, nurture, and educate the labor force essential for capitalist production, while relieving it of reproductive, affectional, relational, and care-centered responsibilities. Consequently, those who engage in care work are often pushed out or at least to the margins of the "time–money earning nexus", depriving them of the opportunity to effectively sell their labor-time in a manner that is recognized within the capitalist world-system. Accordingly, these individuals are frequently left without "free" time, personal space, and even the cognitive-affective capacity to adequately attend to their own needs, as they must allocate their time, energy, and resources to meet the needs of "others" within a system that neglects the demands and well-being of both those requiring care and those providing it (Bryson, 2007).

In neoliberal thinking, dependency that calls for care is often viewed as evidence of weakness, incompleteness, or defect in the adult human condition, which is seen as hindering a person's ability to contribute effectively to society as an active citizen. Although care labor is materially productive in that it sustains individuals through fostering their mental, emotional, physical, and social development, it is systematically positioned within categories of non-value-producing and non-citizenship-defining work under the capitalist world-system given its association with the needs of non-citizens (Lynch, 2021; Müller, 2019; Oksala, 2016; Sevenhuijsen, 1998). Furthermore, the very nature of care labor in its association with "the dirty

work" of managing the often involuntary and uncontrolled leaks of excretions, wastes, soils, and odors of a vulnerable, frail, sick, or disabled body underlines its abjection in the psycho-cultural ethos of the contemporary capitalist world-system. In its attempt to sanitize the concept of the human body from associations with vulnerability, fragility, disability, mortality, and decay, this ethos has constructed a completely managed, contained, intact, cleansed, and controlled prototypical body while systematically obscuring those who fall outside this ideal and those closely associated with them from the public sphere (Banerjee & Rewegan, 2017; Lynch, 2021).

Furthermore, the capitalist world-economy depends on and perpetuates a gendered and racialized division of labor, particularly in domains such as care labor, which remain systematically marginalized, excluded from formal legal protections, and denied institutional recognition or collective bargaining rights (Federici, 2012; Folbre, 2020; Glenn, 2010; Gutiérrez-Rodríguez, 2014; Romero & Pérez, 2016). Particularly in developing and less developed countries, women disproportionately assume the role of primary caregivers, often by default (Coffey et al., 2020). Although this disparity is less pronounced in dual-earning families and highly developed countries that offer robust childcare supports, extensive care facilities, flexible work arrangements, comprehensive social safety nets, strong legal protections, and generous paid parental leave for both parents, women dedicate significantly more time than men to childcare, care work, and housework (Cederström, 2019; Lynch, 2021; Raley et al., 2012).

Within the framework of Cartesian dualism[7], care is associated with the natural, the private, the domestic, the bodily, the emotional, the reproductive, and the

7 A philosophical framework that posits a fundamental distinction between mind and body, reason and emotion, and culture and nature.

animal, thereby also ostensibly aligning with the feminine. Consequently, care work is systematically segregated from, yet is assumed to inherently occupy, a separate sphere from the societal, the public, the political, the rational, the productive, and the masculine, leaving it without the public status, socioeconomic returns, and political power associated with the entities within the latter domain (Bordo, 1986, pp. 450-452; Patel & Moore, 2018, pp. 111-137). Accordingly, caring was assumed to be "natural work" for women as opposed to an autonomous system of social relations, one that is equally available and legitimately exploitable as natural resources and non-human animals (Lynch, 2021, p. 42; Patel & Moore, 2018). Thus, the disproportionate division of care labor also impoverishes women, especially working-class, poor, divorced, immigrant, and otherwise marginalized women intersectionally, in the present as well as in future time, particularly in old age, due to their lack of economic independence and pension entitlements (Coffey et al., 2020). As women's unwaged care and domestic labor often relieve men of care-centered responsibilities, the perspectives and experiences of men are less likely to be shaped by the daily demands of attending to the ongoing corporeal, social, and emotional needs of dependent others. Instead, patriarchal capitalism frequently positions men as "care commanders", socializing them to embody ideals of emotional detachment, hyper-independence, and self-referentiality while maintaining authority over the organization and provision of care-centered, affective, relational, and reproductive labor within domestic, private, and informal spheres (Lynch et al., 2016, pp. 132–157).

These tendencies are reflected at the systemic level within state machinery, legal institutions, labor market structures, and social policies that marginalize care labor, exploit the time, energy, and relational voluntariness of caregivers, and

undermine their efforts at solidaristic or organized resistance, while systematically refusing to establish the political, economic, cultural, and legal conditions necessary to support the provision of care and the well-being of caregivers and dependent others. As such, the systemic embodiment of patriarchal capitalism not only perpetuates a form of "contributive injustice" towards women and caregivers in general but also renders the capitalist world-system itself increasingly vulnerable to care crises (Lynch, 2021). Globally, care labor supports the production of goods and services on a scale comparable to the formal economy, both in terms of output volume, diversity, and the number of individuals engaged in its execution (Folbre, 1994). In fact, unpaid care work performed by women aged 15 and above was estimated in 2020 to hold a monetary value equivalent to three times that of the entire global technology sector (Coffey et al., 2020, p. 8). Despite the undeniable significance of the use value generated by care labor and the crisis-prone tendencies of the capitalist world-system, however, care work remains largely invisible except in instances of long-term neglect or malpractice. Even under such circumstances, it is rarely addressed as a political or structural issue, instead systematically framed as failures fully attributable to "dysfunctional" families, "poor" parenting or the perceived moral shortcomings of the class, race, gender, or marital status of the caregiver (Dodson, 2009).

The voluntary nature of care labor makes it incompatible with the type of compulsion that market imperatives impose on other forms of work within the capitalist world-system. Nevertheless, as is the case with other forms of labor, the political, economic, cultural, and legal frameworks in which care labor is situated profoundly affect the extent to which individuals, institutions, and organizations can enact care, love, or solidarity, as well as sustain the interpersonal networks integral to

these practices (Lynch, 2021; Wilkinson & Pickett, 2018). Failure to simultaneously recognize and address its structural, political, affective, and interpersonal dimensions risks abandoning care labor at the juncture of care and capital to the point of depletion or degeneration, which compromises not only the wellbeing of caregivers and those for whom they care, but also the very fabric of society, which relies on care labor to produce people "in their humanness", as citizens, and as labor force (Lynch, 2021; Oksala, 2016, p. 297). Moreover, in many instances, this labor is supported solely through crowdfunding and philanthropy, yet while the former often lacks the capacity to confront systemic capitalist injustices, the latter can be appropriated by "philanthrocapitalists" as a novel corporate strategy, enabling them to cultivate a valorized public persona, secure legitimacy, and consolidate soft power (Giridharadas, 2019).

Families of children with SMA navigate a complex and contradictory experience at the intersection of the psychological, socioeconomic, bureaucratic, physical, temporal, and emotional implications of caregiving, juxtaposed with the market-driven imperatives of the capitalist system, which both marginalize and commodify this labor while providing minimal institutional support, recognition, or compensation for the efforts involved. An academic effort to navigate this intersection requires confronting the systemic inequalities it exposes through the incorporation of care labor as a critical dimension of analysis. Without this inclusion, the true human cost of global disparities in pharmaceutical access remains inadequately understood, falling victim to the very reductionism inherent in the ethos of *homo economicus*, which systematically overlooks and exploits the relational and affective dimensions central to the experience of *homo curans*.

CHAPTER 3

LITERATURE REVIEW

3.1 Exploring the nexus of intellectual property, innovation, and economic development

The available empirical evidence has yet to establish a definitive link between strengthened intellectual property rights (IPRs), innovation, and economic growth (Auriol et al., 2022; Cho et al., 2015; Neves et al., 2021). Some studies suggest widespread benefits of strong intellectual property rights (IPR) for all stakeholders in the long run (see Dinopoulos & Segerstrom, 2010; Kwan & Lai, 2003), while others argue that IPRs should be relaxed to promote innovation and growth in developing economies (see Deardorff, 1992). Most studies, however, highlight notable variations in outcomes, at least in degree, between the developed economies of the Global North—which seek to safeguard incentives for, control over, and profits from their innovations—and developing or underdeveloped economies, which must navigate a complex array of conflicting considerations, such as the pursuit of trade relations with advanced economies, the satisfaction of domestic demand, resisting external pressures, and managing the trade-offs inherent in their economic contexts (Auriol et al., 2019; Auriol et al., 2022).

The variation in the direction and strength of the impact of IPRs on innovation and growth, as observed in the literature, has been attributed to a range of methodological factors, including the datasets utilized, the selected variables, the level of analysis employed (e.g., firm-level versus state-level), the selected time-frame, and the specific sectors under investigation, among other considerations. Moreover, the impact of robust intellectual property protections on innovation and

economic growth, whether positive or negative, has often been found to be contingent upon various stage- and context-dependent factors (Baker & Avafia, 2011), including, but not limited to, the level of development (Sweet & Eterovic Maggio, 2015), imitative capacity (Falvey et al., 2009), innovation capability (Léger, 2005), distance to the world technology frontier (Chu et al., 2014), degree of globalization, national absorptive capacity (Hammami, 2021), the examined economic sector (see Hu & Png, 2013; Cho et al., 2015; Woo et al., 2015), market size (see Eicher & García-Peñalosa, 2008; Grossman & Lai, 2004), market concentration, the level of local expertise (Parello, 2008), the openness of the economy (Maskus, 2000), the examined channels of production (Lai, 1998), the form of innovation (Bessen & Maskin, 2009), as well as initial IPRs levels and specific national income thresholds (Hudson & Minea, 2013).

Regarding the IPR-innovation nexus, the literature generally acknowledges a trade-off for developing and underdeveloped economies, though there is a lack of consensus as to whether potential increases in foreign direct investment (FDI) inflows (see Branstetter & Saggi, 2011), licensing and technology transfers, and the attraction of multinational corporations (MNCs) or foreign investors (see Dinopoulos & Segerstrom, 2010)—along with, though less consistently, the incentives for domestic innovation—compensate for potential monopolistic effects on the diffusion of knowledge and knowledge-intensive goods, as well as potential reductions in autonomous innovation capacity through mechanisms such as imitation, reverse engineering, and learning-by-doing (Correa, 2000; Odagiri et al., 2010). Indeed, in the context of developing economies, the introduction of stronger patent regulations has often been linked to a rise in non-resident patents produced by foreign firms based in developed countries, while resident patents, export discoveries, domestic

innovation, and autonomous research capacity, tend to either decline or exhibit little change following the enforcement of more stringent intellectual property regimes (see Arza et al., 2023; Auriol et al., 2022; German-Soto & Chapa Cantú, 2018). Moreover, as Odagiri et al. (2010) argue, intellectual property (IP) may be just one of several factors influencing economic growth, and for developing countries in particular, it is likely that there are other more binding factors that play a larger role in driving economic development. Although the aggregate effect of extending patent protection on innovation is more often observed to be at least marginally positive, there remains significant debate regarding the myriad stage and context dependent factors, such as economic complexity, human capital, market size, and economic openness, which influence the distribution of the benefits and drawbacks associated with these effects, potentially resulting in disparities that remain obscured in aggregate data.

Regarding the IPR-growth nexus, the impact of IPRs on the economic growth rate of the host country has been frequently assessed indirectly through intermediate determinants, such as R&D capacity, technology transfer, foreign direct investment (FDI), and total factor productivity (TFP), through which innovation and technology spillovers are commonly considered to contribute, though often conditionally and inconclusively, to productivity, welfare, and economic growth. Some studies have identified strong IPRs as a significant determinant of development, associating their adoption and enforcement with a permanent increase in technology and licensing transfers to the Global South through multinational corporations, a sustained rise in R&D employment within Southern affiliates of Northern multinationals, and a gradual reduction in the North–South wage gap (Dinopoulos & Segerstrom, 2010; Eicher & Newiak, 2013). However, the literature predominantly agrees that strong

IPRs stimulate the highest levels of economic growth in developed economies by driving R&D capacity and returns, but they diverge in their conclusions regarding the conditions under which IPRs foster growth in developing and less developed economies. On one hand, some studies identify a threshold, based on factors such as initial GDP, human capital, innovation capacity, income, openness, or market size, above which IPRs can effectively drive growth (Chu et al., 2014; Diwakar & Sorek, 2016; Kim et al., 2012). On the other hand, other findings suggest a U-shaped relationship where IPRs fuel growth in developed economies through R&D and in less-developed economies through technology transfer, yet leave developing countries burdened by offsetting losses from restrictions on imitation, reverse engineering, and learning-by-doing (Auriol et al., 2022; Chen & Puttitanun, 2005; Falvey et al., 2006). Likewise, although strengthening intellectual patent protection has often been associated with an increase in economic growth on an aggregate level, there is also evidence, although somewhat contested, that it is accompanied by increasing inequalities and losses in welfare (Chu & Peng, 2011; Deardorff, 1992). Finally, one study identified intellectual property protection as a potential placebo on growth (Gold et al., 2019).

Complemented by historical analyses arguing that the adoption and enforcement of a robust intellectual property regime, which the now advanced economies advocate for, was not a prerequisite for their own economic growth during their periods of development (Hudson & Minea, 2013; Peukert, 2017), empirical evidence highlights a lack of consensus regarding the impact of strengthening IPRs on innovation and growth, drawing attention to the inference that a single global standard of IPRs may be sub-optimal and that the world may be in need of an intellectual property regime “which evolves” (German-Soto & Chapa

Cantú, 2018; Hudson & Minea, 2013, p. 13). Here, it is contended that the impact of IPRs emerges as a distributional issue, where, at worst, developing and less-developed countries disproportionately bear its negative consequences, or, at best, fail to fully reap the advantages that accrue to developed countries. Thus, following DeCamp (2007), it can be logically argued that IPRs exert significant distributive effects in at least three key areas: (a) the types of knowledge or knowledge-intensive goods that are developed and for which protection is sought, (b) the differential access various actors have to these knowledge and goods, and (c) the allocation of IPRs themselves among different stakeholders (DeCamp, 2007, p. 315-317; Timmerman & van den Belt, 2013, p. 50). It is within this context that the challenge of deconstructing the distributive complexities inherent in intellectual property regimes finds alignment with Wallersteinian world-systems theory.

3.2 Exploring the impact of patent protections on pharmaceutical industry

Within the context of the pharmaceutical industry, the impact of intellectual property protections has mainly been examined with regards to (i) its ability to stimulate domestic pharmaceutical innovation and/or R&D efforts, and (ii) its effect on access to medicines. Empirical findings concerning the former nexus have yet to establish a definitive link, with most highlighting significant variations in outcomes, at least in degree, between developed and developing or less developed countries. For instance, Liu and La Croix (2014) find that patent protection, alongside factors such as human capital and economic openness, does not significantly influence domestic innovation, although population size and GDP are found to play a role, with GDP being particularly important for developing countries (p. 29). On the other hand, Qian (2007) identifies a conditional positive impact that depends on the level of economic

development, education, and financial freedom of the protection implementing country, with an inverted U-shaped relation between IP strength and domestic innovation. Similarly, Kyle and McGahan (2012) illustrate that increased patent protection had a positive impact on R&D efforts in high-income countries, but not for new treatments for diseases that primarily affect low- and middle-income countries (p. 1171). Finally, Gamba (2017) observes that while patent protection has a positive effect on domestic pharmaceutical innovation in developed countries, the identified effect drops to only 33% and is short-lived in developing and less developed countries, potentially due to limitations in innovation capacity, infrastructure, finances, and human resources (p. 24). Moreover, Gamba (2017) argues that if countries are induced to introduce further protection to stimulate such impact, negative complications may emerge due to royalty stacking or patent hold-up, paralleling Qian (2007) in that the relationship between patent protection and innovation may follow an inverted U-shaped curve. Shifting the focus to the patenting of genes, Sampat and Williams (2019) find that patented genes are initially more valuable than their non-patented counterparts, but overall, gene patents have not significantly impacted subsequent innovation. Similarly, within the context of the human genome sequencing by the public Human Genome Project and the private firm Celera, Williams (2013) shows that intellectual property protections resulted in a 20–30% reduction in subsequent scientific research and product development, indicating that the short-term exclusivity of these patents had lasting negative effects compared to a scenario where the genes remained publicly accessible (p. 24).

Regarding the latter nexus, in response to the anticipated adverse effects of the TRIPS-based intellectual property regime on access to pharmaceuticals in the Global South, numerous studies have been conducted to quantify the impact of

changes in product patents, data exclusivity, secondary patenting, patent term extension, patent linkage, and TRIPS flexibilities, particularly within the context of TRIPS[8] and TRIPS-plus[9] provisions, on the costs, prices, and availability of medicines in developing and less-developed countries (Islam et al., 2019; Tenni et al., 2022, p. 3). First, the global expansion of TRIPS-plus provisions through bilateral and regional trade agreements has been linked to higher costs, increased prices, reduced consumption, greater national expenditures, lower access, and delayed entry of biosimilar alternatives (Tenni et al., 2022). For example, Grootendorst and Hollis (2011) examine the impact of the Comprehensive and Economic Trade Agreement (CETA), finding that EU proposals would significantly extend the exclusivity period for innovative drugs, thereby raising costs for Canadian payers. Lexchin and Gagnon (2014) estimate this would result in a cost increase of 6.2% to 12.9%, while Beall et al. (2019) project an annual rise of $410 million in drug costs. In the case of the US-Thailand FTA, Akaleephan et al. (2009) analyze the effects of data exclusivity and report that the absence of generics would reduce accessibility by 34.9% (p. 177), while Kessomboon et al. (2010) project a 32% increase in the price index and a loss of up to USD 3.3 million for the domestic pharmaceutical industry over 20 years due to patent extensions and data exclusivity. Similarly, Abbott et al. (2012) assess the US-Jordan FTA, finding that market exclusivity and data protection led to a 53% increase in total medicine costs between 1999 and 2004, with a 17% increase after adjusting for inflation and sales volume. Malpani (2009) adds that the data exclusivity clause delayed generic competition for 79% of medicines, leading to a 20% price increase between 2002 and 2006. The

8 A WTO agreement setting minimum global standards for intellectual property rights.
9 Provisions, often included in bilateral and regional trade agreements, that impose stricter intellectual property protections beyond those required by TRIPS.

impact of CAFTA in Guatemala, as explored by Shaffer and Brenner (2009), highlights how the data exclusivity clause restricted access to generics and delayed new market entries. Trachtenberg et al. (2019) also find that FTAs with stricter intellectual property provisions in Chile contributed to both increased volume and higher prices of imported biologics. Lastly, Moir et al. (2018) model the potential effects of the Trans-Pacific Partnership (TPP) in Vietnam, estimating that full utilization of TRIPS flexibilities could enable up to 82% of eligible HIV patients to access antiretrovirals (ARVs), whereas a shift to TRIPS-plus provisions would decrease this figure to just 30% (p. 10). This suggests that in IP-maximalist contexts, constrained policy space for invoking flexibilities may directly result in reduced access to life-saving treatments for large segments of vulnerable patient populations.

On the other hand, the utilization of TRIPS flexibilities, particularly compulsory licensing[10] and parallel importation[11], has often been associated with reductions in national healthcare expenditure, improved access to medicines, and substantial gains in quality-adjusted life years (QALYs). Yamabhai et al. (2011) analyze the impact of government use licenses for seven patented drugs in Thailand, estimating an additional 84,158 patients gaining access and 12,493 QALYs gained over five years (p. 1), while Scopel and Chaves (2016) report a 30% reduction in the price of lopinavir/ritonavir following compulsory licensing in Brazil (p. 8). The impact of parallel importation has been most frequently studied in the European context, with Duso et al. (2014) finding that parallel imports reduced the prices of patented drugs by 11% in Germany, resulting in a €19 million annual increase in

10 The legal mechanism through which a government permits the use of a patented invention without the consent of the patent holder, typically under conditions aimed at safeguarding public health.

11 The authorized import of genuine products from lower-priced markets to foster competition and reduce domestic prices, bypassing the rights holder's consent.

demand-side surplus between 2004 and 2010 (p. 1038). Similarly, Méndez (2018) models the effects of banning parallel importation in Denmark, showing that such a policy would result in higher profits for original producers, increased national healthcare expenditure, and a decrease in both generic firm profits and consumer welfare, while Granlund and Köksal-Ayhan (2015) report a 15-17% price reduction due to competition from parallel imports in Sweden. Together, these examples underscore how strategic regulatory interventions can counterbalance the exclusionary effects of patent-based monopolies, leading to significant price reductions, expanded patient access, and improved health outcomes.

Moreover, the impact of patent expiry, generic entry, and generic pathways has been linked to significant price reductions (Liu & Galarraga, 2017), growing generic market share (Berndt & Aitken, 2011), improved health outcomes (Elek et al., 2017), drops in daily treatment costs (Hill et al., 2017), and increased health savings. Liu and Galarraga (2017) further illustrate the effects of generic availability on drug pricing in the Global South, finding that generic status is the best predictor of market price for antiretroviral drugs in Southern Africa (p. 170). Together, these findings illustrate that intellectual property regimes shape the timing, conditions, and competitiveness of generic entry. Data exclusivity periods and patent linkage mechanisms embedded within bilateral and regional trade agreements effectively delay or constrain the timely introduction of affordable generics, thereby entrenching market exclusivity, sustaining monopolistic prices, and limiting access to essential medicines for patients in low- and middle-income countries, as well as those among the global poor.

Finally, the empirical literature examining the impact of specific patent policies on access to medicines presents mixed findings. While substantial evidence

suggests that extended and robust patent protection may accelerate the launch and diffusion of new pharmaceutical products (Cockburn et al., 2016), some studies indicate that this effect may not be as pronounced in developing and less-developed countries (Dai & Watal, 2021; Kyle & Qian, 2014). Moreover, even if such an effect is observed, it may not lead to reduced prices or enhanced access to medicines, particularly in developing and less-developed countries. Indeed, as Jung and Kwon (2015) argue, stricter intellectual property protections may be linked to significantly reduced access to medicines, even when accounting for country income level, socioeconomic status, demographic characteristics, and healthcare system variations. For example, Borrell and Watal (2002) find that transitioning all drugs from a patent to a no-patent regime could increase the percentage of people living with HIV receiving treatment with new drugs by at least 30% (p. 5). Similarly, Hellerstein (2012) finds that in countries with competitive pharmaceutical markets, drug prices account for approximately 20% of average income, whereas in countries with monopoly supply, they represent over 90% (p. 111). Chaudhuri et al. (2006) estimate an annual welfare loss of up to $450 million due to patent enforcement in India's quinolone market. Likewise, analyzing the effects of patent enforcement on pricing and corporate profits across 43 drugs in India, Dutta (2011) estimates an average price increase of approximately 42%, with a total consumer welfare loss of $378.5 million and a 48% decrease in overall consumer welfare, including the loss of nearly 8.5 million patients, resulting in a 50% reduction in patient access due to patent enforcement and price deregulation (pp. 161, 177).

Overall, empirical research on the impact of patent regimes on drug launch timelines indicates that while more stringent patent provisions can accelerate the market entry of new, particularly "blockbuster" drugs, this effect may be largely

confined to high-income countries where pharmaceutical production is targeted towards lucrative markets and may not translate to increased access to pharmaceuticals. The empirical literature examining the impact of particular TRIPS-plus provisions such as data exclusivity, patent term extensions, and secondary patenting on access to medicines has often identified negative impacts on access to medicines. Studies on patent term extension indicate heightened government healthcare expenditures (Kesselheim et al., 2006; Nelson et al., 2011), increased drug prices (Kesselheim & Solomon, 2010; Kesselheim et al., 2006; Nelson et al., 2011), and delays in drug availability (Kesselheim et al., 2006). Likewise, secondary patents[12] have often been linked to longer monopoly periods that delayed the market entry of cheaper generics (Amin & Kesselheim, 2012; Yin, 2023), increased costs to taxpayers (Nelson et al., 2011), social welfare losses (Yin, 2023), and the practice of "patent evergreening" (Beall et al., 2016). Given the ongoing debate regarding the innovation-stimulating effects of intellectual property protections, their potentially conditional benefits, along with their negative and distributionally skewed implications for pharmaceutical access, it has been suggested that the implementation of such protections should be gradual and context-dependent (Gamba, 2017).

3.3 History of the IP regime: TRIPS, TRIPS-plus, and resistance for access to medicines

"Who owns the patent on this [polio] vaccine?"

"Well, the people, I would say. This is – could you patent the sun?"

— Dr. Jonas Salk, in an interview with Edward R. Murrow, (1955).

12 Patents granted on modifications of existing drugs, such as new formulations, dosages, methods of use, or delivery mechanisms, which can extend market exclusivity beyond the expiration of the original (primary) patent.

The lack of consensus in the theoretical and empirical literature has not significantly impeded the establishment, adoption, and enforcement of intellectual property protection across the core-periphery divide (Richards, 2015/2004). Since its emergence within the capitalist world-system, the protection of intellectual property rights has evolved into a global regime of monopolistic control, expanding in strength, scale, and scope over an increasingly diverse range of patentable subject matter mainly through the enforcement of the Agreement on Trade-Related Aspects of Intellectual Property Rights (TRIPS) and successive bilateral, multilateral, and regional free trade agreements (FTAs) incorporating TRIPS-plus provisions (Löfgren & Williams, 2013, p. 1; Sell, 2011). Despite being preceded by various international agreements on intellectual property (IP), such as the Paris Convention, the Madrid System, and the Berne Convention, the TRIPS Agreement stands out as the most comprehensive, robust, detailed, and legally binding accord ever established in the field of intellectual property rights (IPRs) (Athreye et al., 2020, pp. 315-316). Although prior accords set broad commitments around fundamental principles, TRIPS Agreement is the first to impose binding prescriptions and proscriptions on the national policies of all signatory states, accompanied by significant penalties for non-compliance, thereby signaling a shift from an "international" to a "global" intellectual property regime (Maskus, 2014). With the changing balance of power in the global political and economic landscape, coupled with the fragmentation and globalization of production and value chains, emerging technologies, and increased international mobility of labor, however, has significantly reshaped the scope and impact of the TRIPS-based intellectual property regime, as well as the positions of the key actors involved (Athreye et al., 2020, pp. 316-318). Today, the TRIPS-based intellectual property (IP) regime encompasses a growing set of norms, rules,

institutions, policies, and practices, with enforcement, impact, and resistance varying widely across jurisdictions (Deere-Birkbeck, 2010).

The TRIPS-based intellectual property regime was presented by proponents to promote economic growth, socioeconomic welfare, and technological advancement for all stakeholders by protecting the interests of innovators, creators, and businesses against counterfeiting and imitation, while recognizing the need to harmonize the rights of intellectual property holders with the broader public interest in accessing knowledge, culture, and essential goods (Clift, 2010, p. 3; Deere, 2008, p. 9; El Said, 2022, p. 307). Prior to the 1990s, patent terms differed significantly between industrialized and developing countries, with the former typically granting patents for 15 to 17 years, while the latter offered shorter terms, and many nations, including in Western Europe, exempted critical sectors such as food, medicine, and agriculture from standard patent protections (Moon et al., 2012, p. 1; Watal, 2001). In the context of the profitability crisis (Eren-Vural, 2007b, p. 354) and the increased structural power of the transnational capital (Eren-Vural, 2013, p. 221; 2007b, p. 339), through the efforts of patent-sensitive transnational corporations, the TRIPS Agreement established global minimum standards for patents, copyrights, trademarks, trade secrets, and other forms of intellectual property (IP) in relation to products and processes across all technological fields, including those crucial to human well-being, as part of the broader creation of the World Trade Organization (WTO) in January 1995 (Löfgren & Williams, 2013; ’t Hoen, 2009). As such, the TRIPS Agreement marks the extension of patent protections into fields directly linked to public health, food security, and technological development, such as pharmaceuticals, biotechnology, agriculture, medical technologies, chemical industries, and software for all WTO members. The prescribed standards of

patentability, broadly based on the criteria of novelty, inventiveness, and industrial applicability, mandate a minimum of 20 years of protection for all patents, along with civil, administrative, provisional, and border enforcement mechanisms, all safeguarded by potential penalties to ensure compliance by WTO signatories (World Trade Organization, 1994, Arts. 27, 33, 41–61). By 2000, the TRIPS agreement was implemented by all developed and most developing countries. Overall, the TRIPS Agreement has strengthened intellectual property protections by (1) expanding the range of knowledge eligible for patenting, (2) narrowing the exemptions to the exclusive rights granted to patent holders, and (3) extending the duration of patent protection (Athreye et al., 2020, p. 318).

To mitigate the short-term economic impacts and allow countries time to modify their national IP laws in accordance with TRIPS provisions, transition periods were allocated to all members in consideration of their level of development. Moreover, the agreement came to recognize various "flexibilities," including the ability to define patentability criteria (Arts. 1, 27) in a manner that reflects national priorities, the right to challenge patent grants through patent oppositions (Arts. 32, 62), the establishment of "bolar" and research exceptions (Art. 30) to facilitate the production of generics for regulatory purposes, the allowance for parallel importing (Art. 6) to reduce prices and improve access to goods, and the use of compulsory licensing (Art. 31) as a means to address broader social or economic concerns (Tenni et al., 2022, p. 2; World Trade Organization, 1994). The scope and effective enforcement of these transition periods and "flexibilities", however, had to be shaped and, in many cases, secured through sustained resistance from developing country governments and civil society organizations (Athreye et al., 2020; Eren-Vural, 2007b). Even when TRIPS flexibilities were recognized *de jure*, developing and less-

developed countries have faced significant challenges in their implementation due to pressure, threats, and sanctions from developed countries and international organizations (Correa, 2022; Gopakumar, 2015; Vawda, 2022). Once again, as intellectual property laws operate within the contemporary economic, social, political, and legal context, their implementation and application—whether at the national or global level—ultimately reflect the power hierarchies of the capitalist world-system.

The Uruguay Round and the TRIPS Agreement were reached under duress, as the Global North have persistently employed a combination of "hard diplomacy" and "soft diplomacy" to push developing and less developed countries to accept provisions predominantly aligned with the interests of multinational corporations and advanced economies. "Hard diplomacy" has primarily been exerted through the imposition of trade sanctions, penalties for non-compliance, threats of revoking preferential trade access through mechanisms such as the Generalized System of Preferences (GSP) and the U.S. Special 301 Report, as well as legal coercion through the WTO's dispute resolution system (Clift, 2010; Löfgren & Williams, 2013, p. 14; Muzaka, 2011, p. 68). On the other hand, "soft diplomacy" has been most influential in the form of economic concessions, diplomatic leverage, ideological persuasion through public relations campaigns, the promotion of global norms by entities like the U.S. Trade Representative (USTR) and the International Intellectual Property Alliance (IIPA), capacity-building initiatives through multilateral institutions such as the World Bank and WIPO, along with guarantees of refrainment from resorting to unilateral and bilateral pressures (Deere, 2008, p. 4; Löfgren & Williams, 2013, p. 14). The persistent pressure from core economies to adopt and enforce TRIPS and TRIPS-plus provisions has been supported by empirical analyses suggesting that the

willingness to enforce IPRs is a U-shaped function of the relative size of a country's internal market to its export market. Specifically, advanced economies with large GDPs relative to their export markets have been argued to apply and advocate for IPRs to safeguard profits from and control over their innovations, while less-developed economies with low GDPs relative to their export markets were found more inclined to adopt IPRs to attract trade, technology transfer, and foreign investments. In contrast, developing countries with intermediate GDPs relative to their export markets, due to their greater capacity to resist pressure from the core, tended to prefer infringing IPRs to serve their domestic markets (Auriol et al., 2022; Diwan & Rodrik, 1991).

Although Article 1 of the TRIPS Agreement safeguards members from being required to "implement in their law more extensive protection than is required by this Agreement" (World Trade Organization, 1994), Sell (2011, p. 2) asserts that "TRIPS was never enough." While developed countries, especially the United States, made promises that they would not seek unilateral, bilateral, or further multilateral initiatives if a multilateral trading system was established during the Uruguay Round of Negotiations in 1994 (El Said, 2022, p. 311), they have continued to advocate for the global expansion, enforcement, and tightening of the TRIPS-based intellectual property regime beyond agreed levels with coercive, penal, and reward-based incentives (El Said, 2005, p. 57). Indeed, in the post-TRIPS era, the United States has established Free Trade Agreements (FTAs) with 20 countries, primarily developing nations, which have been increasingly mirrored by the European Union (EU) forging similar agreements with various developing countries, collectively advancing a shift toward strengthening global intellectual property protections beyond the baseline established by the TRIPS Agreement (Sell, 2007; 2011).

Pressure from developed country governments, international organizations, and multinational corporations, often reinforced by patent-sensitive domestic industries (Chaudhuri, 2013; Eren-Vural, 2013), has persistently steered developing and less-developed countries toward aligning their IP frameworks with what are considered the "highest international standards," characterized by longer protection periods, expanded patentable scope, and limited exemptions (Athreye et al., 2020; El Said, 2005, p. 57). In exchange for adopting TRIPS-plus provisions, developing countries were offered trade concessions, including enhanced market access, tariff reductions, technical assistance, and foreign aid, while facing the threat of penalties such as the withdrawal or non-renewal of trade preferences, restrictions on foreign investment, or the imposition of diplomatic pressure, including being labeled as adversaries of trade liberalization or the global anti-terrorism agenda (Baker & Avafia, 2011, p. 13). Domestic deficiencies in socioeconomic, judicial, institutional, resource-based, political, and innovative capacities within developing and less-developed countries have further exacerbated their vulnerability to external pressures (Correa, 2011; Löfgren & Williams, 2013). Finally, the growing polarization within certain knowledge-based sectors in developing and less-developed countries, where emerging exporters and innovators coexist alongside traditional generic producers, has created opportunities for the formation of strategic lobbying coalitions between multinational corporations, foreign associations, their local counterparts, and patent-sensitive segments of domestic industries, collectively exerting pressure to align intellectual property frameworks in these countries with higher international standards (Baker & Avafia, 2011; Chaudhuri, 2013; Eren-Vural, 2013). Together, these factors have not only facilitated compliance with and alignment to TRIPS and TRIPS-plus provisions but have also, with varying success, discouraged developing

and less-developed countries from fully utilizing the transition periods, flexibilities, and leeway provided by the TRIPS agreement—mechanisms widely regarded as key means through which resistance to the advancement of the patent agenda could be mounted (Correa, 2011). This has led to the use of TRIPS flexibilities being variable (Deere-Birbeck, 2010), limited, and sporadic in practice (’t Hoen et al., 2018, pp. 185-193). Overall, given the power asymmetries that underlie its negotiation, enforcement, and expansion, the TRIPS-based intellectual property regime has been framed as a manifestation of “postcolonial vulnerability”, extending from the colonial era into the contemporary capitalist world-system (Deere-Birbeck, 2010; Krishna, 2022; Rahmatian, 2010; Sell, 2003; Yu, 2011).

The earliest responses, most critical scrutiny, and fiercest resistance to the intellectual property regime have emerged in the context of pharmaceutical innovation, health equity, and access to essential medicines. The rationale for extending patent protection to pharmaceutical products and processes has been that it is the most efficient mechanism through which the costs of undertaking the high-risk, capital-intensive, and time-consuming research and development (R&D) processes required for the creation of new drugs, vaccines, and diagnostics could be recouped (Eren-Vural, 2007a, p. 110). Indeed, the development of a new drug is widely regarded as an extraordinarily resource-intensive process, with estimates suggesting that the average cost exceeds $2 billion, while the timeline for bringing a new drug to market typically spans more than ten years (Akınsola & Liang, 2025). Indeed, according to Scherer (2010), the pharmaceutical industry has an R&D on sales ratio that is approximately seven times higher than other manufacturing industries, with as few as one out of 10,000 tested compounds ending up as a marketable drug (pp. 541-543).

Still, the idea that product patents are a fundamental precondition for pharmaceutical innovation is a relatively recent concept, as evidenced by the fact that at the start of the Uruguay Round of negotiations, more than 40 developing countries, including Turkey, provided no patent protection for pharmaceuticals (Chien, 2003, p. 864), while others granted only process patents, which, in a number of countries, including India and Brazil, facilitated the growth of domestic generic pharmaceutical manufacturing industries (Baker & Avafia, 2011, p. 7). In fact, according to Watal (2015), pharmaceutical products and processes remained exempt from patent protection in most European countries until the late 1970s. For instance, Italy and Sweden did not grant pharmaceutical patents until 1978, with Spain, Portugal, Greece and Norway implementing such protection as late as 1992 (Dutfield, 2017). The historical reluctance to provide patents for pharmaceutical products and processes has been linked to various domestic considerations, including the promotion of a local industrial bourgeoisie, the pursuit of autonomous industrial development, the goal of achieving national self-sufficiency, the protection of public interests in access to fundamental needs, and the implementation of protectionist measures to safeguard domestic markets from external competition (Eren-Vural, 2007a, p. 112; Löfgren & Williams, 2013, p. 6).

The campaign for the global expansion of intellectual property rights (IPRs) was largely driven by the lobbying efforts of the Pharmaceutical Research and Manufacturers of America (PhRMA), with major pharmaceutical corporations such as Pfizer and Johnson & Johnson playing pivotal roles in advocating for stronger protections (Sell, 2003, p. 107). In general, developing and less developed countries initially sought to exclude intellectual property (IP) from the Uruguay Round of negotiations in 1994, advocating for a clear distinction between trade rules and IP

regulations. Failing in this attempt, they redirected their efforts towards opposing the provision that would mandate patents on products and processes of fundamental importance to human wellbeing, especially pharmaceutical products and processes. Despite their inability to prevent the inclusion of this provision, the details surrounding the pharmaceutical patent requirements—such as the length of transition periods and the question of retroactive application—were the result of protracted negotiations and compromises, not only between the Global North and Global South but also among developed countries themselves (Athreye et al., 2020, p. 316). By 2005, pharmaceutical patents were almost universally available, in all but the least developed economies (Shadlen et al., 2019).

Mandating pharmaceutical patent protection for all WTO members, the TRIPS agreement has had profound implications for public health, not only for the citizens of developing and less-developed countries but also for socioeconomically disadvantaged populations worldwide. While the scope and magnitude of these effects remain debated, evidence suggests that the TRIPS-based intellectual property regime has exacerbated access issues by: (1) obstructing and delaying the market entry of affordable generic or biosimilar alternatives, (2) shielding essential pharmaceuticals from the downward pricing pressures typically exerted by generic competition, (3) limiting the capacity for autonomous research and development (R&D) in the Global South through the restriction of practices such as reverse engineering, learning-by-doing, and imitation, (4) redirecting local producers in the Global South towards niche markets in developed economies, (5) limiting the dissemination of pharmaceuticals from these producers to countries reliant on affordable supply, and (6) amplifying the challenges of addressing the unique healthcare needs of the Global South (’t Hoen, 2009; ’t Hoen et al., 2011). The

resulting barriers to pharmaceutical access have often been found insurmountable through foreign aid and medicine contributions alone, as these measures fail to provide a comprehensive and sustainable solution to the underlying systemic challenges (Schafheutle et al., 2002).

Amid mounting concerns about the impact of the TRIPS on public health and access to medicines, particularly in the Global South, the TRIPS has faced significant opposition from developing country governments and civil society organizations in its aftermath, with resistance persisting through a variety of legal, diplomatic, and grassroots efforts to this day. Resistance against the TRIPS-based intellectual property regime in the post-TRIPS era has often involved initiatives such as compulsory licensing and the domestic adaptation of TRIPS provisions, particularly in efforts to prioritize public health and address access to medicines during health crises. Despite significant pressure from transnational corporations and the governments of the Global North, efforts in South Africa, India, Brazil, Thailand, and other countries to utilize TRIPS flexibilities to address domestic health needs, particularly during HIV/AIDS crises, have gained important victories, ultimately contributing to the adoption of the Declaration on TRIPS and Public Health at the Fourth WTO Ministerial Conference in 2001. The Doha Declaration marked a landmark achievement by developing country governments and civil society organizations, affirming the sovereign right of member states to interpret and implement TRIPS provisions "in a manner supportive of WTO Members' right to protect public health and, in particular, to promote access to medicines for all" (World Trade Organization, 2001, Art. 4), notably through compulsory licensing and parallel importation, while granting least developed countries (LDCs) an extended timeline to implement pharmaceutical product patents (Mahadew, 2024). In 2003, the

declaration was supplemented by the "August 30th" decision, which established a procedure to ensure that products manufactured under a compulsory license could be exported to countries without domestic production capacity ('t Hoen, 2009). Despite its potential, the application of this mechanism has been limited by complex procedural requirements and ongoing pressure from developed economies for stronger intellectual property protections, with no internationally recognized exception permitting the waiver of enforcement during health emergencies, a gap that the COVID-19 pandemic has once again exposed, revealing the challenges and limitations of de jure flexibilities in ensuring equitable pharmaceutical access (Ballardini et al., 2022, p. 1151; 't Hoen, 2009).

The variation in the impact of and responses to the extension of intellectual property protection to pharmaceutical products and processes has been attributed to a complex interplay of externally-imposed, circumstantial, and capacity-related factors (Löfgren & Williams, 2013). First, pressure in the form of penal, coercive, and reward-based incentives from core countries has primarily been directed at vocal developing nations, including India and Brazil, whose substantial pharmaceutical sectors and historical resistance to patenting pharmaceutical products and processes have positioned them as key opponents the TRIPS-based intellectual property regime (Deere, 2008). Indeed, concessions on agriculture have been reported as effective in Brazil and Argentina (Sweet, 2013), while textile concessions were notably so in India (Watal, 2015). Moreover, a range of circumstantial factors, including but not limited to, the degree of coordination among domestic stakeholders, the level of involvement of the local pharmaceutical sector in the negotiation processes (Sweet, 2013), the extent of domestic mobilization surrounding public health crises (Vanni, 2016), the proportion of public financing allocated to healthcare (Hsieh, 2013), the

level of dependency of the pharmaceutical industry on foreign technology (Eren-Vural, 2007a; 2007b), the structural organization, integration, and political capacity of the domestic pharmaceutical industry (Eren-Vural, 2007a; 2013), the alliances available to the domestic pharmaceutical producers at the time of policy change (Eren-Vural, 2007b), the strategic orientation of local firms (Chaudhuri, 2013; Eren-Vural, 2013), and the sociopolitical dynamics shaped by the interplay of class relations under the influence of transnational capital (Eren-Vural, 2007b; 2013), have proven to be among the most decisive at various points in shaping the positions and interests of developing countries, often driving shifts in their stance vis-à-vis the proponents of the TRIPS-based intellectual property regime. For instance, Sweet (2013) links Brazil's shift from opposing any patent standards for medicines, a stance driven by its HIV/AIDS free-access program starting in 1996, to its eventual complete acceptance of such standards to the lack of an adequately coordinated civil society base, persistent external threats, agricultural concessions, the expectation of immunity from future attacks, the promise of increased foreign investment, and the absence of the domestic pharmaceutical industry at the negotiation table. Chaudhuri (2013), on the other hand, highlights the shift in the orientation of Indian pharmaceutical producers towards export and out-licensing to developed economies, emphasizing a preference for incrementally modified versions of products targeting lucrative diseases, rather than the development of new medicines addressing the healthcare needs of its population, particularly those of the economically disadvantaged.

Finally, insufficiencies in judicial, technical, institutional, innovative, and resource-based capacities have been argues to hinder less-developed and smaller developing countries from resisting the patent-driven agenda of developed countries

and global capital, limiting their ability to exploit the flexibilities in the TRIPS Agreement to protect their public health interests, and safeguard their pharmaceutical industries from the impact of the TRIPS-based regime (Correa, 2011). For instance, Iskander (2013) expresses concern over the long-term access to pharmaceuticals in Egypt in the post-TRIPS era, citing the inadequacy of domestic expertise and training facilities, which restricts the capacity for autonomous pharmaceutical R&D by local firms, whereas Manu (2017) notes that less developed economies in Africa have failed to exploit the flexibilities inherent in the TRIPS Agreement to promote their public health interests given their judicial, institutional, and resource-based capacity issues. Here, exemplifying a case through which the Global South can resist against the TRIPS-based intellectual property regime, Plahte and Reid-Henry (2013) show how Cuba has succeeded in its vaccine and cancer drug development despite having "harmonized" its IP regime with TRIPS provisions through partially preserving its authentic approach to science and modifying its IP law only upon having developed an adequate level of domestic R&D capacity.

Recent analyses highlight an alarming tightening of intellectual property (IP) regimes in many developing countries such as Pakistan, South Korea, Malaysia, China, and Turkey (Löfgren & Williams, 2013, p. 11), alongside a growing trend among local pharmaceutical producers to shift their focus towards incrementally modified versions of existing medicines, targeting lucrative disease areas with the expectation of out-licensing to multinational companies in the Global North. For instance, Chaudhuri (2013) and Eren-Vural (2013) emphasize how the TRIPS-based intellectual property regime has reshaped the priorities of the Indian and Turkish pharmaceutical industries respectively, driving them to produce pharmaceuticals targeting lucrative diseases predominantly affecting the Global North for export

purposes, thereby neglecting the urgent healthcare needs associated with diseases that disproportionately affect populations in the Global South. This shift is mirrored by a corresponding strategy among multinational companies in the Global North, which increasingly target the upper income deciles in developing countries who can afford on-patent or branded generic products as a means of reducing costs, capturing new revenue streams, profiting from shifting disease burdens, and compensating for the deadweight losses resulting from the exclusion of the global poor from access to their products (Flynn et al., 2009; Löfgren & Williams, 2013).

Here, Turkey has been argued to position itself as an "IP-maximalist" in its formal legal framework, characterized by weak patentability criteria and numerous TRIPS-plus provisions (Eren-Vural, 2013; Löfgren & Williams, 2013), although a wider body of literature points to a significant gap between de jure intellectual property regimes and their de facto implementation in many developing and less-developed countries (Papageorgiadis & Sharma, 2016). Indeed, unlike Brazil, India, and South Africa, Turkey has largely failed to safeguard its domestic pharmaceutical industry and public health interest, exhibiting minimal resistance to the implementation of TRIPS-based regulatory frameworks (Eren-Vural, 2013; Semin & Güldal, 2008). According to Eren-Vural (2013), Turkey has failed to defend its generic pharmaceutical sector and prioritize public health due to (1) external pressures to align its domestic IP policies with European standards under the influence of the carrot of EU accession, and (2) finding its domestic pharmaceutical sector divided between export-oriented companies and those focused on addressing domestic need, with the former, in collaboration with multinational corporations, exerting significant influence over policy decisions. Indeed, the dependence of the Turkish pharmaceutical capital on foreign technology and imported biochemicals,

coupled with the dominance of the internationalized segments of domestic manufacturers, has prevented the development of a sufficiently strong set of interests distinct from those of transnational corporations and of the global capital (Eren-Vural, 2007a, p. 119; Eren-Vural, 2007b, p. 354). These dynamics ultimately crystallized in the *Industrial Property Code* (Law No. 6769, 2016) enacted in 2017, which provides 20-year patent protection for pharmaceutical products and processes, bringing Turkey into full alignment with TRIPS obligations. While the law includes provisions for compulsory licensing (Arts. 129–132) that theoretically enable state intervention in cases of public health need, these mechanisms have remained largely underutilized in practice. As such, the Turkish case once again illustrates that although TRIPS flexibilities such as compulsory licensing exist on paper, their practical implementation is contingent upon the very political, institutional, and economic conditions that also shape the negotiation, enforcement, and distributive outcomes of the TRIPS regime itself.

At the onset of the Turkish Republic, its pharmaceutical industry was predominantly import-dependent (Semin & Güldal, 2008, p. 381). During the late Ottoman and early Republican periods, pharmaceutical production was primarily confined to small-scale operations within pharmacy laboratories and workshops. The establishment of the Eczacıbaşı Pharmaceutical Factory in 1952 marked the beginning of industrial-scale pharmaceutical manufacturing (Uvey et al., 2004, p. 47). In 1954, the introduction of the "Foreign Capital Incentive Law," designed to attract foreign investment, alongside the "Pharmacies and Medical Preparations Laboratories Regulations," which imposed stringent financial requirements on producers, facilitated a transition from small domestic enterprises to a surge of foreign capital and domestic-foreign partnerships (Uvey et al. 2004, p. 47). The

decade saw rapid growth in the pharmaceutical sector, with international companies such as Pfizer, GlaxoSmithKline (GSK), Bayer, and Novartis (through its predecessor), entering the Turkish market via direct foreign investment, financing of local firms, and licensing agreements for specific pharmaceuticals. By the 1960s, pharmaceutical production was predominantly conducted under licensing agreements with foreign firms. In response to the increasing influence of transnational pharmaceutical corporations through technology transfer agreements, supply of raw materials, and chemical intermediates, and driven by the competitive pressures faced by local capital, decreasing purchasing power, and foreign exchange shortages, the Turkish government abolished patent protection for pharmaceutical products and processes in 1961 to safeguard national economic interests and foster domestic industry growth (Eren-Vural, 2007a, pp. 113-114). In 1986, local firms in Turkey controlled 62% of the domestic pharmaceutical market, with 90% of the country's demand for pharmaceuticals met by local production, marking one of the lowest market shares held by transnational companies among developing nations (Balance et al., 1992, p. 76). Due to intensifying pressures imposed by the transnational pharmaceutical capital on developing countries in favor of stronger patent protection, increased structural power and mobility of the transnational capital (Eren-Vural, 2007a, pp. 119-121), the formation of a new transnational class alliance (Gill, 1991), increasing lobbying efforts (Eren-Vural, 2007a; 2007b), along with the broader process of privatization and liberalization in the context of globalization (Semin & Güldal, 2008), brought about a policy change towards stronger intellectual property protections in the country throughout the 1980s and 1990s.

Although the abolition of patent protection in Turkey offered local pharmaceutical manufacturers greater commercial independence from transnational

corporations, granting access to alternative technologies and non-patented pharmaceutical chemicals, they remained largely dependent on foreign technology and the imported pharmaceutical chemicals, resulting in no tangible advancements in research and development capacity in the pharmaceutical industry despite having a higher percentage of domestic producers relative to that in India, Brazil, and Mexico. (Eren, 2002; Eren-Vural, 2007a, p. 114; Eren-Vural, 2007b, p. 354; Semin & Güldal, pp. 382-383). In Turkey, pharmaceutical patent protection was introduced in 1999, ahead of the 2005 TRIPS implementation deadline, in alignment with customs harmonization with the European Union, and its domestic regulations, including the patent law of 1995. The introduction of the patent law has led to substantial difficulties for domestic pharmaceutical manufacturers, forcing them to pay royalties for licensed original products while striving to remain competitive in the market. This shift contributed to the growth of foreign pharmaceutical companies, enabling them to expand their market presence either through the creation of new subsidiaries or through the acquisition of local firms (Semin & Güldal, 2008, pp. 383-384). According to Dorlach (2015), with the onset of the AKP era, Turkey's unique form of social neoliberalism fused orthodox neoliberal economic policies with a notable expansion of social policy activism, thereby fostering a highly uneven trajectory in the development of the welfare state (pp. 539-540). This period was marked by the increasing convergence of healthcare with broader economic growth, market regulation, population management, and humanitarian policies, transforming health from a predominantly social policy issue to a multifaceted political discourse, where competing visions of egalitarian healthcare provision intersected with the economic frameworks promoted by the AKP government and international bodies such as the World Bank (Yılmaz, 2022).

3.4 From orphan drugs to orphan populations: The systemic challenges of access in rare disease healthcare

The definition of rare diseases (RDs) is not universally agreed upon and varies considerably across different jurisdictions. A study by Richter et al. (2015) identifies approximately 296 distinct definitions used by 1,109 organizations globally, with around 88% of these definitions being based explicitly or implicitly on prevalence thresholds, which are typically expressed as a fraction, a percentage, or a fixed number of cases per population. These prevalence thresholds vary widely, with the average threshold ranging from 5 to 76 cases per 100,000 people, with a global mean of approximately 40 cases per 100,000. As of 2020, it is estimated that between 350 and 475 million people are affected by rare diseases worldwide (World Economic Forum, 2020), approximately 80% of which affect children (Dodge et al., 2011). In some instances, definitions may also incorporate qualitative descriptors such as disease severity, high morbidity, premature death, challenges in obtaining proper diagnosis, screening, and treatment, the need for lifelong management, and substantial financial burdens, though such combinations are relatively rare (Adachi et al., 2023, p. 2; Richter et al., 2015, pp. 909-910).

In response to the need to have a common and comprehensive definition of rare diseases, Rare Diseases International (RDI) signed a landmark agreement with the World Health Organization (WHO) in 2019 to develop an operational definition that can be used globally, which brings together a core definition and a complementary descriptive framework (Wang et al., 2024). On one hand, according to the core definition, a rare disease is “a medical condition with a specific pattern of clinical signs, symptoms, and findings that affects fewer than or equal to 1 in 2000 persons living in any WHO-defined region of the world”, which may include genetic

diseases, cancers, infectious diseases, poisonings, immune-related diseases, idiopathic diseases, and undetermined conditions (Rare Diseases International, 2022, n.p.). There are currently approximately 8,000 defined rare diseases worldwide, and the vast majority of them are identified as particularly difficult to treat due to the complexity of their impact occurring at the cellular and organismal levels (Gaffke et al., 2021), along with their severity, immediacy, and irreversability (Nguengang Wakap et al., 2020). The prevalence of orphan diseases varies significantly across regions, with an estimated 25-30 million affected individuals in the United States, 30 million in Europe, 25 million in the Middle East, 40-50 million in Latin America, 50 million in Africa, and 200 million in the Asia-Pacific region, influenced by factors such as population size, genetic diversity, the availability of reliable clinical and epidemiological data, effective monitoring systems, and the existence of standardized diagnostic criteria (Adachi et al., 2023, pp. 5-6).

Due to limited profit potential given the small patient size per rare disease indication, rare diseases are often categorized as "orphan diseases," a term reflecting the limited incentives and the prohibitive costs for the pharmaceutical industry to invest in the research and development of treatments targeting these conditions, which have been correspondingly referred to as “orphan drugs” (Gammie et al., 2015, p. 2). To overcome the market failure in addressing the unmet medical needs of individuals affected by such conditions, approximately 50% of countries worldwide have implemented legislations (Postma et al., 2022; p. 1), regulations and policies to incentivize the research and development of orphan drugs and to address the issues with the licensing, pricing, and reimbursement of treatments for rare diseases with various strategies such as market authorization, market exclusivity, special grants, tax credits, and accelerated approval (Gammie et al., 2015; Luzzatto et al., 2018). Today,

evidence suggests that the research and development of orphan drugs presents several potential advantages, including expedited development timelines, reduced research and development costs, an increased probability of clinical and regulatory success, the ability to command premium pricing, lower marketing expenditures, and a diminished risk of generic competition (Melnikova, 2012; p. 267). Indeed, the size of the global orphan drug market has exhibited steady and significant growth in the last decade, and is estimated to reach US$300 billion by 2028 (EvaluatePharma, 2023).

Despite the advantages associated with orphan drug development, however, the prices of already approved orphan drugs remain exceedingly high, often reaching hundreds of thousands, or even millions, of dollars or euros per dose (Luzzatto et al., 2018). Indeed, orphan drugs are typically priced up to five times higher than non-orphan drugs, with some of the most expensive treatments on the market falling into the orphan drug category (Chambers et al., 2020). This substantial cost burden significantly limits the ability of insurance companies and government agencies to provide reimbursement for these treatments (Zamora et al., 2019). This issue is particularly pronounced in less developed and developing countries, where constrained financial resources further exacerbate the challenges of ensuring access to these life-saving medications. Although orphan drugs constitute a small share of pharmaceutical budgets, with low per capita spending on rare diseases due to their budgetary impact being largely tempered by the low prevalence of the conditions they address, the controversy surrounding the pricing of orphan drugs is exacerbated by the fact that the financial burden of subsidizing orphan drug development often falls on society, particularly as the acquisition costs of these drugs have risen disproportionately compared to increases in drug spending for common indications

(Postma et al., 2022; p. 1). Overall, there is substantial ongoing debate on whether these prices are an accurate reflection of the true costs or inherent value of orphan drugs (Jayasundara et al., 2019; Nuijten, 2022; Pierzynowska et al., 2020; Sabatini et al., 2022). Here, the pricing of orphan drugs is often justified as a means to recover the substantial investments involved in research and development (R&D), manufacturing, regulatory approval, distribution, and post-treatment monitoring, while critics contend that the high prices of these therapies are driven by monopolistic pricing strategies afforded within the current intellectual property framework, where profit maximization remains the primary incentive rather than equitable access (Jayasundara et al., 2019, p. 1).

As underlined in the descriptive framework of Rare Diseases International (RDI), on the other hand, persons living with rare disease (PLWRD) face distinct and shared challenges regarding accessing adequate, timely, and affordable diagnosis, treatment, and care due to the low prevalence and/or incidence of their conditions, which often causes patients, families, and caregivers heavy emotional, financial, and social burdens. While varying significantly by geographical location, individual insurance schemes, condition, and socioeconomic status, access to affordable diagnosis, care, and treatment for persons living with rare disease (PLWRD) remains a critical priority across patient communities globally. The access of persons living with rare disease (PLWRD) to screening and diagnostic services is often limited by the lack of availability, affordability, and coverage of these technologies, lack of trained specialists, and the inadequacies of Health Technology Assessment (HTA) processes, leading to a 4-5 year delay in the reception of an accurate diagnosis on average (Marwaha et al., 2022). Likewise, the treatment of these conditions is complicated and delayed by the lack of adequate clinical data, the specialized

expertise required for their management, the absence of adequately trained and sensitized specialists, along with gaps in primary care physician education. The absence of comprehensive patient data and epidemiological studies on rare diseases impedes the accurate assessment of their prevalence and incidence, thereby hindering informed decision-making, effective advocacy, further innovation, and the allocation of resources, which are essential for addressing the hidden disease burden through measures such as newborn screening programs and the establishment of specialized care centers. The substantial uncertainty surrounding market approval and reimbursement decisions, stemming from the insufficient availability of clinical data that demonstrates the clinical benefit, cost-effectiveness, potential for widespread adoption, and affordability of novel drugs, technologies, and therapies may contribute to additional delays and complications (Young et al., 2017, p. 1). The inadequate political recognition, prioritization, and support for rare diseases limit funding, further restricting the accessibility and availability of essential medicines for people living with rare diseases (Lopez Gousset & Bolz-Johnson, 2021, pp. 2-3). Today, fewer than 10% of patients with rare diseases are reported to receive disease-specific treatment worldwide (Melnikova, 2012).

Given the lack of curative treatments for the majority of rare diseases, the inadequacies in access to and the scope of supportive care have also been recognized as a significant challenge in managing these conditions, with many patients and families reporting dissatisfaction with the level of physical, psychological, social, and informational support (Depping et al., 2021). In fact, evidence suggests that not only the burden of living with a rare disease is approximately 10 times higher than mass market diseases for a per patient per year (PPPY), but also the absence of treatment is associated with a 21.2% increase in total costs on the same basis (Cioffi

et al., 2021, p. 1). Finally, people living with rare diseases often face sociocultural alienation, blame, exclusion, discrimination, and stigma due to a lack of awareness and understanding surrounding their condition in settings of education, employment, the workplace, and elsewhere (Belzer et al., 2022; Jaeger et al., 2015).

Even when a treatment is available, the high cost of treatments for rare diseases places a significant financial burden on national governments, particularly in developing and less developed countries, where inadequate government reimbursement and insurance schemes often fail to cover these expenses, thereby shifting substantial out-of-pocket expenses onto patients and their families, exacerbating existing inequities in access to essential healthcare. Especially in resource-constrained environments, low socioeconomic contexts, rural communities, less developed economies, and in the case of legal disinfranchisement, patients and their families often face the difficult decision of balancing the need for essential treatments with the demands of their subsistence needs (Adachi et al, 2023; Lopez Gousset & Bolz-Johnson, 2021). According to a study by Chan et al. (2020), merely 19.4% of the 31 low-income countries and territories included in the sample had implemented policies to facilitate access to medications for rare diseases. Combined with various systemic and resource-related challenges—such as inadequate healthcare infrastructure, limited access to specialized genetic testing and diagnostic tools, low medical awareness, insufficient capacity for genome sequencing, and the absence of systematic RD registries—along with a lack of policy prioritization and insufficient funding for RD care due to the burden of more prevalent diseases, patients in less developed and developing countries are often left to navigate the healthcare system without adequate support. This results in delays in diagnosis, limited therapeutic options, and poor health outcomes, with only the most

socioeconomically privileged individuals able to access the necessary diagnostic services, treatment, and care (Adachi et al., 2023). Finally, health crises, such as the COVID-19 pandemic, have been illustrated to exacerbate challenges for patients with rare diseases by overwhelming healthcare systems, especially in developing and less developed countries, leading to the delays in or cancellation of essential medical procedures, which, in turn, can worsen existing health conditions and impede timely access to necessary care (Aktaş, 2021).

Caregiving for patients with rare diseases throughout their diagnostic journey and thereafter has a profound impact on both parental and non-parental caregivers, who provide extensive support in various ways, including administering medications, assisting with mobility, accompanying patients to medical appointments, coordinating medical treatments, managing complex healthcare regimens, offering emotional and psychological support, ensuring access to specialized care, advocating for the patient's needs, assisting with daily living activities, monitoring for potential complications, managing financial matters, navigating insurance systems, and seeking out appropriate resources, all of which are characterized by significant time commitments, considerable emotional strain, physical exertion, and substantial financial burdens (Sandilands et al., 2022). First, caregivers of individuals with rare diseases have been reported to endure multifaceted set of emotional challenges, such as anger, fear, guilt (including transmission guilt, guilt associated with non-caregiving activities, and guilt towards unaffected children), shock, devastation, sadness, loneliness, grief, and at times, relief upon receiving a formal diagnosis. These burdens have also been reported to precipitate suicidal ideation at times, combined with significant levels of clinical depression and anxiety. Caregivers have often also reported encountering difficulties stemming from a lack of knowledge

among general practitioners, dissatisfaction with the quality and accessibility of available information, and a general lack of public awareness regarding rare diseases. Socially, caregivers were found to face strained relationships with their children and partners, diminished opportunities for leisure, feelings of being overlooked, unheard, or silenced by healthcare and social systems (Currie & Szabo, 2019) and the pervasive effects of stigma and social alienation, compounded by a plethora of practical burdens including financial strain, the necessity of traveling for medical appointments and treatments, challenges in balancing caregiving duties with professional commitments, poor work-life balance, challenges in navigating disparities between rural and urban settings, and the reinforcement of traditional gender roles, which disproportionately affect female caregivers (Boettcher et al., 2021; Cardinali et al., 2019; Pelentsov et al., 2015; Sandilands et al., 2022, pp. 15-16). In the face of such burdens, caregivers of individuals with rare diseases have been found to adopt a variety of coping mechanisms, including adjusting their expectations of normal life, embracing gratitude and hope, seeking support through spiritualism/faith, establishing routines, and actively pursuing disease-related knowledge to manage the emotional and physical demands of caregiving. They have also reported relying on multiple sources of support, such as family, friends, healthcare professionals, carer groups, self-help groups (Cardinali et al., 2019), employers, educational institutions, and access to respite care, which collectively help alleviate the strain and enhance their capacity to provide care (Adachi et al., 2023; Sandilands et al., 2022, p. 13). Overall, building on Moon et al.'s (2012) concept of “neglected populations” (p. 1), the persistent inaccessibility of orphan drugs for rare diseases can be reframed as the emergence of “orphan populations,” comprising individuals living with rare diseases in developing and less-developed

countries, as well as those among the global poor, who are systematically excluded from essential healthcare, including timely access to quality diagnostic services, treatment, and care.

Similar to other developing and less-developed countries, the financial burden faced by individuals and families living with rare diseases in Turkey is particularly pronounced, with substantial out-of-pocket expenses that are frequently not reimbursed by the public healthcare system. In Turkey, diseases with a prevalence of approximately 0.1–9 per 100,000 individuals are classified as rare, a threshold that is significantly lower—by a factor of 50—than those commonly used in the European Union and the United States. Consequently, the number of conditions categorized as "rare diseases" in Turkey is markedly lower compared to those recognized in the European Union (Czech et al., 2020; Kılıç et al., 2013). It is estimated that between 5 and 7 million people in Turkey are affected by rare diseases (Aksu, 2019). Estimates indicate that individuals living with rare diseases in Turkey incur substantial out-of-pocket expenses for a range of needs, including but not limited to specialized nutrition, transportation and accommodation, medical and non-medical devices and supplies, pharmaceuticals, patient care, laboratory and imaging tests, hospitalization, and specialist consultations (Koçkaya et al., 2023, p. 6). In fact, Koçkaya et al. (2023) find that, as of 2020, the average cost of rare diseases per household was Ł22,743 (€2,877), with 41% of respondents reporting allocating more than 10% of their annual household income to expenses associated with the disease. Of particular concern, more than 7.5% of respondents indicated that they dedicated more than their entire annual household income to disease-related expenditures (Koçkaya et al., 2023, p. 4). Moreover, of the 105 rare drugs listed by the European Medicines Agency (EMA), 34 were unavailable in Turkey, while of the 71 that were accessible,

23 were licensed and 48 were unlicensed, with reimbursement coverage provided for 17 licensed and 17 unlicensed products (Koçkaya et al., 2021, p. 1). In the absence of sufficient government support or insurance coverage, many families resort to grassroots fundraising efforts, social media campaigns, and organization-led initiatives to secure the necessary financial means for treatment and care. Nonetheless, the financial burden associated with rare diseases often results in significant delays in treatment or even a complete lack of access to life-saving interventions for individuals living with rare diseases in the country. Within this context, the challenges in accessing the gene therapy Zolgensma for SMA patients and their families exemplify a clear manifestation of this broader economic, political, and affective reality.

3.5 Access challenges and the struggle for treatment in spinal muscular atrophy (SMA) care: The case of Turkey

Spinal muscular atrophy (SMA) is a genetic autosomal recessive neurodegenerative disorder caused in approximately 95% of cases by mutations in the survival motor neuron 1 (SMN1) gene on chromosome 5q13.2, which lead to impaired SMN protein production and subsequent degeneration of the alpha motor neurons of the spinal cord anterior horn cells (Lefebvre et al., 1995; Verhaart et al., 2017). The carrier frequency of SMN1 gene mutations is estimated to be approximately 1 in 50 individuals, whereas the global prevalence of spinal muscular atrophy (SMA) is reported to range from 1 to 3 cases per 10,000 live births (Çelik et al., 2024, p. 665). Currently, it is one of the most common orphan diseases, the most common neurodegenerative disease in childhood (Vill et al., 2021), and the most common monogenic cause of infant mortality worldwide (Özdemir et al., 2022). Although this

relationship is complicated by limitations in research, small sample sizes, and disparities in health registries, surveillance systems, and the availability of screening programs across jurisdictions, the variability in the prevalence of spinal muscular atrophy (SMA) has been linked to differences in gene pools and the level of parental consanguinity (Veerhart et al., 2017).

The condition is characterized by progressive motor impairment, proximal muscle weakness, neuromuscular atrophy, respiratory complications, paralysis, and in the most severe forms, early death, often due to respiratory failure (Verhaart et al., 2017). The severity, onset, and trajectory of spinal muscular atrophy (SMA) vary, with clinical manifestations ranging from severe congenital (Type 0) or infantile forms (Type 1-2) to milder forms with later onset and slower progression (Type 3-4) (Moultrie et al., 2025). Indeed, SMA Type 0 is the most severe form of spinal muscular atrophy, typically presenting in utero or within the first few weeks of life, and is characterized by profound muscle weakness, respiratory insufficiency, and the absence of motor milestones, leading to a markedly poor prognosis with most affected individuals not surviving beyond the first few months of life. Type 1 SMA is the most common and severe form of spinal muscular atrophy, typically presenting in infants before six months of age, with symptoms including severe muscle weakness, impaired motor skills, respiratory difficulties, and failure to achieve key motor milestones, leading to a poor prognosis, with most affected individuals not surviving beyond two years without intervention. SMA Type 2 typically manifests in early childhood with significant motor impairment, Type 3 emerges in childhood or adolescence with moderate, progressive weakness, and Type 4 presents in adulthood, characterized by mild, slowly progressive weakness, with a relatively normal life expectancy (Moultrie et al., 2025).

Over the past decade, significant advancements have been made in the treatment of spinal muscular atrophy (SMA) with the development of disease-modifying therapies (DMTs). Approved by the FDA in 2016 and the EMA in 2017, the first disease-modifying therapy against spinal muscular atrophy (SMA) is Spinraza (Nusinersen), which is an antisense oligonucleotide that works by modifying the splicing of the SMN2 gene to increase the production of functional SMN protein. Spinraza (Nusinersen) is administered via intrathecal injections, initially given at four loading doses at days 0, 14, 28, and 63, followed by maintenance doses every four months. Evrysdi (Risdiplam), approved by the FDA in 2020 and the EMA in 2021, is a small molecule that modulates SMN2 splicing to enhance SMN protein production that is orally administered on a daily basis. Zolgensma (Onasemnogene abeparvovec), approved by the US FDA and EMA in 2019, is a gene therapy that delivers a functional copy of the SMN1 gene via a one-time intravenous infusion (see Appendix A for detailed information on currently approved treatments for SMA). Collectively, the development of these therapies has contributed to a reduction in disability, extended patient survival, and improved overall prognosis for SMA patients, ultimately positioning the condition as treatable (Moultrie et al., 2025). Indeed, although achieving normal or near-normal neurodevelopment in children with SMA is now possible, it is highly contingent upon early intervention, with treatment outcomes exhibiting considerable variability based on SMN2 copy number of the patient, clinical status, and the number of surviving motor neurons at the onset of treatment (Dangouloff & Servais, 2019).

This thesis will focus on Zolgensma (onasemnogene abeparvovec-xioi), a gene theraphy developed and marketed by the multinational pharmaceutical company Novartis for pediatric patients younger than 2 years old, which has demonstrated

promising effects in improving neuromuscular function in children with spinal muscular atrophy (SMA), especially upon early intervention (Hoy, 2019). According to Novartis, as of 2024, the therapy has been approved in more than 54 countries, often through contracts incorporating risk-reducing mechanisms such as payment for results, cost sharing, conditional access, tiered pricing, and performance criteria, with over 4000 patients treated worldwide through clinical trials, managed access programs, and commercial settings (Novartis, 2024, p. 7). However, clinical data remains limited and inconclusive, with existing research indicating that Zolgensma does not completely cure the condition (Chen, 2020). Upon its launch, Zolgensma was recognized as "the most expensive drug ever," at over 2 million US dollars for a single-dose treatment, maintaining its position among the highest-priced medicines globally to this day (Nuijten, 2022; Stevens et al., 2020). Different value-based and cost-based pricing models have yielded significantly varied estimates of Zolgensma's profit margins, ranging from negligible (see Nuijten, 2022) to substantial (see Thielen et al., 2022), with these discrepancies largely attributed to the lack of transparency from pharmaceutical companies regarding the costs of research and development, as well as drug manufacturing (Thielen et al., 2022). Other studies found that Zolgensma may be relatively more affordable than chronic treatments of SMA (Craddy et al., 2023).

In Turkey, estimates indicate a higher-than-average carrier frequency, incidence, and prevalence of Spinal Muscular Atrophy (SMA) compared to global averages. Özdemir et al. (2022) estimate the carrier frequency of SMN1 deletion in Turkish population as 1/27.7. In contrast, Ünver et al. (2024) show that the frequency of SMA in the Turkish population may be as high as 1 in 6,000, with ministry records estimating around 150 new cases a year and 3,000 registered patients in the country

(Republic of Turkey Ministry of Health, 2023a, p. 3). Moreover, the prevalence of SMA in Turkey has been shown to exhibit a gradient, increasing from west to east, a pattern that has been associated with higher rates of parental consanguinity in the eastern regions, with one study estimating that approximately 69% of individuals affected by SMA in Turkey have a history of consanguineous marriages (Ekici et al., 2012).

In Turkey, the Social Security Institution (SGK) is granted regulatory authority to determine the types, quantities, durations of use, and payment procedures for health services and pharmaceutical products it finances, with treatment reimbursement being covered within the framework of the Health Implementation Communiqué (SUT). Nusinersen, the first and only SMA treatment available in Turkey, was included into the SGK reimbursement scheme following regulatory decisions, with eligibility for reimbursement granted to SMA Type 1 patients in July 2017 and to Types 2 and 3 in February 2019 (Republic of Turkey Ministry of Health, 2023a). In Turkey, Nusinersen is publicly funded through the Social Security Institution (SGK), whereby authorized hospitals submit specialist-approved treatment protocols for SGK evaluation and, upon approval, receive payment directly from the institution without requiring patients to bear upfront costs. In fact, in a statement dated January 31, 2023, the Spinal Muscular Atrophy Scientific Advisory Board of Turkey revealed that 1,024 patients were receiving treatment free of charge (Republic of Turkey Ministry of Health, 2023b). Criticized for on the grounds that it lacked clear standardization, was applied inconsistently across cases, and failed to adequately capture the complexities of patient conditions, the "physical scoring" requirement for continuation doses of Nusinersen (Spinraza) in SMA patients was also abolished under the Health Implementation Communiqué

(SUT) in 2022. Evrysdi, on the other hand, was added to the Ministry of Health's Foreign Drug List by the Turkish Medicines and Medical Devices Agency (TİTCK) on September 2020, it has not yet been included into the SGK reimbursement scheme. Similarly, Zolgensma was added to the same list in 2020 by ministerial decree following a court ruling, yet it has not been incorporated into the SGK reimbursement scheme. In December 2021, Turkey introduced the SMA Carrier Screening Program, and in May 2022, spinal muscular atrophy (SMA) was included in the National Newborn Screening Program (Republic of Turkey Ministry of Health, 2023a, pp. 6-7). In December 2021, Turkey introduced the SMA Carrier Screening Program, and in May 2022, spinal muscular atrophy (SMA) was included in the National Newborn Screening Program (Republic of Turkey Ministry of Health, 2023a, pp. 6-7). Today, SMA carrier screening is mandatory for all couples applying for a premarital health certificate through family physicians and is also offered, upon request, to already-married couples planning to conceive, whereas newborn screening is universally conducted within the scope of the national newborn screening program. Both programs are accompanied by free genetic counseling, clinical follow-up, and, for couples identified as carriers, access to in vitro fertilization with preimplantation genetic testing at no cost. However, significant knowledge gaps persist among both the general public and healthcare professionals, limiting the effectiveness of early intervention efforts (Çelik et al., 2024).

The management of spinal muscular atrophy (SMA) in Turkey incurs significant financial costs, with medication representing the largest expense. According to Öztürk et al. (2023), the annual total cost for a patient with SMA Type 1 is 2,839,875 TL, of which 2,814,364 TL is attributed to medication. In comparison, the total cost for SMA Types 2 and 3 is slightly higher at 2,922,321 TL, with

2,918,082 TL allocated to medication. The costs for inpatient and outpatient care are relatively low, with SMA Type 1 patients incurring 6,594 TL for inpatient care and 1,478 TL for outpatient services, while SMA Types 2 and 3 patients face inpatient costs of 219.74 TL, outpatient costs of 91.41 TL, and equipment usage costs of 177.78 TL (Öztürk et al., 2023; p. S147). In the face of these challenges, the SMA community in Turkey has organized into two major non-governmental organizations (NGOs), namely the Türkiye SMA Foundation (Türkiye SMA Vakfı) and the SMA Walk with Me Association (SMA Benimle Yürü Derneği), which advocate on behalf of patients and families, addressing their medical, financial, social, and caregiving challenges. The individual fundraising efforts of families and organizations ultimately culminated in a nationwide campaign in early 2021 (Adiller & Güreller, 2021, pp. 406-407).

In its statement dated January 31, 2023, the Spinal Muscular Atrophy Scientific Advisory Board of the country emphasized the lack of a comparative scientific study proving the superiority of Zolgensma over other treatments and consequently stated its refusal to allow children to be used as test subjects (Republic of Turkey Ministry of Health, 2023b). Accordingly, families seeking to access Zolgensma for the treatment of their children with SMA often resort to fundraising via social media campaigns, organization-led initiatives, and grassroot efforts to raise the substantial costs required for treatment abroad, with both the families and their supporters advocating for state approval and coverage of this high-cost therapy. Finally, many families have utilized petition platforms to advocate for the full coverage of all SMA treatments.

Children with spinal muscular atrophy (SMA) face significant challenges in meeting their basic physical needs, such as nutrition, excretion, respiration, mobility,

and daily activities, which severely impair their ability to live independently. On one hand, their condition often calls for a wide variety of specialized equipment and resources, including respiratory care equipment, home adaptations, wheelchairs, and voice amplification devices (Brandt et al., 2022). On the other hand, children with SMA often become highly dependent on caregivers who bear a wide array of responsibilities, including administering medications, assisting with mobility, accompanying patients to medical appointments, coordinating treatment plans, managing complex healthcare regimens, providing emotional and psychological support, ensuring access to specialized care, advocating for the patient's needs, helping with daily living tasks, monitoring potential complications, handling financial matters, navigating insurance systems, and securing necessary resources (Evkaya Acar et al., 2021; Sandilands et al., 2022). In turn, Şimşek and Çetin (2024) found that parental caregivers of children with SMA often experience significant emotional distress, including feelings of helplessness arising from both accepting the situation and lacking time for themselves, distress related to the symptoms of the disease and concerns for their healthy children, stress due to the demanding treatment process and financial strain, fear of death and future pregnancies, and unhappiness due to abrupt changes in their living conditions. These challenges have driven intense and insistent pleas for urgent intervention in Turkey, exemplified by tweets such as: "Imagine watching your child melt away in front of your eyes. Please see our situation, help us save our children. We just want our little ones to live! We do not want to live in fear of death every day!" (Evladınızın gözünüzün önünde eridiğini düşünün! Lütfen halimizi görün, bizi anlatın, yavrularımız yaşasın istiyoruz sadece! Her gün ölüm korkusu yaşamak istemiyoruz!) or demands such as: "The 75 million transferred to the Sovereign Wealth Fund should be used for children with SMA."

(Varlık Fonu'na aktarılan 75 milyon SMA hastası çocuklar icin kullanılsın.) (Adiller & Güreller, 2021, p. 410). The provision of necessary equipment and resources for managing the condition, along with psychological, social, and financial assistance through support networks plays a crucial role in reducing the burden of both parental and non-parental caregivers of SMA patients (Walter et al., 2021).

Overall, the experiences of SMA patients and their families in Turkey underscore a significant access issue, with the complexity of treatment options, the absence of conclusive clinical data, and systemic fragmentation among key stakeholders exacerbating the challenges in securing essential pharmaceuticals. This lack of alignment between NGOs, the government, families, and the public, largely due to the absence of conclusive clinical evidence regarding the efficacy or superiority of Zolgensma, has led to divergent approaches among stakeholders, contributing to highly variable patient outcomes. In this evolving landscape, shaped by both domestic factors, such as the reconfiguration of capacities, priorities, organizations, and alignments among key stakeholders, and the influence of broader global structures within the world-system, the issue of access, along with the ongoing struggle to overcome it, persists.

CHAPTER 4

METHODOLOGY

The present study employs a qualitative single-case study design to investigate the specific manifestations of pharmaceutical access and exclusion that arise in relation to the SMA gene therapy Zolgensma in Turkey, with particular attention to how these dynamics are experienced, negotiated, and contested by various stakeholders. Single-case study methodology was chosen for its capacity to provide an in-depth, thick, context-sensitive, and multi-perspectival account of complex phenomena, especially when the boundaries between the phenomenon and its context are not clearly defined. The case of Turkey is analytically significant and theoretically generative due to its semi-peripheral position in the global pharmaceutical economy, its distinctive regulatory and health policy environment, its relatively high disease incidence, prevalence, and carrier frequency, and its ongoing struggles around rare disease governance, particularly concerning SMA. It allows for the exploration of how global inequities in pharmaceutical innovation are locally experienced, mediated, resisted, and redefined by affected communities and national actors operating within a structurally constrained policy environment. On the other hand, as one of the most expensive single-dose drugs currently on the global market, a treatment targeting an orphan disease, and a product of advanced gene therapy technology, Zolgensma serves as a critical entry point for examining the tensions surrounding high-cost pharmaceutical innovation, the global intellectual property regime, access to medicines, and health equity. In particular, it foregrounds questions of how the benefits and burdens of pharmaceutical innovation and access are distributed across socioeconomic classes and along the core–periphery divide.

To map key actors, trace public and policy debates, and identify major thematic trajectories shaping the discourse on SMA in Turkey, this study systematically reviewed a range of publicly available materials, including ministry documents, institutional reports, newspaper coverage, fundraising appeals, petition texts, official statements, and social media content. Furthermore, a comprehensive review of secondary theoretical and empirical literature was conducted in order to situate the Turkish case within broader global discussions on orphan drug regulation, intellectual property regimes, rare disease policy, and the structural determinants of pharmaceutical access and exclusion. Semi-structured interviews constituted the principal method for collecting primary qualitative data. In total, 14 semi-structured interviews were conducted with a diverse group of stakeholders representing the main social actors, including parental and non-parental caregivers of children with SMA (9), adult-onset SMA patients (3), representatives from the two major SMA advocacy organizations (5), physicians specialized in pediatric neurology and rare diseases (1), legal experts with experience in healthcare law and patients' rights (1), and representatives from grassroots organizations (1). It should be noted that there was partial overlap between participant roles due to the multi-positionality and embeddedness of many actors within the SMA community in Turkey. A detailed overview of participant roles, affiliations, and gender is provided in Appendix B. Purposive and snowball sampling strategies were combined to identify and reach individuals who possess firsthand experience or specialized knowledge relevant to the case. The initial sample was drawn from major SMA advocacy organizations, medical departments involved in SMA care, and health law committees of bar associations. Subsequent participants were recruited through referral chains, which

broadened the participant pool and facilitated the inclusion of adult-onset SMA patients.

The relatively small sample size of interviewees reflects, in part, the limited size of the SMA community in Turkey, a contextually bounded population characterized by close networks and overlapping spheres of engagement. Accordingly, the study prioritized analytical depth and perspectival variation over statistical representativeness, consistent with its qualitative and interpretive orientation. Despite multiple attempts, representatives from relevant governmental agencies, regulatory bodies, and pharmaceutical companies—including Novartis—declined to participate or withdrew from scheduled interviews. As a result, direct insight into corporate decision-making processes and state rationalities was limited, constituting a significant constraint on the perspectival breadth of the study. However, indirect insights into both governmental and corporate positions were captured through official publications (see Republic of Turkey Ministry of Health, 2023a; Novartis, 2024), public statements, and the accounts of other stakeholders who interacted with these institutions through personal struggles for treatment and care access, advocacy efforts, mediation of medical policy, or legal procedures.

All interviews were conducted in Turkish, either in person, via secure video conferencing platforms, or in written format, depending on participant preference and accessibility considerations. The interviews conducted in-person or via video conferencing platforms lasted between 45 to 90 minutes and were audio-recorded with the informed consent of participants. The interviews were guided by a flexible protocol that started by open-ended questions designed to invite personal narratives, contextual insights, and critical reflections, with the direction of each conversation shaped by participant priorities to prioritize exploratory engagement and participant

comfort. Interviewees were encouraged to elaborate, challenge, or clarify meanings throughout the interaction, fostering an atmosphere of co-constructed understanding, with follow-up questions probing emergent themes introduced by the participants themselves when appropriate. All interviews were subsequently transcribed verbatim, anonymized, and translated into English for analysis. All interview recordings and transcripts were securely stored on the password-protected personal computer of the researcher. In keeping with the methodological commitments of the research to collaborative knowledge production and ethical attentiveness, participants were offered the opportunity to review their interview transcripts and the initial interpretations derived from them to confirm, amend, clarify, or expand their statements through member checking, thereby enhancing both the validity and ethical integrity of the collected data. Throughout the data collection process, the researcher also maintained a detailed reflexive journal to record evolving insights, shifts in positionality, emotional responses, and emerging ethical tensions to maintain critical self-awareness.

The data generated through semi-structured interviews were analyzed using thematic analysis, following a structured and iterative approach aimed at "identifying, organizing, and interpreting key patterns of meaning" within the dataset (Clarke & Braun, 2016, p. 297). Thematic analysis was chosen for its flexibility and suitability in capturing the complexity of participants' experiences, particularly in research contexts that foreground social meaning, positionality, and power (Kiger & Varpio, 2020). Accordingly, the collected data was analyzed initially through a round of open coding to identify salient concepts, patterns, and recurring narratives emerging organically from participant accounts. Subsequently, axial coding was conducted to examine relationships among the generated codes, with particular

attention to patterns, tensions, and thematic convergences across accounts. Finally, selective coding was employed to synthesize and refine a set of core themes that captured the central dynamics under investigation in the context of the broader conceptual, theoretical, and analytical framework of the study (Williams & Moser, 2019). Throughout the analytical process, particular attention was paid to preserving the contextual integrity of participants' narratives, with codes and themes being interpreted in relation to the specific social, political, and emotional contexts from which they arose. To ensure that the final analysis remained faithful to the participant accounts, the thematic structure was revisited and refined in dialogue with participants, particularly in cases where clarification, elaboration, or reinterpretation was provided through feedback.

Inspired by the methodology proposed by Cadaval Narezo (2022), the research methodology is informed by a set of principles rooted in collaborative, respectful, and caring research. Centered on relational accountability, critical reflexivity, ethical attentiveness, and the co-construction of knowledge, these principles inform (1) the explicit recognition of the partiality and situatedness of all knowledge claims, (2) the rejection of extractive or objectifying research practices, (3) the prioritization of reciprocal and dialogical engagement with all participants, (4) the commitment to emotional and political care throughout the research encounter, (5) the flexibility to adapt research tools and protocols to participants' needs and contexts, (6) the affirmation of participants as knowers and co-interpreters rather than subjects of study, and (7) the grounding of research outcomes in shared meaning-making (Cadaval Narezo, 2022). In this study, they have been operationalized through flexible interview protocols, participant-led meaning-making, shared transcript review, and a sustained effort to foreground the voices,

concerns, and interpretations of participants themselves, while maintaining attentiveness to the emotional, sociocultural, and structural contexts in which these narratives are embedded. In this study, these principles were operationalized through a range of practices, including the explicit acknowledgment of the emotional and interpretive labor participants undertook in discussing a high-stakes and emotionally charged topic; the prioritization of trust-building and relational care throughout the research process; and the implementation of flexible interview protocols. Open-ended questions were used to allow participants to guide the direction of the conversation, while themes introduced by participants were followed up instead of imposing a predetermined agenda. Space was intentionally created for clarification, elaboration, and contestation of meanings during and after interviews, with transcripts and preliminary interpretations returned to participants for review, correction, or expansion through member-checking. A reflexive journal was also maintained to document evolving insights, shifts in positionality, and emerging ethical tensions, as part of an ongoing commitment to critical self-awareness and accountability. Finally, particular attention was paid to building trust and rapport with participants. I developed sustained connections with several members of the SMA community through social media engagement and extended communication. In some cases, participants welcomed me into their homes, and several caregivers introduced me to their children as an “abla” (older sister). These relationships constituted an integral component of its relational and collaborative orientation, enhancing both the depth of insight and the ethical integrity of the study.

The given research received ethics approval (2025-17T) from the ethics review board of Boğaziçi University, ensuring that all procedures adhered to established ethical standards for research involving human participants. Given the

sensitive, emotionally heavy, and politically contested nature of the research topic, particular care was taken to uphold high ethical standards throughout the research process. To ensure psychological safety and participant comfort at all stages, interviews were scheduled flexibly to accommodate participants' health, caregiving, emotional, and professional circumstances, with all participants being given space to pause, redirect, or discontinue the conversation if needed. In addition, the method of member checking was employed as an ethical imperative grounded in respect, reciprocity, and authenticity in data collection and analysis.

As a researcher positioned within the Turkish sociopolitical context yet shaped by academic training rooted in Western critical traditions, my positionality inevitably informed the framing, conduct, and interpretation of this study. First, this research was also shaped by my prior experience as a content specialist in the biotechnology sector, which provided a working familiarity with the communicative strategies, regulatory discourse, and internal narratives of value within the pharmaceutical industry, while also prompting sustained reflexivity regarding my proximity to its potential biases and embedded assumptions. As a researcher engaged in, but not representative of, the SMA community and broader rare disease advocacy ecosystem, the study was carried out with an acute awareness of the asymmetries of power, privilege, and interpretive authority inherent in qualitative research. Moreover, the evolving and variable position of the researcher across interactions with different participants, at times as an outsider, at others as a confidante, or with a kin-like proximity, served as an epistemologically productive space where trust, care, and dialogical co-construction became integral to the research encounter. At the same time, however, this proximity necessitated careful attention to the risks of over-identification, assumptions of shared experience, and the ethical challenges of

navigating complex emotional terrains. Finally, engaging closely with caregivers, patients, and advocates, and listening to narratives marked by exhaustion, grief, fear, pain, resilience, and hope, I often found myself navigating moments of profound affective proximity. The emotional burden of bearing witness to these accounts, while methodologically generative, also posed challenges that required sustained reflexive accountability regarding my positionality, interpretive authority, and ethical responsibilities. This process necessitated both critical attentiveness and open disclosure about how empathy, emotional labor, and relational ethics shaped my interactions with participants and became integral to the production and interpretation of knowledge throughout the research process. In recognition of knowledge production as an embedded, relational, political, and partial process, the research did not strive for detachment, neutrality, or broad generalizability, but instead aimed to render its methodological advantages and limitations transparent, accountable, and ethically attuned.

Overall, to ensure the trustworthiness of the study, efforts were directed toward establishing credibility, transferability, dependability, and confirmability, in line with established qualitative research standards (Ahmed, 2024). Credibility was enhanced through methodological transparency, prolonged engagement with the SMA community, and the triangulation of interview data with secondary literature and publicly available documents. Transferability was supported through the provision of thick, context-rich descriptions of both the case and the participants' situated experiences. Dependability was addressed through consistent documentation of the research process, including the creation of an audit trail recording data collection, coding, and thematic development. Finally, confirmability was strengthened by mechanisms of member-checking and reflexive journaling (Ahmed,

2024, pp. 2-3). Together, these strategies reflect the broader epistemological and ethical commitment of the research to situated, dialogical, trustworthy, and accountable research practice.

CHAPTER 5

TRACING THE BARRIERS TO ACCESS:

PERSPECTIVES FROM THE TURKISH SMA COMMUNITY

5.1 From inaccessibility to inclusion? Nusinersen and the struggle for access to first-line SMA treatment

The introduction of Nusinersen (Spinraza) in Turkey marked a pivotal shift in the treatment landscape for spinal muscular atrophy (SMA), offering the first pharmacological option for a condition previously defined by the inevitability of progressive decline and early mortality. Introducing the period prior to Nusinersen's formal approval, a pediatric neurologist emphasized the limited therapeutic possibilities at the time and the rapid clinical deterioration often observed in early-onset cases: "Motor functions, orthopedic issues, recurrent lung infections, problems with nutrition, aspiration, weight gain, swallowing, sucking... These would all intensify over time, and as the respiratory issues worsened, we would intubate these patients. But even then, in intensive care, we generally lost them before they turned two." (P07) She adds that the availability of Nusinersen has since significantly altered this trajectory:

> The patients we currently have are generally not doing so poorly with this treatment... The earlier we start it, the better the outcome... Still, (Spinraza) is a treatment that has proven effective in keeping these children alive... and at the very least, compared to the past, mortality rates have dropped significantly—even for SMA Type 1. (P07)

One prominent patient advocate underscores the same shift: "We no longer use the word "fatal" for SMA. Now we only say it's fatal if no treatment is used" (P13).

However, the availability of Nusinersen in Turkey was neither immediate nor inevitable. Families, clinicians, patients, and advocates report a prolonged and

multifaceted struggle for access, marked by institutional inertia and regulatory delays. One prominent patient recalls:

> Before the first treatment for the disease was introduced in Turkey... We had already started following developments through Facebook groups. Then, we met with government institutions to push for the treatment to be included in the early access program and made available first to Type 1 patients. We tried to reach politicians, spoke out to the public and media, organized official actions. There were around 300–400 patient families who all knew each other. (P06)

Moreover, multiple patient advocates recall that their efforts were mediated by a complex and sometimes uneasy political, ideological, and fiscal context (P01, P02, P03, P04, P06, P13). Regarding this point, the same interviewee above added:

> Because we worked in the field of health, we were tolerated by the government and the state—we were able to protest even during the pandemic and under the conditions of a state of emergency. We received support from both the ruling party and the opposition. We are a compassionate nation. (P06)

Yet, this support was reported to present its own limits and contradictions, with the same interviewee recalling that they were often met with the following question in meetings with government officials: “This much of the budget... how can it be allocated to a single disease?”. (P06)

This collective mobilization unfolded alongside a period of profound uncertainty with the emergence of hope in a context where both formal access pathways and clinical guarantees of safety and efficiency were absent. With increasing awareness of global clinical trials but no formal avenues to participate, families resorted to improvised strategies such as “medical queuing” around trial access:

> When we learned that Spinraza was starting to be tested on people, we thought—could we get our patient into a trial?... But we were already too late for that... Everyone was lining up at the door of the doctor they thought might enter their baby into a trial, thinking, maybe if my baby gets the drug early, we can save them. (P06)

The urgency of these efforts was underscored by the mounting loss of life. As the same advocate notes: “The state boasts about how fast it acted, but during those 7 months, we lost 35 babies.” (P06)

Ultimately, the inclusion of Nusinersen (Spinraza) in Turkey’s national reimbursement scheme for SMA Type 1 patients in July 2017—and for Types 2 and 3 in February 2019—was the outcome of approximately 2.5 years of sustained, multifaceted effort by an emerging SMA community in the country. As such, the former inaccessibility of and the struggle for access to Nusinersen (Spinraza) in the late 2010s bears a striking resemblance to the contemporary therapeutic, bureaucratic, socioeconomic, and affective landscape surrounding Zolgensma. Multiple interviewees explicitly drew parallels in terms of temporal urgency, distributive injustice, familial despair, and the absence of formal access pathways, unfolding within a shared context shaped by improvised and prolonged efforts to navigate global pharmaceutical capital, an opaque healthcare system, uncertain clinical outcomes, and the relentless demands of a rare, progressive, and currently incurable disease (P01, P02, P03, P04, P06, P07, P13). A parental caregiver and a non-affiliated patient advocate underlines this parallels by saying: “Every treatment available in Turkey has been attained through struggle.” (P04) An NGO representative and non-parental caregiver similarly notes: “We clawed our way to this point with our bare hands, and that’s exactly why we know its value.” (P06) On the other hand, a pediatric neurologist specializing in rare diseases notes how access to Nusinersen (Spinraza) was limited by its high price point and overall cost to the healthcare system in Turkey:

> Nusinersen isn’t a cheap drug either... When it first came out, we couldn’t even give it to all the patients with Type 1 SMA... We had to exclude those whose respiratory issues exceeded a certain threshold and only provide it to

> the relatively better-off cases. Even in that limited form, we were told it accounted for roughly a third of the Ministry of Health's budget. (P07)

However, while the struggle for access to Nusinersen (Spinraza) ultimately culminated in concrete state action and reimbursement within a relatively defined timeframe, the pursuit of Zolgensma access remains protracted, decentralized, fragmented, contested, and unresolved at a national level. Unlike Nusinersen (Spinraza), which marked the first pharmacological breakthrough in the treatment of SMA, Zolgensma emerged as a second-line intervention with an exponentially higher financial burden on both the public system and individual fundraising efforts. Still, this parallel, while imperfect, underscores a pattern wherein global advancements in SMA treatment continue to emerge as contingent, unevenly distributed, and mediated by national policy constraints and broader pharmaceutical inequities; ultimately rendering such breakthroughs prolonged and contested sites of struggle for pharmaceutical access for the SMA community in Turkey.

5.2 Ongoing struggle: The structural inaccessibility of Zolgensma in Turkey

The interviews exhibit a range of articulations regarding the inaccessibility of Zolgensma, voiced by parents, NGO representatives, patient advocates, medical professionals, and legal experts. Together, these perspectives converge to frame the issue as a systemic injustice with significant ethical, clinical, and political implications within the rare disease policy landscape in Turkey—variously described as a geographically contingent exclusion, violation of the right to life, a denial of citizenship entitlements, a breach of the social state principle, a barrier to case-specific and timely medical intervention, and a structural determinant of therapeutic delay, suboptimal outcomes, and access asymmetry. A legal expert, drawing on constitutional protections, national jurisprudence, and international human rights

instruments such as the Universal Declaration of Human Rights (UDHR), International Covenant on Economic, Social and Cultural Rights (ICESCR), Convention on the Rights of the Child (CRC), European Convention on Human Rights (ECHR), and ILO Convention No. 102 underscored that both constitutional and international provisions collectively affirm the legal obligation of the state to ensure timely and equitable access to life-saving treatments, stating:

> The principle of the social state enshrined in the Turkish constitution, along with the state's obligation to promote the material and moral well-being of the individual, the principle of equality before the law, the right of everyone to protect and develop their material and spiritual existence, the right to live in a healthy and balanced environment, and the right to social security, collectively affirm that all citizens' right to life is safeguarded under the state's guarantee and positive obligations.... International treaties likewise affirm the right to life, health, and social security...stating that everyone has the right to social security....to medical care for themselves and their families..... and to protection in cases of illness or livelihood disruption due to conditions beyond their control (UDHR). Everyone has the right to benefit from all necessary measures that ensure access to the highest attainable standard of physical and mental health (ECHR). Regarding the provision of life-saving medications for patients it is emphasized that...while those benefiting from health assistance—or the heads of their households—may be required to contribute to healthcare costs in the event of illness, such contributions must be determined in a way that does not impose a heavy financial burden on the individual (ILO No:102). (P14)

Accordingly, she characterized the inaccessibility of Zolgensma as a failure of institutional accountability, where regulatory and financial barriers override guaranteed rights to health, life, and social security:

> So far, lawsuits filed to demand coverage of the drug's cost have been rejected on the grounds that "the drug is not licensed in Turkey, is not included under the provisions of the Health Implementation Communiqué (SUT), its efficacy has not been conclusively established, it is considered a low-probability treatment, and the state may exercise discretionary authority to protect public financial resources." Yet, regulations cannot contravene the Constitution or the law, and restrictions that compromise the essence of the right to social security are, therefore, clearly unlawful. The coverage of treatment costs falls within the constitutional duty and obligation of the state under the right to health and social security. In fulfilling its economic and social responsibilities, the state may not enact limitations that would effectively abolish the right to life or the right to preserve and improve one's

> physical and mental integrity. Imposing conditions other than medical necessity for the coverage of treatment costs is legally impermissible. (P14)

On the other hand, highlighting both the constitutional principle of the social state and the necessity of individualized medical intervention, one leading NGO spokesperson and parental caregiver outlined the guiding position of their organization as the following:

> There's no such thing as the disease—there is only the patient. Every patient has different needs and treatment requirements. Based on the principle of the social state, all patients in our country should be able to access the treatments they need free of charge, guided by their physicians... Our goal is to have all three treatments—each with different mechanisms and modes of administration—covered by reimbursement. (P01)

Adding another dimension to the discussion, an adult-onset SMA patient and NGO member emphasized that while recent years have seen considerable progress in treatment development, access remains socioeconomically and geographically contingent:

> Until recently, none of these treatments were available in our country... In the past few years, there's been rapid and significant progress... But of course, access still depends on which country you're in. We're still in a position of having to demand things, but it wouldn't be right to lose hope. (P03)

Echoing this point, a founding NGO member and parental caregiver agrees that: "These aren't prices that healthcare systems in developing or underdeveloped countries—like Turkey—can easily absorb on their own." (P01) In addition, a pediatric neurologist with extensive experience in SMA care points out the structural limits of national healthcare funding for high-cost therapies targeting SMA in the Turkish context, noting:

> Turkey, like many European countries, tries to finance access to medication for these patients without imposing a blockade. Gene therapy—given it only requires a single dose—makes sense, but the cost is enormous. Not every country can afford that. Yes, maybe the U.S. or some Northern European countries can manage it, but they don't have as many children with SMA as we do. We estimate that around 150 babies with SMA are born in Turkey each year… and from the perspective of our national economy, that's not a negligible number. If we had very few patients, maybe it would be feasible,

> but in a society with this many SMA patients, I don't think it's currently possible—given our income level and economy—to offer gene therapy to every child. (P07).

Beyond concerns about economic feasibility and disease prevalence, some participants emphasized the impact of international patent regimes and the profit-maximizing imperatives of pharmaceutical companies as key structural barriers to equitable access to Zolgensma in Turkey. As one legal expert explained:

> Global and national legal frameworks surrounding intellectual property rights directly influence access to treatment. Novartis, is able to sell the drug at an extremely high price due to patent protection. The TRIPS Agreement grants patent holders 20 years of exclusive rights over pharmaceuticals, making affordable access to the drug particularly difficult for low-income countries in the absence of generic production and price competition. Under Turkey's current Industrial Property Law, patented drugs are similarly protected. While compulsory licensing—under which the state may authorize production without the patent holder's consent in cases of public interest or public health—offers a legal pathway, no such mechanism has been invoked for Zolgensma in Turkey. Access to ultra-high-cost medications such as Zolgensma remains severely restricted in other developing and less developed countries like Mexico, Brazil, and India due to high pricing, patent barriers, limited public health budgets, and inadequate reimbursement frameworks. Although some countries have mechanisms to ease patent protections through compulsory licensing, this approach does not always yield rapid or effective solutions. (P14)

On the impact of the profit-maximizing imperatives of pharmaceutical companies, she noted that access to the treatment is shaped as much by the commercial priorities of pharmaceutical capital as it is by the national budgetary constraints of Turkey, with licensing and reimbursement applications reflecting strategic rather than purely clinical considerations:

> On the Social Security Institution (SGK) side, decisions to include drugs in the reimbursement scheme may at times be shaped more by budgetary constraints than by medical or scientific criteria. However, in applications for licensing and inclusion in SGK reimbursement list, commercial concerns often play a determining role for pharmaceutical companies as well. Even when a drug has received FDA and EMA approval abroad, companies frequently do not apply for licensing and reimbursement in Turkey for all indications in which the drug is known to be effective. In some cases, companies may refrain from applying for reimbursement even after obtaining

> market authorization, and in certain instances, they may introduce the drug to the market while withdrawing their applications entirely. (P14)

Elsewhere, it was emphasized that despite the shared parental desire to ensure the best possible care and outcomes for their children, access to Zolgensma, as long as it remains contingent upon individual efforts, is largely determined by socioeconomic privilege, mobilization of networks, and digital literacy:

> Every parent wants their child to receive the best possible treatment and to live the healthiest life they can, and they do everything they can to access alternative therapies... but when you take a broader view, it becomes clear that this problem can't be solved through individual efforts alone. Because while patients and families who are well-connected, who use social media effectively, who can reach certain places—those people can access treatment... others who don't have the same position or the same luck, simply can't. (P01)

A different form of concern regarding the implications of individualized efforts to access Zolgensma was voiced by a spokesperson of another major SMA organization and non-parental caregiver, who emphasized not only the inefficiency and unsustainability of fragmented fundraising strategies, but also their broader opportunity costs. The interviewee stressed that private procurement typically occurs at full market prices, whereas public reimbursement could enable the state to negotiate lower rates, thereby allowing limited national resources to be allocated more efficiently and equitably across the wider SMA community:

> Families, sports clubs, singers, and influencers all launched campaigns... There were even suggestions that a portion of the national lottery funds should go there. We said this issue couldn't be solved this way. We didn't support these actions, and because of that, we were branded as the villains. Millions of dollars from Turkey went abroad for a single medication. Just imagine—how many hospitals, how many clinics, how many physical therapy centers could have been built with that money? If the state had included Zolgensma in reimbursement, a portion of that amount could have been used to provide gene therapy to newborns or very young patients, and many other things could have been done for other SMA patients. (P06)

NGO representatives and patient advocates also emphasize the clinical implications of the inaccessibility of Zolgensma, particularly in the context of the delays and loss

of efficiency in medical intervention that emerge as families are left to their own means to access the treatment:

> The most critical point is that, regardless of which treatment it is, early intervention yields much more successful outcomes... Families end up losing time during the fundraising process in individual campaigns... and as a result, the patient's benefit from the treatment also decreases. (P01)

Finally, multiple NGO representatives, patient advocates, and caregivers highlighted how the absence of a formal, equitable access mechanism compounds the psychological, logistical, and financial toll already borne by families (P01, P02, P03, P04, P05, P09, P10, P11, P12, P13). As one NGO representative and parental caregiver explains:

> (Families) are already dealing with the burden of a severe illness in every possible way... and on top of that, when you add the need to raise money for treatment, the inability to access treatment, and the uncertainty around the continuity of the treatment they do access, the problems become even greater. We want the treatment to be affordable without any of this process being necessary. (P01)

In the interviews, caregivers consistently articulated the inaccessibility of Zolgensma as a fundamental violation of their children's right to life, a denial of their own rights as citizens, and an exclusion from the functional reach of the state's duty of care. Recalling the challenges she has faced in securing access to Nusinersen (Spinraza) due to eligibility criteria and subsequently in obtaining legal permission for her fundraising campaign for securing access to Zolgensma, a parental caregiver and non-affiliated patient advocate notes:

> You won't give me the drug available in my own country, you won't bring in the other treatments, and you won't let me raise money and go abroad to get this drug with my own means. Isn't that basically saying my child deserves to die? In the end, my cousin in Germany said, "If it's illegal, then it's illegal—if I have to go to jail, I'll go," and launched the GoFundMe and PayPal campaign. (P04)

This sense of abandonment was echoed by multiple parental caregivers (P04, P05, P09, P10, P11, P12), who emphasized that access to treatment is not a matter of

charity but an ethical obligation grounded in their children's fundamental right to life. Indeed, one mother remarked: “This is a medication our children are entitled to... I can’t even bring myself to call it a medication—it’s their right to live, absolutely.” (P09). Likewise, a father questioned the calculus that underpins pharmaceutical inaccessibility of Zolgensma in Turkey by saying: “So, a child’s life is worth two million euros? Two million euros?” (P11) While another father reinforced the call for Zolgensma, noting its potential as a permanent therapeutic solution:

> If a research committee were to examine the children and families who have already received the treatment, they would see that it truly is a permanent solution. When a permanent treatment exists, why should we keep delaying the disease with repeated injections? (P05)

The emotional, logistical, and ethical toll of navigating such systemic inaccessibility was elaborated in detail by another mother, who described her experience of launching a fundraising campaign not only as a pursuit of treatment but as a desperate plea for recognition:

> Forget walking, eating, talking—I just wanted my son to be able to breathe next to me... I launched the campaign... I’d go on the news if I had to, I’d do this, I’d do that, I’d tear myself apart... Of course, my goal is to access gene therapy, but at the very least, I thought they’d say: “Let’s give this child Spinraza already, this mother is raising hell”. (P04)

In the same interview, she reflects on the disillusionment, exhaustion, and isolation emerging from her experience of civic abandonment in the struggle for access to Zolgensma:

> I am a tired mother... The state cannot be one that abandons us like this. I’m trying to improve my son’s quality of life... You’re supposed to support me... I’m your people, aren’t I?... That’s why you’re in charge, isn’t it? (P04)

She further articulates the painful realization that hope for her child’s health is contingent on her socioeconomic status, stating:

> Of course, I’m a mother who wants her son to talk, to walk, to run, to go to school, even to be picky about his food. You understand what I mean? If they told me right now there’s a 1% chance he could breathe with this drug, I’d

> still give it to him. I'm a mother—but in this country, I can only take that 1% chance if I have the money to do it. (P04)

The broader ethical stakes are also clearly outlined when she questions the logic of a system that demands millions from families fighting to keep their children alive:

> This tiny little piece of life has fought so hard to survive, to hold on—and I don't want families to have to spend millions on a device or a drug just to keep them going. I want my state to cover it. You get me? He needs it. (P04)

Finally, she remarks how navigating a landscape of pharmaceutical inaccessibility leaves families feeling politically abandoned, ethically cornered, and medically desperate— to the point of considering options as extreme as readily offering their child for experimental use in clinical trials:

> You know what we said at one point? "If you need test subjects, we'll be your test subjects." Letting your child be used as one... It's such a heavy, awful thing. But that's how desperate we were—we were forced into saying it. (P04)

Another mother shared how the moral and emotional toll of fundraising intersects with class and dignity, stating:

> No middle-income family could come up with sixty million today. Even if our entire family sold all our houses, we still couldn't have raised that money—unfortunately. Speaking for myself, I'm someone who couldn't even ask another person for a glass of water in my life, and yet I was made to beg. That's why I feel hurt. I'm hurt by my country. (P09)

Reflecting on both the financial and psychological toll of the fundraising process for her daughter, one father remarked:

> For a family without a steady income, it's almost impossible to raise this kind of money. You could call it a fortune. I can't even begin to describe what I went through psychologically. Let me just say this: may no one ever be tested through their child's health. Even answering this question is hard—it brings everything back. (P05)

This experience is echoed by the words of another participant, stating:

> This amount of money was impossible to gather—even if we sold everything we owned. In our daily lives, we're the kind of people who wouldn't ask a neighbor for a grain of salt. But when it came to keeping a baby alive, to making sure a mother's arms wouldn't be left empty, we tried to reach out to everyone. (P10)

A father, who had to travel to Germany to receive the gene therapy, has chosen to stay there for his child to receive consistent care and undergo necessary operations smoothly, and is currently learning German to become eligible for lifelong migration, evaluates the inaccessibility of Zolgensma in Turkish context with the level of access in Germany:

> Germany gives this drug even to refugees… Of course, that's partly because of its level of development, but… the fact that Turkey can't give it to its own children and ends up abandoning them to death—that's just heartbreaking. (P11)

Overall, although the context is complicated by inconclusive clinical evidence regarding the long-term efficacy and safety of Zolgensma, caregivers, patient advocates, and healthcare professionals consistently frame the issue as a structural problem of therapeutic access at both the individual and national levels. While financial constraints are frequently cited as a primary barrier, interviewees articulate divergent normative claims for access, invoking the right to life, social citizenship, and the obligations of the welfare state. At the same time, the interviews revealed moments of inter-organizational tension among NGOs and advocacy actors, particularly around divergent strategies for navigating access barriers, including disagreements over the ethics, sustainability, and distributive consequences of individualized fundraising, which underscore the heterogeneity of civil society responses and the difficulty of formulating unified advocacy agendas within a fragmented and emotionally charged policy landscape. As the following section will demonstrate, the absence of a formal and equitable access mechanism not only restricts treatment availability but also produces substantial clinical, economic, and psychosocial burdens for affected families in Turkey.

5.3 Social burden of caregiving: Participation, stigma, scrutiny, and the politics of visibility

For families engaging in crowdfunding to access Zolgensma, the campaign process frequently transforms into a site of public exposure, moral surveillance, reputational risk, social vulnerability, and the gradual erosion of personal and familial boundaries. While intended as a means of life-saving mobilization, campaigns often require public self-disclosure, emotional performance, and strategic appeals to visibility. This forces caregivers into a near-impossible position, where they must simultaneously assert their dignity and perform vulnerability to garner support. In a striking paradox, one mother reflects: “We finished the campaign with the people’s money. It was completed with small contributions from many people… but still, the harshest accusations and reactions we faced came from the public.” (P09)

> In response to the stigma attached to fundraising campaigns—particularly those circulated via social media, many families explicitly reject characterizations of their efforts as desperate or humiliating. Instead, they frame their campaigns as acts of agency and political resistance. The same mother poignantly asserts: “We’re not begging, we’re resisting. We never begged. This was resistance. To us, it was a story of resistance. It was our son’s story.” (P04)

Despite this reframing, caregivers consistently report being subjected to derogatory commentary, accusations of deceit, and the persistent gaze of a skeptical public. This experience was partially shaped by wider concerns regarding fraudulent fundraising, with the presence of scammers, some of whom were encountered during the research process, contributing to an atmosphere of pervasive distrust and intensified public scrutiny. One mother, reflecting on her campaign experience, recalls receiving messages such as “These people are scammers,” “Stop begging,”, “You brought this on yourselves by marrying a relative." and “Gypsy woman, enough already.” She responds with quiet defiance: “You face ignorance and hurtful accusations, but are you going to fight for your child’s life, or waste your strength on

them?" (P04) The experience of stigma and disbelief was reported to be further intensified in smaller or more conservative communities, where both public familiarity with SMA and support for crowdfunding efforts were perceived to be more limited. One father describes the compounded difficulty of campaigning in a small city like Sakarya:

> [SMA campaigns] are something people in small towns don't really care about, know about, or even believe in. They say, "Wait, the state doesn't provide the drug?" We say, "No, it doesn't." Then suddenly we're met with accusations like, "You're begging," or "You're scammers." (P11)

Beyond the stigma and scrutiny that caregivers face from the public, many also report a profound sense of institutional silence, media indifference, social abandonment, and an alarming sense of invisibility at critical moments of their campaigns. Indeed, the ability to mobilize effectively and quickly often hinges on being seen, heard, and endorsed—conditions not equally available to all. Participants attribute these differences to a range of factors, including disparities in social capital, geographic location, digital fluency, and in some cases, sheer chance. For some, the lack of media coverage or celebrity amplification significantly limited the reach, momentum, and success of their efforts. As one mother noted:

> They didn't put us on any TV programs. It was only in the newspapers, and if you were lucky, maybe a few news shows would cover it. Most celebrities didn't even want to share the campaign. You know what they say—sometimes a few seconds is your whole life. (P04)

Likewise, one father recounts a particularly disillusioning encounter with local elites and provincial authorities, describing a pattern of symbolic support without significant follow-through:

> We went to businessmen in Sakarya. We wrote to them repeatedly, tried hard, but got nothing. Then we went to the governor—he asked us for examples of similar campaigns, and we told him: Çorum, Tekirdağ, places like that. He said, okay, I'll talk to them, see how they did it. But after that? Nothing. He just used us for PR. We took a photo together, he posted it on Instagram, gave a tiny gold coin, and that was it. (P11)

By contrast, some families encountered a markedly more supportive environment, as one mother recounts: “The doors we knocked on weren’t slammed in our faces. From the district governor to the local headman, to the provincial governor—whoever we went to, they tried to help us. We weren’t turned away by anyone.” (P09) Likewise, reflecting on the factors that contributed to the success of their campaign, one father emphasized the importance of communication, trust, and familial solidarity:

> When parents are able to express themselves clearly and explain the disease to others, they can usually find some level of support from those around them. Once the right communication is established with the right people, the campaign begins to gain momentum. With a sincere and transparent campaign, it is possible to reach the goal. (P05)

As these accounts reveal, the campaign process often operates not simply as a mechanism for financial mobilization but as a socially embedded and uneven terrain, shaped by intersecting dynamics of visibility, recognition, and social perception. In the absence of a centralized, institutional access pathway that could eliminate the need for public fundraising altogether, individual campaigns remain subject to highly variable levels of support, exposure, and legitimacy. Beyond the burden of performing visibility, participants also noted the consequences of not engaging in this performance. In this case, opting out of public fundraising, whether to preserve privacy, focus on caregiving, or due to emotional fatigue, has been reported to be misread as parental negligence or a lack of urgency. An NGO representative and bereaved mother highlights how the absence of formal access channels forces caregivers into an impossible position in which they must choose between spending time with their children and fundraising for their treatment: “Parents who choose not to pursue this method [raising money through individual campaigns] so they can

spend time with and care for their children are made to look as if they aren't even seeking treatment." (P02)

Indeed, in the absence of a formal institutional support system, families are forced to reconfigure their lives in response to the demands of caregiving, often at the cost of social integration, economic stability, and emotional wellbeing. One adult SMA patient and NGO representative explains how the shock of diagnosis sets off a cascade of social restructuring for affected families:

> The diagnosis changes everything. After the initial shock, families and close relatives have to reshape their lives around what SMA demands. We frequently see divorces, abandonment, and as a result, the entire burden falling on a single parent. Parents—especially those left to care for the child alone—struggle immensely to keep working, find a job, manage the household economy, and balance their time. (P03)

These social pressures are further intensified by a quieter but equally consequential form of isolation, wherein the rhythms of daily life are reoriented around care work and protection for many families as they navigate the loss of everyday social participation. As one NGO representative and non-parental caregiver explains:

> On the psychological side, there's a burden that comes with not having a "healthy" child, not being a "normal" family. Even if that burden lessens over time, you still can't participate in society like other people. People visit you, but you don't visit them. You keep your distance when there's illness or even a risk of illness. If you have another child at home, you become overly protective, worried they might catch something and pass it to their sibling. Family, relationships, contact, weekdays, weekends... all of it has to play out differently. (P06)

As such, patterns of stigma, skepticism, and selective endorsement contribute to a broader politics of visibility in which the success of life-saving efforts becomes contingent on factors such as geographic location, media access, digital literacy, and existing networks of influence. Beyond the politics of visibility, however, the absence of a centralized and equitable access mechanism also shapes patterns of

social withdrawal, familial restructuring, and care-driven isolation, as families are forced to reorient their daily lives around the demands of treatment-seeking and long-term caregiving, often in ways that marginalize them from broader social participation.

5.4 Financial burden of caregiving: The price of hope and the geography of survival

In interviews, caregivers, patient advocates, and NGO representatives emphasized that beyond the immediate demands of treatment access, the ongoing costs of sustaining life with SMA present a substantial and often underacknowledged financial burden. All participants emphasized that the disease necessitates the acquisition and continuous maintenance of a wide range of medical technologies, support equipment, medical consumables, and services such as cough-assist machines, aspiration devices, feeding tubes, orthotic supports, wheelchairs, enteral nutrition formulas, and regular physiotherapy sessions, all of which involve recurring expenditures for families. While some of these are partially reimbursed by the state, others remain entirely uncovered. One mother detailed the gap between public reimbursement and out-of-pocket expenditure:

> All of a sudden, our lives welcomed 7 or 8 devices—like the BiPAP machine, a nasogastric tube, a cough assist machine. The state doesn't cover the cough assist device at all, and only 20% of the others, which is basically nothing. For the aspiration device alone, we go through maybe one box of consumables per day—two if the illness flares up. You pay 2,000 to 2,500 liras, and the state reimburses maybe 500 of that. (P04)

Another mother and NGO representative described how daily life becomes tethered to a complex assemblage of electricity-dependent devices, noting that their utility bills doubled due to constant machine use: "Right now, my son lives connected to six different medical devices at home, and every one of them runs on electricity. As a family, we pay twice the electricity bill that you do." (P01) One non-

parental caregiver and NGO representative clarifies that their reference to SMA as a "disease of the rich" was not a comment on its etiology or incidence, but rather on the uneven socioeconomic capacity to manage its demands and secure comprehensive care, stating:

> When you're wealthy, when you have more financial means, it's easier to cope. A government official's child gets sick, so does a soldier's, a worker's, a civil servant's, a villager's—but how you deal with the illness, how you care for your child, that differs. You need to use high-quality medical supplies, but the state only reimburses for the cheaper ones. (P06)

Yet as one parental caregiver and NGO representative points out, the financial burden of SMA care cannot be easily reduced to a binary of full state support versus total neglect. The reality lies in a fragmented middle ground, where partial reimbursements and theoretical service entitlements often fall short in practice:

> SMA is one of those situations where neither extreme is true—not like some families say, "we have no support, we can't access anything," but also not like the state claims, "we're giving you treatment, devices, and full access to physical therapy." The truth is somewhere in between. (P01)

While participants acknowledged the existence of state-sponsored supports such as home care services, disability pensions, and educational accommodations for children with chronic illnesses, many emphasized the unevenness and fragility of these provisions in practice. These services, though formally guaranteed, were frequently described as inconsistently implemented, bureaucratically fragmented, and regionally contingent.

> There are home care and education services. You can make an appointment and get support like wound care, lab services, reports, even at-home physical therapy. And yes, if you meet the income criteria, there's disability and home care allowance, too. That's the theory. But in practice? If you look province by province or city by city, you have to ask: how much can families actually access any of this? (P01)

Likewise, one father explained that this spatial inequality extends to civil society support:

> There are NGOs in Turkey and around the world that support SMA patients, but unfortunately, they cannot reach every child. Not every child is able to contact these organizations, express themselves, and ask for support. A child living in a rural village, for instance, would struggle to access these NGOs without an intermediary. (P05)

Geographic disparities in infrastructure, access, and institutional responsiveness emerged as a recurring theme in interviews, revealing how the social geography of health mediates treatment continuity and care equity in Turkey. An adult SMA patient living in Sivas described how even routine procedures surrounding SMA care and treatment required his friends to travel across provinces:

> There were times we had to go to the hospital four or five times—for the report, for the prescription, for the administration of the drug. There were families coming regularly to Sivas from Yozgat, Malatya, Maraş, and other places just to access the medication and supportive therapies. Some didn't have cars, so they had to ask others for help, pay for gas, or even take taxis between cities. Those coming from out of town definitely suffered greater financial losses because of this. (P08)

Moreover, drawing from her own relatively privileged location, one mother and NGO representative emphasized disparities in the quality and availability of municipal services across provinces in Turkey:

> Access to everything in Istanbul is much easier than it is for a patient living in Ardahan. I mean everything—from medical supply stores to even attending an accessibility expo. At this point, I think municipal governance plays a major role. For example, in my city, the municipality offers services like bathing and barber care for patients. Are these available in other cities? Are they as widespread? Are they handled as professionally? I don't know. I live in a city with relatively good accessibility, but a family in another province might not be as lucky. In some cities, there are even playgrounds with equipment that children in wheelchairs can use—but these don't exist everywhere. (P01)

The cumulative toll of these disparities is perhaps best illustrated through the longitudinal journey of one mother, whose experience encapsulates the diagnostic, logistical, and psychological strain of navigating a highly uneven care landscape from rural Turkey to the United States:

> We started when my son was just one month old. We saw seven different doctors. Finally, when we went to Erzurum University, a professor there told me, “Don’t make me say something unpleasant now. Come back in a month.” A month later, we went to a hospital in Gebze where the doctor wrote “SMA?” on the paperwork for diagnosis. Once we got the diagnosis, we went to Ankara. We decided to hand-deliver the documents to avoid them getting lost in the mail and to speed up the process. When you apply, you mail everything, and then it gets sent back for missing documents… sometimes the process would get delayed by one or even two months. We lived in Ankara for 4–5 months while my son received his maintenance doses. Then we returned to our home in Kars. We were supposed to travel to Ankara every four months for the medication. But my son got sick in Kars. A cold turned into pneumonia, and we had to go to the Erzurum Regional Training and Research Hospital. He stayed in intensive care for about three weeks. Every day, the doctor there told us, “Your child has SMA, he’ll die before age two anyway. Don’t exhaust him in intensive care. Don’t exhaust yourselves in the hospital. Take him home and spend time with him.” During that time, I kept trying to reach a well-known pediatric neurologist in Istanbul by phone. No ICU beds available. No ICU beds available. Two weeks passed. Still no ICU bed. I could only see my son for five minutes a day, and yet I left him in the ICU in Erzurum to go see the neurologist in Istanbul. When the doctors in Istanbul agreed to admit my son, I fainted from excitement—because I was going to be with him again, and I had hope he would get better. After two months in the ICU, my son was discharged and came home. We moved from Kars to Istanbul, but for a physical evaluation, we still had to go to Ankara. After we raised the required amount for Zolgensma, we went to the U.S. (P04)

As the testimonies in this section illustrate, the financial strain associated with SMA care in Turkey is compounded by the cumulative costs of medical devices, consumables, energy use, and ancillary services, many of which are only partially reimbursed or entirely uncovered by existing state mechanisms. These burdens are further exacerbated by regionally uneven access to public services, healthcare infrastructure, and municipal support, revealing significant geographic disparities in the continuity and quality of care. In the absence of a formal, equitable, and comprehensive access pathway capable of standardizing care across geographies and mitigating the influence of individual and locational privilege, factors such as socioeconomic status, household income, urban proximity, and social capital become decisive in shaping the availability, timing, continuity, and quality of treatment for

SMA-affected families. In this light, the intra-national inequalities mapped across regions of Turkey reflect and reproduce broader disparities between countries in the global pharmaceutical landscape, underscoring how the absence of institutionalized access frameworks renders both national and global health systems susceptible to exclusion, unpredictability, and unequal outcomes. This pattern is particularly salient in patient journeys in which rural families, confronted with limited treatment options, insufficient care quality, a lack of coordinated referral systems, bureaucratic delays, and medical gatekeeping, are often compelled to relocate first to relatively more developed provinces in proximity, then to major urban centers such as Ankara or Istanbul, and ultimately, for those with the means, to seek advanced and expensive therapies abroad in developed countries.

5.5 Psychological burden of caregiving: Navigating fear, hope, resilience, and identity under the weight of care

Participants describe an intense and often unrelenting transformation of the self and family life, marked by anticipatory grief, fear of loss, persistent anxiety, helplessness, emotional exhaustion, role entrapment, guilt, self-doubt, and the reconfiguration of personal, professional, and relational identities. This burden is neither static nor uniform; it unfolds differently across time, social position, and caregiving configurations, yet remains deeply embedded in the lived experience of managing a progressive and currently incurable disease, particularly in the face of systemic barriers or visible clinical deterioration. At the same time, many accounts include reflections on hope, resilience, and intimate joy, often anchored in small but meaningful milestones of progress, connection, and recognition. This layered emotional landscape is illustrated by one mother’s reflection on the process of

internal change following the diagnosis of her son, which she describes as a slow and disorienting reorientation of herself and her reality:

> Everyone wants to have a healthy child. Everyone wants a good life. No one accepts the possibility of bad things happening, and no one thinks it will happen to them. Who were you? What did you do? What made you happy? It takes time to rediscover that, to accept what's happened, and to change your life accordingly. (P09)

Her account articulates a powerful experience of role entrapment and emotional responsibility, wherein the boundaries between caregiving and selfhood become increasingly blurred:

> Because my son was diagnosed late and only received the medication after experiencing muscle loss, he's now a disabled individual and will always need support in some form. He's become an enormous responsibility for me. I've turned into a kind of mother who has to include him in everything I do. I feel like he constantly needs me. I still feel that way. Honestly, I don't really get to go anywhere without him. (P09)

This gradual reconstitution of identity is further elaborated as she articulates a transformation in her maternal role, wherein caregiving becomes a continuous, embodied negotiation between fostering autonomy and managing dependency, and parenting itself is restructured around the temporal demands, emotional investments, and repetitive labor of sustaining daily function:

> In truth, I'm trying to teach my son—who I feel I can't live without—how to live without me. That's really what my motherhood and my relationship with him are built on. My biggest dreams are for him to be able to cook his own meals, go to the bathroom on his own, get into a car, get out of a car... These are things that most people do so easily, they don't even think about them. But I think about them a lot—"Will my son be able to do them?" "Can he?"—and I put in so much effort just so he can. (P09)

Broadening the discussion of caregiving-induced transformation to include professional identity, another mother and non-affiliated patient advocate reflects:

> After my son's diagnosis, I stopped working. I was an independent woman—someone who stood on her own feet and wasn't financially dependent on anyone. But during that time, because of his illness, I hit rock bottom. I lost my self-confidence. (P04)

Her narrative also reveals how the absence of professional support and therapeutic guidance fuels an acute sense of destabilization of her moral and maternal identity, intensifying feelings of guilt, inadequacy, and self-surveillance in the face of systemic neglect:

> Because of the pandemic... I couldn't bring a physiotherapist home, I couldn't send him to rehabilitation centers. And I don't know anything myself. How am I supposed to help this child develop? Do you see what I mean?... I felt like an inadequate mother. I was constantly questioning, "Am I a good mom?" Every single moment, I was thinking about it. I tried—but was it the right way? Even while playing with my son, I kept wondering, "Can I get him to move his arm more with this game?" But at the same time, I worried: "Am I taking away the joy from it?" (P04)

This sense of self-surveillance and moral disorientation intensifies under conditions of chronic uncertainty, isolation, and a persistent fear of loss, culminating in a state of psychological depletion to a point where the clinical and technological devices designed to preserve the child's life paradoxically become sources of anxiety and emotional hypervigilance. The same mother describes a near-constant state of anticipatory grief, sustained vigilance, and emotional fatigue:

> You wear down in every way—financially, emotionally. Since the day you find out about the illness, the worry and fear... you go beyond even the fear of losing them. You start fighting other battles. Losing someone is terrible, but do you know what's worse? Living with the fear of losing them every single day. Not being able to sleep at night. When the door closes... you're alone. They say being a mother is a beautiful thing, but when your child sleeps, you stay awake, checking if he's still breathing. Even with machines hooked up, unless you've jolted awake at the sound of a beep because his heart rate spiked or his oxygen dropped, you wouldn't understand. (P04)

Several participants described the cumulative emotional toll of caregiving and campaign coordination as manifesting somatically in the form of fatigue, insomnia, chronic sleep deprivation, hormonal disruption, and lack of appetite. One father recounted how the intensity of his wife's distress led to a premature suppression of lactation:

> While my wife was taking care of our child, she was also trying to manage the social media campaign from home. I was more involved on the ground, trying to handle things in person. But it was harder for her... after all, she's a mother and felt completely powerless. She was trying to do something online. Trying to take care of her child. Because of all the stress, her milk dried up. (P11)

Amid sustained emotional exhaustion and identity destabilization, several participants underscored the regulatory function of early psychological adaptation and the mitigating impact of coordinated, multidisciplinary professional support in sustaining caregiver resilience and care continuity. One NGO representative and mother articulates this position by stating:

> When you're pregnant, you plan a future for your child—and when you receive a diagnosis, a life outside of everything you had imagined and planned begins. Your priorities shift, and a lot of things emerge that you have to fight for. The earlier you're able to accept this and adapt your life accordingly, the more it benefits your child. From learning your rights to receiving psychological support, having a social worker show up and support you through this process is incredibly valuable. Being met by a team that includes a psychologist, physiotherapist, nurse, social worker, and of course a doctor—receiving the diagnosis that way, and then continuing the process together—is truly meaningful. Believe me, it would make this experience... I don't want to call it a burden, but it would lighten the load on everyone. (P01)

Despite the recognized value of coordinated psychosocial and therapeutic support, however, multiple participants emphasized that such services often remain materially or temporally inaccessible to many families, particularly amid the temporal demands of care labor, the urgency of sustaining a medically fragile child, and the financial burdens associated with the treatment, care, and management of SMA (P01, P02, P03, P10). One mother illustrates how the relentless demands of caregiving for an infant with SMA have rendered the pursuit of psychological support logistically and financially unfeasible for her:

> Life is such that you don't even have time to seek support. While you're fighting for a baby with SMA, you don't have the time—or the money—for psychological help. That kind of luxury just doesn't exist. You have a baby you need to keep alive, racing against time. You give it everything you've got. And what's left behind is frayed nerves and a life that's completely

> upended and nearly impossible to piece back together. Can you imagine not seeing your family for months, not even being able to sit down for a single breakfast together? I couldn't do any of that for months. I wish the public watching us had really seen what my life looked like. If the state had witnessed this struggle, they couldn't have stayed indifferent. These days were so difficult, only someone who's lived through it could ever understand. (P10)

Here, the difficulty of accessing psychological support is not merely a matter of time or awareness, but also of resource allocation under scarcity. Especially among families with limited or moderate income, caregivers often face painful trade-offs between their own mental wellbeing and the child's critical medical needs. As one NGO representative and mother explains:

> Specialists' support is really important… One of the most common requests we hear from families is for psychological help: "I need psychological support, but I can't get it." And it's true—you'd have to set aside a serious budget for that. But my child also needs a new cannula every month. I have to protect them from infections. A single cannula costs around four thousand five hundred lira. And then there's the choice: spend that money on psychological support for myself or on something visibly vital for my child. Of course, we have to choose what's more visibly urgent. I'm speaking from the perspective of a middle-income family. For lower-income families, this is an even greater problem. (P01)

Psychological strain was not limited to caregivers alone. Adult SMA patients themselves offered powerful insights into the mental burdens of navigating a world that is structurally unprepared for their needs. One participant describes how even everyday activities are charged with emotional and logistical tension due to the persistent need to anticipate and mitigate inaccessibility, bodily limitations, and stigma:

> Is it psychologically difficult to live with SMA every single day? It really is. You're going out somewhere and, excuse me, you have to think about whether you'll even be able to use the restroom because sitting down and getting up is a struggle. You can't visit people. You hesitate about traveling during holidays. When you're thinking about getting married, you have to think very deeply—there's a huge fear about whether the person you want to build a life with will accept this. And you live constantly with the anxiety of, "Will my child be born with SMA? Will they not?" (P08)

Despite the pervasive emotional toll and structural pressures described above, several participants also emphasized the sustaining role of hope, particularly as it emerged through incremental signs of clinical progress, renewed connection, or restored capacities in their children.

> After the gene therapy, for ten minutes, half an hour, an hour, two hours... I could take my son into my arms, free from the tube in his nose, the PAP machine, the oximeter on his foot. He used to bite my finger—and I would send the photo of that bitten finger to my family, just to share my happiness. Now he can talk and express himself, he goes to school, he has friends. I can have conversations with my son—sometimes we even argue. If you saw him *ablası*, you wouldn't believe the things he says, he's got us all wrapped around his finger. He's picky about food, he can't give up rice. He asks his grandmother for white soup, for yogurt soup. I want everyone to experience this. (P04)

Altogether, the psychological burden associated with SMA caregiving emerges at the intersection of clinical uncertainty, structural insufficiencies, and the enduring physical, temporal, emotional demands of long-term care. While participants describe profound disruptions to personal, moral, professional, and relational identity, psychosocial stability, and sense of agency, alongside sustained emotional exhaustion, anxiety, fear, isolation, and self-surveillance, they also emphasize the stabilizing role of early psychological adaptation, coordinated professional support, and the sustaining presence of hope.

5.6 The feminization of caregiving: Gendered responsibilities and expectations

Across interviews, a recurring theme emerged regarding the gendered distribution of caregiving labor within the familial and social structures surrounding SMA care, wherein mothers and female caregivers were consistently positioned as the primary coordinators and executors of care work, often at the expense of their professional aspirations, social participation, and personal well-being. This gendered expectation was not perceived as coincidental or purely circumstantial, but rather as deeply

embedded within broader cultural scripts, socioeconomic constraints, and normative assumptions about maternal duty, emotional labor, and domestic responsibility, particularly acute within the context of the prevailing family dynamics and socioeconomic conditions in Turkey (P01, P02, P04). As one NGO representative and mother explains:

> Care responsibilities here generally fall on women... The first to leave their job, the first to withdraw from social life, the one expected to assume the overall responsibility for the patient is almost always the woman. This expectation that women should take on this role is imposed on them by society as a form of pressure. And due to family dynamics in Turkey, it's overwhelmingly assumed that everything related to the child should come from the mother. (P01)

She continues by noting that this expectation extends even to highly educated and professionally active women: "I know so many mothers who, in this situation, had to reorganize their entire lives... and had to quit their jobs." (P01)

The weight of caregiving is not experienced in isolation, but in accumulation with the multiple intersecting roles women are expected to maintain. She further articulates how the caregiving role becomes embedded within an already dense matrix of gendered social roles, stating: "None of your roles in life ever end. Right now, and even before, I've been someone's child, someone's older sister, someone's teacher, someone's neighbor. And alongside all that, as a parent, I'm also the parent of a sick child." (P01) These cumulative demands are compounded by internalized societal expectations of emotional endurance and self-sacrifice. As the same participant elaborates, caregiving mothers are often expected to remain unfailingly strong, resilient, and selfless, with limited social space to express emotional fatigue or to seek respite:

> Mothers are burdened with ideas like "I have to be a very strong mom," "I have to succeed at everything," and people get crushed under that weight. No—sometimes we need support with this, but even expressing that can be seen as selfish. Society equates a woman's role with performing miracles,

> with being extraordinarily strong. But that's not how it is. A mother of a sick child can struggle. She can stumble sometimes. She might want to create a private space for her own. She might want to be alone once in a while. (P01)

She continues by highlighting how even modest desires for solitude or ordinariness are socially interpreted as selfishness, reflecting a broader cultural denial of maternal subjectivity:

> For example, I hear this from a lot of mothers: "I just want to have an ordinary, normal day like everyone else, and in that moment, I don't want to talk about illness or disability." Defining a private space or time for yourself is sometimes seen by society as selfish. No. I might just want to go to the hairdresser like other women do. (P01)

Several accounts underscored how this burden becomes further intensified in one-parent contexts, often shaped by patterns of marital dissolution, abandonment, abuse, or preexisting family fractures, which leave women logistically, financially, physically, and emotionally isolated in their caregiving roles. One representative of an SMA-focused NGO highlights how widespread this phenomenon is among caregivers navigating SMA diagnoses:

> Divorces, abandonment, and the resulting burden falling entirely on one parent are processes we see very frequently after an SMA diagnosis. Parents—especially mothers who are left to care for their children alone—struggle immensely to keep working, to find employment, to manage the household economy, and to organize their time. (P02)

This structural dynamic is powerfully illustrated in the testimony of one mother and patient advocate who, following a separation, was left to coordinate care on her own: "You're married, you have a special needs child, and you're tearing yourself apart at home just to keep them alive. I'm fighting to give comfort to my son—not to the husband who withheld his support from us." (P04)

In dual- or single-parent households alike, caregiving responsibilities rarely extend to one child alone. Mothers described the unique emotional and logistical strain of managing the needs of both the child with SMA and their healthy siblings.

One NGO representative and mother reflects on this burden of emotional calibration and divided attention:

> And on top of it all, if you have a healthy child too, you're also expected to manage that whole process—and I think families, especially mothers, are left alone in this. How will that child define their relationship with their sibling? How do you manage time and attention between a sick child and a healthy one? Inevitably, the more visible needs of the sick child take priority, and the mother can't always engage with the other child the way she wants to. (P01)

While the emotional, temporal, and structural weight of caregiving falls disproportionately on women, several participants emphasized the critical role of extended family, peer support, and community solidarity in sustaining their caregiving capacity. Mothers who reported active involvement from grandparents, siblings, spouses, or neighbors described greater flexibility in managing care tasks, reduced isolation, and occasional opportunities to re-engage with their professional or personal lives. One mother, for instance, noted that shared responsibilities with her partner's family enabled her to travel for advocacy work and continue her professional commitments:

> Not being alone is so important… In that sense, I'm one of the very lucky women. Because I've been able to get full support from my husband, my own family, and my in-laws, I can carry out this work today… I can keep working, manage a foundation, travel to other cities, and be present in places even if it means leaving my child behind.(P01)

Whereas others pointed to the emotional and logistical relief provided by informal peer groups, with another mother stating:

> We had a group we formed with other SMA mothers—I'll never forget their support… While we were running the campaign, every day, a mother from the group would message saying she had lost her baby. Some mothers stayed in the group to share their experiences, others couldn't bear it and left. (P04)

Still, such support networks were described as unevenly available and often contingent upon social class, geography, or pre-existing familial dynamics, reinforcing the need for formal and widely accessible institutional mechanisms of psychosocial support for caregiver relief and solidarity. Taken together, these

narratives illustrate a structurally gendered stratification of responsibility in the context of SMA care, wherein women disproportionately absorb the physical, emotional, and logistical demands of caregiving, often with minimal institutional recognition or reprieve.

5.7 The "miracle drug" narrative: Hope, hype, and the politics of therapeutic promise

Interviews illustrated a spectrum of perspectives regarding Zolgensma's clinical value and long-term efficacy, ranging from strong confidence in its therapeutic potential to critical skepticism about its designation as a definitive or superior treatment option. While widely recognized for its innovative potential, the long-term efficacy, safety profile, and optimal use cases of Zolgensma were frequently described as uncertain, with several participants cautioning against its characterization as a curative or "miracle" intervention in the absence of conclusive longitudinal data. A pediatric neurologist specializing in rare diseases emphasized the current limitations in the evidence base surrounding Zolgensma, noting the heterogeneity of patient responses and the evolving nature of clinical knowledge:

> If you ask whether Nusinersen is completely ineffective and gene therapy is incredibly effective, the truth is that, the scientific evidence is only just starting to emerge. The current data is constantly being updated... We have a group of patients who received gene therapy and are doing very well without having taken Nusinersen... But there are also patients who initially said, "I've had gene therapy, I don't need anything else," and then later started experiencing symptoms again or showed halted motor function progress, which led them to request Nusinersen. And on the other side, there's also a group of patients on Nusinersen who haven't been doing too badly either. As for Zolgensma, none of these are definitive cure therapies yet—we simply don't have enough accumulated knowledge to say whether they are or aren't curative. It's a treatment with potential side effects, one we estimate to be effective in about half of cases, and from what we've seen, it may also lose effectiveness over time. Even on its own website, it says "no cure," so I think it's a topic open to debate. (P07)

She adds that gene therapy could be considered case-by-case depending on patient profile and clinical response:

My personal opinion is, if a patient is doing very well on Nusinersen and has the financial means, sure, they can still go and get gene therapy if they can afford it. But (it is when) they're not doing well on Nusinersen, that gene therapy should be considered as a chance worth taking. (P07)

While clinicians emphasized individualized clinical judgment, patient advocates and NGO representatives raised concerns about the ethical, structural, and discursive implications of how Zolgensma is marketed, represented, and circulated as a "miracle drug" within both public discourse, noting its potential to obscure scientific uncertainty, reinforce unrealistic expectations, and legitimize exclusionary pricing points. One NGO representative and non-parental caregiver articulated this critique explicitly:

> One of the 25 patients enrolled in Zolgensma's clinical trials for SMA Type 2 was the child of one of our association's founders. I don't think it's appropriate to use the word "rescue" for this treatment—it reinforces the myth of a miracle drug. Real-world data on Zolgensma hasn't yet been sufficiently published. The available results are mostly based on children who received gene therapy while they were still presymptomatic and then received excellent follow-up care. We believe the global outcomes of this therapy are not as strong as they're made out to be, but there's still no adequate report or systematic data. That will take many years to emerge. (P06)

Reflecting on the structural power dynamics shaping pharmaceutical access, the same participant criticized the emotional and financial asymmetries between patient families and stakeholders in control of access and pricing:

> Capitalism is brutal, and resources are limited. While patient families approach the issue emotionally, neither the state nor the pharmaceutical companies do. Even when the initial data was insufficient to prove benefits or investigate side effects, the pharmaceutical company was able to say, "I have a drug—take it or leave it." When a drug trial is conducted, even the format of the results can be influenced by the company. We see pharmaceutical companies as more dangerous than weapons manufacturers. (P06)

Overall, the framing of Zolgensma as a "miracle drug" operates discursively to defer critical scrutiny of its evolving safety and efficacy profile, the scarcity and

opacity of longitudinal clinical data, and its exceptionally high price point, thereby obscuring the structural dynamics that shape both therapeutic access and evidence production. These accounts also reveal a structural paradox wherein pharmaceutical manufacturers rely on post-marketing data to substantiate long-term efficacy and safety yet, set pricing thresholds so high that access is limited to a small subset of patients. The result is a feedback loop that limits participation, reinforces clinical uncertainty, and shifts both financial and medical risk onto families, particularly those with limited resources and urgent therapeutic needs.

These narratives collectively demonstrate the compounded structural, economic, psycho-social, and ethical burdens that characterize the lived realities of SMA in Turkey, as articulated across interrelated domains of therapeutic access, caregiving labor, and the precarious conditions of daily survival. Within this landscape of therapeutic inaccessibility, systemic insufficiency, and structural marginalization, participants articulated a shared set of demands, including universal and equitable access to all currently approved SMA treatments; expedited special judicial mechanisms, greater public awareness and the expansion of premarital and newborn screening; broader availability of supportive services, particularly physiotherapy; reduced bureaucratic delays and inconsistencies in accessing existing treatments and services; comprehensive reimbursement for medical equipment and consumables; improved opportunities for the social, educational, and professional inclusion of patients, as well as sustained psycho-social support for caregivers and families. What emerges, then, is a demand for systemic transformation for the implementation of an ethical healthcare infrastructure capable of sustaining life and dignity for all patients in need, grounded in the situated knowledge of those most affected, whose everyday labor and struggle continue to compensate for, expose, and

unsettle the enduring failures of institutional provision and global pharmaceutical capitalism

CHAPTER 6

GLOBAL REGIMES, NATIONAL POLICIES, AND EVERYDAY STRUGGLES: AN INTEGRATED ANALYSIS

6.1 Exporting innovation, importing inequality: Zolgensma, global IP, and pharmaceutical access for the peripheral patient

Empirical patterns from Turkey reveal how access to Zolgensma is either contingent upon a country's capacity to authorize, finance, and institutionalize high-cost therapies, or upon the individual capacities of affected families to substitute the institutional provision of the treatment through the use of their private financial means, social capital, and digital mobilization capabilities. In both cases, access remains conditioned by positionalities—be it the global economic location of the state or the relative privilege of the individual—along existing structural hierarchies. The findings collectively demonstrate that, due to the absence of formal, timely, and equitable access pathways in the Turkish context, SMA functions as a vector through which existing structural inequalities are exacerbated, as burdens and opportunities related to diagnosis, treatment access, supportive care, conditions of caregiving, and patient outcomes are disproportionately distributed along intersecting lines of socioeconomic class, gender, geography, and global development hierarchies. Indeed, across interviews, the most frequently cited burdens included diagnostic delays, lack of or delayed access to high-cost therapies, limited availability of supportive care, sustained financial strain, emotional and psychological exhaustion, feminization of care labour, social stigma, and regionally uneven access to essential medical services, all of which were consistently reported to be compounded by intersecting vulnerabilities such as lower socioeconomic status, limited educational resources,

weaker social networks, single parent household configurations, gendered expectations around caregiving, and residence in underserved or rural areas. Likewise, the most consistently prioritized opportunities, such as early diagnosis, timely and optimal treatment access, continuity of supportive care, and coordinated multidisciplinary support for patients and caregivers, were reported to be disproportionately more accessible to families with higher socioeconomic status, closer geographic proximity to major medical centers, and stronger institutional or social capital.

These findings reflect, resonate with, and extend key insights documented in epidemiological, economic, and policy-oriented research on spinal muscular atrophy (SMA) in Turkey, by elucidating how structural dynamics of prevalence, healthcare financing, institutional access, and the privatization of financial burden converge in the lived experiences of affected families. First, the extraordinary annual cost of SMA management, driven overwhelmingly by medication, which was quantitatively found to constitute over 99% of the total expenditure per patient (Öztürk et al., 2023), was consistently reflected in interview accounts of partial reimbursement, high out-of-pocket spending on medical devices, consumables, and services, along with resource-intensive home care. Here, the reimbursement landscape, described in institutional documents as stratified and incomplete (Republic of Turkey Ministry of Health, 2023a, 2023b), was experienced by participants as a source of uncertainty, perceived injustice, and structural exclusion, particularly with regard to the inaccessibility of Zolgensma, the inconsistent application of eligibility criteria for Spinraza (abolished in 2022), and the limited reimbursement of supportive devices, medical consumables, and care services. Moreover, Turkey's relatively high spinal muscular atrophy (SMA) carrier frequency and above-average disease prevalence

(Özdemir et al., 2022; Ünver et al., 2024) align with ethical and fiscal concerns raised by stakeholders, who questioned the feasibility of publicly financing ultra-high-cost therapies such as Zolgensma, particularly given the intersection of substantial per-patient expenditure with a comparatively large eligible population under the constraints of a developing healthcare economy. These concerns were situated within a broader calculus of fiscal prioritization and opportunity cost, wherein stakeholders emphasized the comparative utility of allocating limited resources toward broader public health needs across disease areas, and the potential to address a wider spectrum of unmet needs within the SMA community itself through more distributive and sustainable investments.

Moreover, reflecting the broader tensions documented in the literature surrounding the clinical ambiguity, pricing opacity, and cost-effectiveness of Zolgensma (Chen, 2020; Craddy et al., 2023; Nuijten, 2022; Thielen et al., 2022), participants presented various articulations of the treatment. For some, it represented a life-saving intervention to which all children should have an inherent right, while others framed it as a critical option in select, case-specific scenarios where optimal outcomes might otherwise be compromised. Many emphasized its prohibitively high cost, highlighting the fiscal strain it imposes on national solidarity and the difficult trade-offs it poses in resource allocation. Still others, resonating with the official position of the Turkish government, characterized it as an experimental therapy, insufficiently supported by robust evidence, that risks positioning vulnerable children as subjects of clinical uncertainty. In particular, the multiplicity of these articulations illustrates how pharmaceutical innovation, when decoupled from equitable access pathways and clinical transparency, generates competing imaginaries of therapeutic hope, entitlement, exclusion, and ethical ambiguity surrounding high-cost therapies

like Zolgensma. Consequently, the scarcity of real-world efficacy and safety data becomes both a justification for restricted access and a byproduct of that very restriction, forming a self-reinforcing cycle of epistemic and material exclusion that not only undermines clinical evaluation but also sustains the inaccessibility of high-cost therapies like Zolgensma beyond the fiscal reach of many national health systems in developing and less-developed contexts, as well as the private means of the global poor.

These dynamics are both emblematic of and intensified by Zolgensma's designation as an orphan drug, a regulatory status that encapsulates a constellation of structural conditions typically associated with rare disease therapies, including the inherently limited market size, elevated costs of research and development, complex and resource-intensive manufacturing processes, extended regulatory timelines, and heightened levels of clinical and financial risk (Gammie et al., 2015). The inaccessibility of Zolgensma in Turkey, despite active demand, clinical need, and the existence of supporting regulatory pathways illustrates that, while regulatory mechanisms such as market authorization, market exclusivity, special grants, tax credits, and accelerated approval have successfully incentivized investment in rare disease research and contributed to the expansion of a profitable and rapidly growing global orphan drug market (Gammie et al., 2015; Luzzatto et al., 2018; Melnikova, 2012), this incentivization has not necessarily translated into improved access for patients outside advanced economies or beyond privileged socioeconomic classes.

Thus, the Turkish case offers a situated empirical extension of what Moon et al. (2012) have termed "neglected populations," positioning SMA patients as a paradigmatic example within the domain of rare genetic conditions who, by virtue of their socioeconomic location and structural position within the global political

economy, remain systematically excluded from the benefits of pharmaceutical innovation. Under such conditions, where the cost of treatment cannot be absorbed either through the structural position of states within the global political economy or the socioeconomic status of individual households, pharmaceutical access becomes increasingly conditioned by patients' positionality within the commercial lifecycle of the drug, including participation in clinical trials, early access programs, or post-marketing surveillance initiatives. Therapeutic eligibility under such conditions is shaped less by clinical urgency or epidemiological burden and more by either the capacity of states or individuals to absorb exorbitant treatment costs through public reimbursement or out-of-pocket expenses, or by the strategic value of patients within the production, validation, and circulation of biomedical knowledge and capital, as trial participants, data sources, or symbolic beneficiaries aligned with corporate imperatives of pharmaceutical innovation and market expansion.

As such, the case of access to Zolgensma in Turkey exemplifies a broader pattern of extraction within the capitalist world-system, wherein substantial amounts of capital, through both direct financial burdens on families and strained healthcare systems, and critical clinical data generated by patients in peripheral and semi-peripheral contexts, funneling into transnational corporations and the economic elites of core economies. As the generated profit accrues predominantly to the core, patients race against time, either to gather sufficient funds through private means or to secure spots in rare clinical trials, early access programs, and other highly limited pathways to treatment, despite the often inconclusive data regarding the clinical safety and efficacy of these therapies. Although these communities bear 90% of the global disease burden (Coates et al., 2021), treatments remain out of reach, and even when the clinical data provided by patients from these communities is a critical input

for the development, approval, and profitability of such treatments, their participation rarely translates into tangible benefits for their communities back home.

Alongside the capitalization on disease-specific urgency, patient vulnerability, parental desperation, and therapeutic hope, the monopolistic pricing practices surrounding Zolgensma, despite inconclusive evidence regarding its long-term safety and therapeutic efficacy, are sustained and legitimized by the structural protections guaranteed by the TRIPS-based intellectual property regime. On one hand, as demonstrated by the critical literature review conducted in this thesis, despite the inconclusive and variable nature of empirical evidence regarding the impact of stringent intellectual property regimes on pharmaceutical innovation, socioeconomic development, and access to medicines, at best, the TRIPS-based intellectual property regime allows developing and less-developed countries to participate in a global framework whose primary benefits accrue to advanced economies and transnational capital. At worst, it externalizes the systemic burdens of this regime onto these states, which disproportionately bear its adverse consequences while remaining structurally constrained in their capacity to realize its purported benefits. Under these conditions, the case at hand extends DeCamp's (2007) argument that intellectual property regimes generate significant distributive effects by shaping unequal access to knowledge and knowledge-intensive goods, and lends empirical support to critical perspectives on global pharmaceutical governance that reveal how the TRIP-based IP regime systematically withholds essential treatments from those who most urgently need them, thereby sustaining the very disease burdens they claim to alleviate (see Moon et al., 2012; Pogge, 2023; Sell, 2007; Sonderholm, 2010; 't Hoen, 2009; 't Hoen et al., 2011; Timmerman & van den Belt, 2013).

On the other hand, as the broader historical context reviewed in this thesis illustrates, the formation, negotiation, expansion, and global enforcement of the TRIPS-based intellectual property regime have been shaped under asymmetrical power relations, whereby advanced economies have leveraged both soft and hard diplomatic tools to promote an expansive patent agenda that prioritized the interests of advanced economies and patent-intensive transnational capital over public health imperatives, equitable access to medicines, and the developmental needs of the rest of the world. Likewise, as evidenced by the legal landscape reflected in interviews, ministry documents, institutional reports, and secondary literature, while flexibilities were formally instituted into the agreement to mitigate these imbalances and enable countries to safeguard public health, their application has remained limited, fragmented, and politically fraught in practice (Correa, 2011; Deere-Birbeck, 2010; ’t Hoen et al., 2018). Within this context, the inaccessibility of Zolgensma in Turkey signals how developing and less-developed countries operating within the constraints of the TRIPS-based intellectual property regime may find themselves unable to secure access to essential therapies even in the presence of significant public demand and clinical justification, as they often lack the political leverage, institutional capacity, and economic autonomy necessary to negotiate equitable pricing, invoke TRIPS flexibilities, or resist TRIPS-plus obligations, leaving them financially, legally, diplomatically, and structurally constrained in their ability to prioritize the public health needs of their citizens.

When examined through the lens of World-Systems Theory (WST), the semi-peripheral positionality of Turkey within the global capitalist system, as conceptualized by Wallerstein (1974, 1974/2011), offers a critical framework for understanding the inaccessibility of ultra-expensive, knowledge-intensive, patent-

protected pharmaceuticals such as Zolgensma. Turkey's semi-peripheral status within the capitalist world-system, characterized by limited bargaining power, uneven development, and dependency on core countries for high-tech goods, knowledge-intensive products, capital, and knowledge, results in constrained economic autonomy, a reliance on alignment with core countries for economic and political benefits, restricted capacity for research and development, inability to negotiate favorable global pricing, and limited power to invoke flexibilities or resist pressures from core states and transnational capital.

First, Turkey's economic dependency on core countries for high-value-added goods, capital, and knowledge limits its capacity to finance, develop, and produce alternatives to high-tech ultra-expensive therapies domestically, which places the onus on foreign pharmaceutical companies, often driven by transnational capital in core countries, to supply such drugs at exorbitant prices. With limited capacity to produce knowledge-intensive, high-tech pharmaceutical products, Turkey is either forced to absorb the high costs of cutting-edge treatments priced and patented according to the profit-maximization objectives aligned with the interests of core economies and global capital, or leave the public demand unaddressed and displace the financial burden of procurement downward onto patients and families, many of whom must rely on private fundraising, informal networks, or strategic alignment with the drug's commercial lifecycle to secure treatment.

The review of Turkey's pharmaceutical policy and industry dynamics in this thesis further demonstrates this structural dependence of the Turkish pharmaceutical industry on foreign technology, capital, and imported biochemicals has facilitated the entry of transnational capital, marginalized domestic production, and diminished the capacity for independent policy-making that could prioritize public health over the

imperatives of global capital, thereby consolidating the influence of multinational corporations in shaping pharmaceutical policies and reducing the ability of the nation to resist the implementation of TRIPS and TRIPS-plus provisions (see Eren-Vural, 2007a; 2007b). This dependency not only undermined the structural organization and political capacity of the domestic pharmaceutical industry, both of which were integral for the development of an independent pharmaceutical policy prioritizing public health needs over the interests of core countries and transnational capital, but also contributed to the emergence of transnational capital as a key ally for a significant portion of domestic producers, thereby incentivizing a strategic shift towards export-oriented production for incrementally modified versions of existing medicines targeting lucrative disease areas (see Eren-Vural, 2007a; 2007b; 2013). Overall, the inaccessibility of Zolgensma in Turkey not only marks an insufficient domestic capacity to reimburse high-tech, knowledge-intensive, ultra-expensive, patent-driven pharmaceuticals or develop their alternatives, but also reflects an inability to challenge the very terms that govern access, pricing, and distribution of such pharmaceuticals, thereby subordinating Turkey's public health priorities and developmental autonomy to the interests of core economies and transnational capital.

Beyond economic dependency, Turkey’s semi-peripheral position within the capitalist world-system compounds this vulnerability through limiting its bargaining power vis-à-vis the core countries and the transnational capital, particularly in sectors, markets, industries, and platforms dominated by core countries and transnational corporations. Such asymmetry in bargaining capacity appears to have exacerbated access to knowledge-intensive, high-tech, ultra-expensive medicines such as Zolgensma in Turkey through impeding the capacity of the Turkish state to (1) secure more favorable and equitable pricing, distribution, and terms of access in

negotiations with global capital, and (2) resist the systemic pressures from core countries and transnational capital in forums governing terms of pharmaceutical pricing, trade, distribution, access, and patenting. For instance, as the broader historical context reviewed in this thesis illustrates, the limited capacity of Turkey, alongside many other semi-peripheral and most other peripheral economies, to resist against external pressures such as the imposition of trade sanctions, penalties for non-compliance, threats of revoking preferential trade access, economic concessions, diplomatic leverage, and ideational persuasion, have played a significant role in compelling the global adoption of and compliance with TRIPS and TRIPS-plus provisions. Thus, Turkey's semi-peripherality not only restricts its ability to negotiate favorable terms directly with transnational capital but also significantly impairs its capacity to push for equitable policies within global forums governing pharmaceutical access, ultimately contributing to the persistence of pricing logics that keep high-cost products like Zolgensma beyond the reach of its economic capacity and its population.

Finally, albeit variably pursued, Turkey's semi-peripherality has also motivated a strategic alignment with the interests of core countries and transnational capital for the pursuit of economic, political, developmental, and diplomatic benefits throughout its recent trajectory. A key example of this dynamic is Turkey's aspiration for EU accession, which, as noted by Moore & Dannreuther (2009), has often been framed as the zenith of its efforts for upward mobility in transitioning towards core status. As evidenced by Eren-Vural (2013) and Semin and Güldal (2008), the prospect of EU accession has catalyzed domestic policy reforms in Turkey, driving the alignment of its intellectual property policies with EU standards at an earlier, accelerated, more expansive, and more stringent pace than mandated by the TRIPS

Agreement. The prioritization of alignment with EU standards over the preservation of domestic pharmaceutical policies, which could protect the interests of local producers and the public, has reinforced Turkey's vulnerability to global capital, leading to the subordination of its citizens' healthcare needs to the profit-driven objectives of core economies and multinational pharmaceutical corporations.

Echoing analyses of internal peripheries within nation-states (Eriksson et al., 2016), these macro-level vulnerabilities are further compounded by internal inequalities that mirror the center–periphery logics of the world-system, reappearing within the national terrain of the semi-periphery as uneven geographies of access and exclusion. As the empirical findings illustrate, disparities in access to diagnosis, treatment, and supportive care were most pronounced across lines of socioeconomic status, gender, and regional location, with families residing in rural or underserved provinces frequently reporting delayed diagnosis, fewer treatment options, limited access to specialized care, and increased reliance on informal networks and personal fundraising. Within this nested core–periphery structure, internal flows of patients, financial resources, embodied labor, and medical data move disproportionately from rural and underserved regions toward metropolitan hubs such as Istanbul, Ankara, and Izmir, where displaced patients receive care, both fundraised and state resources are concentrated, clinical cases are evaluated by central institutions, and organized advocacy is predominantly situated. This internal stratification may reflect how semi-peripheral states, constrained by limited resources, external dependency, and pressures to demonstrate alignment with core norms, tend to concentrate infrastructural development, biomedical innovation, and institutional capacity in select urban regions, thereby reproducing global hierarchies at the national and regional scales.

Drawing on Wallerstein (1974, 1974/2011), the inaccessibility of Zolgensma in Turkey can be conceptualized as a manifestation of the complex interplay between domestic structural vulnerabilities, external geopolitical pressures, and the strategic imperatives emblematic of its semi-peripheral positioning within the contemporary-capitalist world-system. Together, Turkey's reliance on core countries for high-tech goods, capital, and knowledge, its limited bargaining power with core countries and transnational capital, its vulnerability to external pressures from the core, and its strategic alignment with core economies have significantly impaired its capacity to finance and produce alternatives to high-cost therapies, negotiate for more favorable terms, and challenge the very terms underlying the monopolistic pricing of high-technology, patent-driven, knowledge-intensive, ultra-expensive pharmaceuticals. Neither entirely impaired by international norms nor free to set them, neither fully dependent in its economic decisions nor completely free from the dictates of global capital, Turkey remains, in significant ways, subordinate to the influence and dominance of core states and global capital. As a result, it is left to navigate a delicate balance between conforming to the interests of the core and global capital, pursuing its domestic developmental objectives, and addressing the healthcare needs of its citizens. This structural dilemma is further illustrated by the legal landscape explored in the interviews, wherein Turkey’s simultaneous formal commitment to international legal standards on the right to health and social security, along with its constitutional guarantees to its citizens, on one hand, and its compliance with the TRIPS-based IP regime, fiscal constraints, and national priorities on the other, create an impasse at the intersection of domestic law, international obligations, limited resources, and sustained public demand, producing stark disparities between de jure rights and de facto access to ultra-expensive pharmaceuticals like Zolgensma.

In this regard, the examined case offers a situated lens into how semi-peripherality is lived, materialized, and embodied through fragmented infrastructures of care, the precarity of therapeutic access, and the individualized burden of pharmaceutical procurement. The dynamics identified here may resonate across other semi-peripheral and peripheral contexts that, to varying degrees, navigate structural resource constraints, limited bargaining capacity, and economic dependency within the global political economy. In such settings, access to high-cost therapies is shaped less by clinical urgency than by structural positionality, where the capacity to authorize, fund, negotiate for, or mobilize access to life-saving treatments becomes a function of socioeconomic positionality, at both the individual and systemic levels.

6.2 Care work as containment: Gender, labor, and the management of pharmaceutical abandonment

Across interviews, caregivers recounted how they became the *de facto* coordinators of care through daily practices such as administering respiratory support and suctioning, managing feeding regimes, conducting daily physiotherapy and range-of-motion exercises, sourcing and maintaining specialized medical equipment, tracking and restocking consumables, translating clinical documents, navigating opaque bureaucratic procedures, and orchestrating high-stakes fundraising campaigns for inaccessible treatments. In this context, care work is spatio-temporally expansive, extending across and beyond conventional boundaries of time and space as it reorders the temporal rhythms of daily life and reconfigures domestic, institutional, and digital environments in accordance with the urgent, continuous, and often unpredictable demands of sustaining life under conditions of medical precarity and institutional insufficiency. Reflecting what Lynch (2021) describes as the

fundamental mismatch between the rigid linearity of "clock-time" under capitalism and the cyclical, open-ended "timescape" of care, the temporality occupied by caregiving is shaped by cycles of medication, emergencies, clinical monitoring, and bodily needs, collapsing the distinction between day and night, weekday and weekend, labor and rest. Families reported recalibrating their daily lives around the temporal rhythms of care work, transforming kitchens into sterilization zones, living rooms into physiotherapy units, and online platforms into campaign headquarters.

In parallel, caregivers found themselves assuming a wide range of responsibilities typically carried out by trained professionals, acting simultaneously as informal clinicians, physiotherapists, bureaucratic intermediaries, translators, medical supply coordinators, and public campaign organizers. Accordingly, care work is irreducibly embodied, sustained through the continuous physical presence, stamina, health, and functional capacity to perform routine labor-intensive tasks, necessitating a body that is not only present but constantly operational in its capacity to lift, carry, move, adjust, respond, watch over, and remain alert across prolonged periods of physical and emotional strain. It is also profoundly affective and relational, grounded in an intimate and often asymmetrical interdependence between caregiver and patient, demanding sustained emotional attunement to the needs of the patient as caregivers navigate cycles of anticipatory grief, vigilance, fear, love, and guilt. Finally, aligned with critiques of how care work is abjected due to its intimate proximity to bodily vulnerability, decay, and dependency (Banerjee & Rewegan, 2017; Lynch, 2021), care labor in this context involves repetitive, physically demanding tasks that are high-stakes in their consequences, encompassing the daily management of bodily fluids, medical waste, tracheal suctioning, wound cleaning,

tube feeding, and infection control performed within strictly privatized and depoliticized domestic spaces.

Echoing longstanding critiques of how the capitalist world-system simultaneously depends on and marginalizes care labor to maintain the conditions of its own reproduction (Federici, 2012; Patel & Moore, 2018), these practices, while deeply affective, relational, embodied, spatio-temporally unbounded, "dirty", and frequently learned through necessity rather than design, accumulate into a form of survival labor that sustains life in the gaps left by fractured state support and a global pharmaceutical system in which essential treatments remain materially out of reach. Drawing from interview data, it is evident that institutional mechanisms of support, such as partial reimbursement for medical supplies, limited home care services, disability pensions, caregiver allowances, premarital or newborn screening, and restricted access to treatments are nominally available in the Turkish context. However, their accessibility is frequently undermined by fragmented implementation, bureaucratic opacity, limited resource allocation, and pronounced disparities across regions and socioeconomic groups. Moreover, even when such provisions are secured, they often fall short of meeting the clinically recommended frequency, intensity, or quality of care required to sustain optimal health outcomes. In the absence of coordinated, consistent, and equitable state provision and formal access pathways to essential therapies, families are compelled to absorb the logistical, emotional, and clinical burdens displaced by the state and the global pharmaceutical regime, in order to mitigate, to the best of their capacity, the potential harms produced by delayed, denied, inadequate, unreliable, and partial access to treatment and care.

Within this context, the burden of care labor emerges, in part, from the state's constrained capacity, limited reach, and partial retreat from its responsibility to provide coordinated, equitable, consistent, high-quality, and adequately resourced care and treatment, resulting in the systematic displacement of clinical, logistical, emotional, and financial responsibilities onto individual families. This displacement at the level of the state is further compounded by a global pharmaceutical regime whose reliance on market exclusivity, monopolistic pricing, and profit-oriented distribution logics shifts the burden of therapeutic access onto individual households, informal networks, and national health systems already operating under resource constraints. Here, in line with critical analyses of neoliberal capitalism as a system that systematically externalizes the costs of social reproduction, privatizes survival, and renders care labor invisible, uncompensated, and structurally unsupported (Federici, 2012; Fraser, 2016; Müller, 2019), caregivers do not have the luxury of operating at the margins of formal provision. Instead, they are compelled to actively substitute for its failures, absorb its absences, and compensate for its fragmentation, sustaining therapeutic continuity through informal, inadequately compensated, and structurally unacknowledged means.

Indeed, within the context of SMA caregiving in Turkey, care labor is marked by a paradox of visibility. On one hand, it is made culturally legible through dominant narratives that reduce it to an innate expression of maternal devotion, familial obligation, gendered responsibility, moral duty, and the unfolding of destiny. At the same time, it is rendered hyper-visible as public performance through fundraising campaigns, media appearances, and social media appeals, where strategic displays of suffering, devotion, and moral urgency become necessary for mobilizing financial and social support in the absence of sufficient institutional provision. At the

same time, and reflecting broader critiques of how care labor is systematically excluded from institutional recognition, economic compensation, and structural support due to the expendability and interchangeability of life under the capitalist world-system (Federici, 2012; Ferrarese, 2017; Fraser, 2016; Lynch, 2021; Patel & Moore, 2018), the material, emotional, logistical, and clinical labor that sustains caregiving remains structurally invisible, left unrecognized and undercompensated, while being routinely subsumed under normative expectations of kinship and gender. This simultaneous hyper-visibility and structural invisibility renders care labor both indispensable and disposable: celebrated as affective spectacle, yet consistently overlooked in policy, privatized in responsibility, depoliticized through sentimentality, and disregarded in its role as a form of compensatory survival labor.

Here, care labor is organized along profoundly gendered lines, with caregiving roles overwhelmingly assumed by mothers, who take on the bulk of physical, emotional, bureaucratic, and clinical responsibilities. Lending empirical weight to critiques that unpaid caregiving interrupts the monetizable labor time recognized within the capitalist world-system (Bryson, 2007; Lynch, 2021), mothers in this study most frequently described quitting their jobs and assuming primary responsibility for orchestrating treatment logistics, administering daily medical care, navigating institutional barriers, managing the household, attending to the needs of siblings without medical diagnoses, and sustaining the emotional continuity of the family. In parallel, fathers were often reported to engage in income-generating activities, conduct field-based fundraising, liaise with external actors, and oversee the sale of family assets in efforts to mobilize financial support. This division of labor reflects and reproduces the structural feminization of care, wherein caregiving is socially, institutionally, and culturally configured as women's work, thereby further

privatizing it into the domestic sphere, expected by default, devalued as labor, and naturalized through gendered ideologies of maternal sacrifice and resilience. Across the interviews, mothers frequently described a profound erosion of selfhood, as the boundaries between caregiving, motherhood, womanhood, and personhood collapsed into each other, at times clashing, at times coalescing, leaving little space for their individual identity. Echoing Bryson's (2007) account of how caregivers are often left without "free" time, personal space, or even the cognitive-affective capacity to attend to their own needs, this collapse of roles was frequently accompanied by an acute sense of spatial, temporal, and cognitive saturation, wherein the all-consuming nature of caregiving left no part of daily life untouched, unclaimed, or unstructured by its demands. These dynamics were exacerbated in single-mother households, where the absence of a second caregiver magnified the emotional, logistical, physical, and financial pressures of care, concentrating the totality of responsibility onto one person, with limited avenues for redistribution, respite, and support.

Despite all, the very practices of care that emerge in response to abandonment can constitute a situated form of resistance, unfolding from the margins, sometimes in direct opposition to the system, and at others in defiance of its absences, fragmentations, disparities, and inadequacies. In resonance with Marxist feminist calls to reimagine care as a form of commoning and collective resistance (Federici, 2019; Lynch, 2021; Oksala, 2016), caregivers described mobilizing informal networks of solidarity, sharing resources, exchanging medical knowledge, and mentoring newly diagnosed families. Rather than waiting indefinitely for institutional acknowledgment and provision, families built parallel systems of support, sustaining life, sharing new forms of knowledges, and asserting their agency, presence, and value within systems that have excluded, marginalized, or

failed to accommodate them. Alongside these generative practices, caregivers also engaged in direct and explicitly political forms of resistance, articulating claims to therapeutic access, state accountability, their children's right to life, and their own entitlements to care as citizens through collective mobilization, public demonstrations, organized campaigns, and formal appeals to state institutions. These efforts were described in interviews as instrumental in securing the public reimbursement of Nusinersen, which was widely framed by caregivers as a hard-won victory achieved through persistent advocacy and collective pressure. However, resistance remains ongoing, particularly in relation to the inclusion of Zolgensma in public coverage, as well as broader demands for the reimbursement of essential consumables, supportive devices, and care services that remain financially and logistically inaccessible for many families.

The empirical dynamics examined in this section illustrate that care labor, as it unfolds in the context of SMA caregiving in Turkey, cannot be understood as a mere extension of familial devotion, domestic obligation, maternal instinct, cultural custom, personal sacrifice, moral responsibility, or the unfolding of destiny. Instead, care labor emerges as a structurally embedded, embodied, affective, spatio-temporally unbounded, and gendered form of survival work that operates at the intersection of systemic abandonment and intimate responsibility, wherein families are compelled to take on the coordination and execution of essential care tasks in response to the fragmented, delayed, and uneven provision of treatment and care. As such, the burdens taken on by families should be situated within broader processes of structural displacement, wherein the responsibilities of care are offloaded from a state apparatus marked by constrained capacity, limited reach, uneven provision, and

partial retreat, as well as from a global pharmaceutical regime shaped by profit-maximizing imperatives and restrictive intellectual property frameworks.

6.3 Nested peripheries: Bridging care labor with World-System Theory

By linking the microcosm of intimate caregiving practices to the macrocosm of the capitalist world-system, this analysis demonstrates how gendered survival labor undertaken in response to pharmaceutical abandonment becomes embedded within broader circuits of capital accumulation, intellectual property regimes, and geopolitical stratification, rendering the domestic sphere a critical site for the manifestation, negotiation, and potential contestation of systemic disparities in pharmaceutical access. A closer examination of the inaccessibility of Zolgensma in the Turkish context, through the intersecting lenses of Marxist feminist analyses of care labor and world-systems theory, may illustrate a patterned reproduction of peripherality across global, national, and intimate scales. At each level, a figure of the "other" is structurally produced and spatially distributed as a site of exclusion, subsequently naturalized, justified, and operationalized within the contemporary capitalist world-system. Here, Turkey may function as a semi-peripheral state within the capitalist world-system; rural provinces as internal peripheries marked by constrained institutional capacity and fragmented provision; caregivers as affective and embodied peripheries whose labor is essential yet devalued; and patients with rare diseases as peripheral to pharmaceutical value chains, instrumentalized for their biomedical data while denied equitable therapeutic access.

These layered formations of peripherality do not operate in isolation but interact recursively, producing compounded forms of marginalization across registers of geography, class, gender, and biology. What emerges, then, is a constellation of

nested peripheries, which are reproduced and distributed through the profit-maximizing logics of global capital, the uneven reach of resource-limited state provision, and the intimate infrastructures of caregiving that absorb, substitute, and resist systemic failure, fragmentation, and abandonment. These layered peripheries materialize in the lived experiences of therapeutic inaccessibility and caregiving within the SMA community in Turkey, where structural inequalities across scales converge to shape everyday life through forms of access that are limited, stratified, and unreliable as a consequence of global and domestic hierarchies.

CHAPTER 7

CONCLUSION

This thesis has examined the barriers to accessing the SMA gene therapy Zolgensma in Turkey through an analysis grounded in world-systems theory and Marxist-feminist critiques of care labor. It interrogated how global socioeconomic hierarchies, intellectual property regimes, domestic regulatory constraints, and intimate economies of caregiving intersect to shape the ways in which therapeutic inaccessibility to a rare disease treatment is structured, experienced, and resisted in a semi-peripheral context within the contemporary capitalist world-system. Drawing on a comprehensive review of secondary literature, documentary analysis of ministry documents, institutional reports, and public communications, as well as 14 in-depth semi-structured interviews with caregivers, patients, clinicians, NGO representatives, patient advocates, the study found that the absence of a formal, equitable access pathway to Zolgensma in Turkey is shaped by a constellation of structural forces, including (1) the profit-maximizing logics of pharmaceutical capital governing the marketization of high-cost orphan drugs; (2) the monopolistic pricing structures and exclusionary market behavior incentivized by the TRIPS-based intellectual property regime; and (3) the resource-constrained, fragmented, and regionally uneven implementation of public healthcare provision in Turkey. In the absence of formal and equitable access pathways, families are forced to absorb the burden of therapeutic procurement either through fundraising campaigns or through direct participation in the production of therapeutic value via rare clinical trials and early access programs with highly limited quotas. Access to these informal pathways remains uneven, as geographic location, socioeconomic status, social capital, digital

literacy, and gendered norms collectively structure unequal prospects for therapeutic access, campaign viability, caregiving sustainability, and patient outcomes over time. The absence of conclusive longitudinal data on the clinical safety and efficacy of Zolgensma further complicates the access landscape in multiple ways through enabling regulatory hesitancy, deferring state accountability, intensifying familial precarity, and problematizing the very conditions under which therapeutic value itself is constructed, priced, and withheld. Given the time-sensitive, progressive, and emotionally urgent nature of SMA as a severe pediatric-onset condition, this uncertainty also raises critical questions about how therapeutic hope and affective vulnerabilities become entangled with market imperatives in ways that commodify urgency and sustain inflated, opaque valuations.

In this context, therapeutic access is shaped less by clinical eligibility or urgency of need, and more by one's embedded position within intersecting structures of privilege, whether through the ability to be provided for as a citizen, to mobilize financial resources, or, at the very least, to fundraise, to be seen, and to be believed. These dynamics constitute a multi-scalar architecture of exclusion, referred to in this thesis as "nested peripheries," in which peripherality is reproduced and distributed across global, national, familial, and embodied scales. From Turkey's constrained semi-peripheral positioning within the capitalist world-system, to the infrastructural neglect of rural provinces, to the feminized burden of under-compensated care labor, and finally to the biomedical marginalization of SMA patients themselves, each layer reveals a site of extraction without adequate reciprocation—where capital, care, data, and life itself are mobilized in the production of structural value, yet systemic support remains absent, limited, uneven, conditional, or precarious, giving rise to interlocking global, national, affective, and biomedical peripheries, respectively.

Within these nested peripheries, care labor also emerges as a site of resistance, where families not only absorb systemic abandonment, but actively contest it through a set of embodied, affective, and relational practices that reconfigure domestic and public spaces, mobilizing social networks, and asserting claims to visibility, dignity, and survival.

While this thesis provides an empirically grounded and theoretically informed analysis of therapeutic inaccessibility through the case of Zolgensma in Turkey, several methodological and analytical constraints should be noted. First, the absence of direct participation from representatives of pharmaceutical companies, regulatory agencies, and state institutions limited the scope of inquiry into policy development and corporate strategy to secondary sources and indirect accounts from other stakeholders. Second, the study is constrained by its single-case design, purposive sampling strategy, and relatively small participant pool, which preclude the possibility of statistical generalizability, empirical exhaustiveness, or comprehensive representativeness. Third, data collection occurred during a period of ongoing clinical, regulatory, and financial change surrounding Zolgensma, which may reduce the temporal stability of certain empirical findings. Finally, as with all qualitative research, the interpretations advanced here are shaped by the researcher's positionality within specific sociopolitical and academic contexts, and remain subject to the epistemological contingencies of working within emotionally charged, relationally complex, and structurally uneven research environments.

Still, in making these arguments, this thesis advances three key contributions. First, it provides one of the few empirically grounded analyses of therapeutic inaccessibility to SMA gene therapy within the Turkish context and thus contributes to a broader understanding of pharmaceutical exclusion across semi-peripheral

settings. Second, it extends Marxist feminist critiques of care labor by demonstrating how uncompensated or undercompensated caregiving functions not only as a site of gendered inequality, but also as a structurally essential mechanism for survival in contexts of pharmaceutical abandonment. Finally, by conceptualizing "nested peripheries" as a multi-scalar framework that bridges the macro-analytic scope of world-systems theory with the embodied, affective, and relational dimensions of care labor, this thesis contributes to an emerging empirical and theoretical agenda concerned with tracing how marginalization is reproduced, distributed, and contested across global, national, familial, and intimate scales.

The findings of this study carry several implications for policy design, institutional reform, and future scholarly inquiry. At the international level, the case of Zolgensma in Turkey contributes to a growing body of evidence pointing to the inadequacy of current global governance frameworks for addressing access to medicines in structurally disadvantaged contexts. It highlights the distributive consequences of orphan drug legislation and the political-economic limitations of TRIPS-compliant models of innovation, particularly in states with constrained health budgets and large at-risk populations. The persistent inaccessibility of high-cost gene therapies such as Zolgensma outside of advanced economies underscores the urgent need for the development of alternative regulatory frameworks that facilitate more equitable pricing negotiations, enable broader and less politically constrained application of TRIPS flexibilities, and support innovation models that "de-link" drug prices from R&D costs, profit-maximizing imperatives, and monopolistic market structures (Moon et al., 2012; 't Hoen, 2009). It also underscores the importance of incorporating access-oriented metrics into health trade negotiations and international regulatory frameworks. To allow for fully informed decision-making and public

accountability, such efforts must be accompanied by increased transparency in pharmaceutical pricing models, clinical trial data, and post-market safety and efficacy outcomes. At the national level, the examined case reveals the need for comprehensive and regionally responsive care infrastructures that can mitigate the disproportionate burdens currently borne by families, especially by women, under conditions of therapeutic inaccessibility. Without depending on the precarious goodwill of public fundraising or the systematic exploitation of unpaid, feminized labor under the banner of familial duty, these infrastructures should secure comprehensive reimbursement mechanisms for medical consumables and equipment, expanded home-based care services, access to multidisciplinary clinical teams, provision of psycho-social support, and complete recognition of the caregiving labor performed by families across all stages of the therapeutic journey.

Here, several lines of inquiry are warranted for future research. First, comparative studies across similarly situated semi-peripheral or peripheral countries could contextualize the examined case and trace broader geographies of therapeutic exclusion within the Global South. Second, longitudinal analyses of patient and caregiver trajectories could illuminate how medical, bureaucratic, and affective burdens evolve under shifting policy regimes. Third, interdisciplinary research on the nexus of gender, pharmaceutical access, and care labor could enhance understanding of how structural neglect is absorbed, redistributed, or resisted within the intimate domain of the household. Fourth, empirical research on patent regimes and orphan drug policies in resource-constrained settings could clarify the contested relationship between pharmaceutical innovation, access, and global health equity. Finally, studies examining the micro-politics of drug pricing, procurement, and regulatory decision-

making in semi-peripheral and peripheral contexts would provide critical insight into how access is managed, delayed, or denied within uneven global political economies.

Taken together, these findings underscore the urgent need for multi-level reform, demonstrating that therapeutic inaccessibility is not simply a consequence of policy failure, but a structurally produced outcome situated within "nested peripheries," where exclusion is reproduced across global, national, familial, and embodied scales, and the burdens of care, cost, and uncertainty are disproportionately displaced onto those least equipped to bear them. As the contours of global health governance continue to evolve, the case of Zolgensma in Turkey reminds us that pharmaceutical innovation, in the absence of equitable access, produces not only new treatments, but new exclusions. As the inverse relationship between therapeutic availability and clinical need remains a defining feature of the contemporary pharmaceutical order, the global distribution of life-saving therapies continues to mirror—rather than remedy—the geographies of inequality. This reality underscores the imperative that any domain so fundamental to human health and collective well-being must be governed not by the logics of scarcity, monopoly, and profit, but by enduring commitments to equity, accessibility, and care. Where global capital withdraws and the state defers, it is the labor of care that fills the void—not to sustain the system, but to make survival possible in spite of it. Overall, this thesis calls for a reorientation of both research and policy toward those whose labor sustains what institutions have abandoned, whose lives are most profoundly shaped by structural exclusion, and whose perspectives must therefore be central to any meaningful project of systemic redress.

APPENDIX A

OVERVIEW OF APPROVED TREATMENTS FOR SPINAL MUSCULAR ATROPHY (SMA)

Treatment	Active Ingredient	Treatment Type	Manufacturer	Administration Method	Administration Frequency	Approval Year & Approx. Prices[13]
Spinraza	Nusinersen	Antisense oligonucleotide (SMN2 splicing modifier)	Biogen	Intrathecal injection	Initial: 4 loading doses over 2 months Maintenance: every 4 months	2016 (FDA) 2017 (EMA) ~$156,000 per dose ~$750,000 first year ~$375,000 annually
Zolgensma	Onasemnogene abeparvovec	Gene therapy (SMN1 gene replacement)	Novartis	Intravenous infusion	One-time administration	2019 (FDA) 2020 (EMA) ~$2.1 million single dose
Evrysdi	Risdiplam	SMN2 splicing modifier	Roche	Oral (liquid or tablet)	Daily	2020 (FDA) 2021 (EMA) 2025 (tablet FDA) ~$93,000-$340,000 annually

References for Appendix A

Moultrie, F., Chiverton, L., Hatami, I., Lilien, C., & Servais, L. (2025). Pushing the boundaries: Future directions in the management of spinal muscular atrophy. *Trends in Molecular Medicine, 31*(4), 307–318. https://doi.org/10.1016/j.molmed.2024.12.006

Sagonowski, E. (2020, August 7). Roche gets a green light to challenge Biogen, Novartis with SMA med Evrysdi. *Fierce Pharma.* https://www.fiercepharma.com/pharma/roche-set-to-take-biogen-novartis-sma-evrysdi-approval

13 Approximate prices adapted from Sagonowi (2020). The rest of the data adapted from Moultrie et al. (2025)

APPENDIX B

TABLE OF PARTICIPANT ROLES

Participant ID	Role(s)	Gender
P01	• Representative from SMA Foundation Turkey • Parental caregiver	Female
P02	• Representative from SMA Foundation Turkey • Parental caregiver (bereaved)	Female
P03	• Representative from SMA Foundation Turkey • Adult- onset SMA patient	Male
P04	• Parental caregiver (single parent) • Independent advocate	Female
P05	• Parental caregiver	Male
P06	• Representative from SMA Walk with Me Organization • Non-parental caregiver	Male
P07	• Physician and academic specializing in pediatric neurology and rare diseases	Female
P08	• Adult-onset SMA patient	Male
P09	• Parental caregiver	Female
P10	• Parental caregiver	Female
P11	• Parental caregiver	Male
P12	• Parental caregiver	Male
P13	• Representative from a grassroots organization focused on SMA	Male
P14	• Legal expert and academic specializing in healthcare law and patients' rights	Female

APPENDIX C

ETHICS COMMITTEE APPROVAL

T.C.
BOĞAZİÇİ ÜNİVERSİTESİ REKTÖRLÜĞÜ
Sosyal ve Beşeri Bilimler İnsan Araştırmaları Etik Kurulu (Sbinarek)

2025 AİLE YILI

Sayı : E-84391427-050.04-232583 20.05.2025
Konu : 2025-17T Kayıt Numaralı Başvurunuza İlişkin
Etik Kurul Onay Kararı (2025-04)

Sayın Doç. Dr. Zühre AKSOY TERZİBAŞOĞLU

Tez danışmanlığını yürütmekte olduğunuz öğrenciniz İnci Gizem Gümüş'ün "Yaşamsal İlaçlara Erişimde Küresel Eşitsizlikleri Anlamak: Türkiye'de Zolgensma Örneği" başlıklı projesi ile Boğaziçi Üniversitesi Sosyal ve Beşeri Bilimler İnsan Araştırmaları Etik Kurulu'na (SBİNAREK) yapmış olduğunuz, *2025-17T kayıt numaralı başvurunuz*, 05.05.2025 tarih ve 2025/04 sayılı kurul toplantısında incelenmiş olup projenize etik onay verilmesi uygun bulunmuştur.

Bu karar, tüm kurul üyelerinin çevrim içi katılımıyla gerçekleştirilen toplantıda oy birliği ile alınmıştır. Onay mektubu, tüm üyeler adına Komisyon Başkanı tarafından e-imzalanmıştır.

Bilgilerinizi rica ederiz.

Saygılarımızla,

Dr. Öğr. Üyesi Işıl ERDUYAN
Kurul Başkanı

Bu belge, güvenli elektronik imza ile imzalanmıştır.
Belge Doğrulama Kodu : 09AI-GK9R-0P2P Belge Doğrulama Adresi : https://www.turkiye.gov.tr/bogazici-universitesi-ebys

Adres: Boğaziçi Üniversitesi 34342 Bebek/ İstanbul
Telefon No: Faks No:
e-Posta: İnternet Adresi: www.bogazici.edu.tr
Kep Adresi: bogaziciuniversitesi@hs01.kep.tr

Bilgi İçin: Sevin KOÇ KÖKSAL
Koordinatör
Telefon No: 0 212 359-6837

REFERENCES

Abbott, R. B., Bader, R., Bajjali, L., Elsamen, T. A., Obeidat, T., Sboul, H., Shwayat, M., & Alabbadi, I. (2012). The price of medicines in Jordan: The cost of trade-based intellectual property. *Journal of Generic Medicines, 9*(2), 75–85. https://doi.org/10.1177/1741134312447499

Adachi, T., El-Hattab, A. W., Jain, R., Nogales Crespo, K. A., Quirland Lazo, C. I., Scarpa, M., Summar, M., & Wattanasirichaigoon, D. (2023). Enhancing equitable access to rare disease diagnosis and treatment around the world: A review of evidence, policies, and challenges. *International Journal of Environmental Research and Public Health, 20*(6), 4732. https://doi.org/10.3390/ijerph20064732

Adiller, D., & Güreller, M. (2021). Twitter ve kamusal alan kavramı üzerine bir içerik analizi: Spinal müsküler atrofi (SMA) hastası çocukların kampanyalarıyla ilgili örnek olay çalışması. *Türkiye İletişim Araştırmaları Dergisi, 38*, 400–415. https://doi.org/10.17829/turcom.932145

Adorno, T. W. (2005). *Critical models: Interventions and catchwords* (H. W. Pickford, Trans.). Columbia University Press. (Original work published 1963)

Ahmed, S. K. (2024). The pillars of trustworthiness in qualitative research. *Journal of Medicine, Surgery, and Public Health, 2*, 100051. https://doi.org/10.1016/j.glmedi.2024.100051

Akaleephan, C., Wibulpolprasert, S., Sakulbumrungsil, R., Luangruangrong, P., Jitraknathee, A., Aeksaengsri, A., Udomaksorn, S., Tangcharoensathien, V., & Tantivess, S. (2009). *Extension of market exclusivity and its impact on the accessibility to essential medicines, and drug expense in Thailand: Analysis of the effect of TRIPs-Plus proposal*. *Health Policy, 91*(2), 174–182. https://doi.org/10.1016/j.healthpol.2008.12.009

Akçalı, E., & Korkut, U. (2015). Urban transformation in Istanbul and Budapest: Neoliberal governmentality in the EU's semi-periphery and its limits. *Political Geography, 46*, 76–88. https://doi.org/10.1016/j.polgeo.2014.12.004

Akinsola, O. K., & Liang, W. (2025). Intellectual property rights (IPR) under WTO agreements: Balancing innovation and access.

Aksu, B. (2019). Rare disease and orphan drug situations in Turkey and around the world. *Aurum Journal of Health Sciences, 1*(2), 95–109.

Aktaş, P. (2021). Chronic and rare disease patients' access to healthcare services during a health crisis: The example of the COVID-19 pandemic in Turkey. *Health Expectations, 24*(5), 1812–1820. https://doi.org/10.1111/hex.13321

Alliance for Regenerative Medicine. (2023). *The sector snapshot: August 2023*. https://alliancerm.org/the-sector-snapshot-august-2023/

Almond, G. A., & Verba, S. (1963). *The civic culture: Political attitudes and democracy in five nations.* Princeton University Press. https://www.jstor.org/stable/j.ctt183pnr2

Alnasseri, S., Kaya, Y., Nakhae, N., Daryani, H., & Caramento, A. (2011). Development in (semi-) peripheral countries: The cases of Turkey, Iran, and South Africa. *World Review of Political Economy, 2*(3), 495–512. http://www.jstor.org/stable/41931938

American Society of Gene and Cell Therapy. (2024). *Gene, cell, & RNA therapy landscape report: Q1 2024 quarterly data report.* https://asgct.org/publications/landscape-report

Amin, S. (1976). *Unequal development: An essay on the social formations of peripheral capitalism* (B. Pearce, Trans.). Monthly Review Press. (Original work published 1973)

Amin, T., & Kesselheim, A. S. (2012). Secondary patenting of branded pharmaceuticals: A case study of how patents on two HIV drugs could be extended for decades. *Health Affairs, 31*(10), 2286–2294. https://doi.org/10.1377/hlthaff.2012.0107

Andreski, S. (2013). *Max Weber on capitalism, bureaucracy, and religion.* Routledge.

Arnold, C., & Naseemullah, A. (2024). Seeking autonomy in the semi-periphery: Neomercantilism and diversification in Turkey. *Studies in Comparative International Development, 59*(4), 665–689. https://doi.org/10.1007/s12116-024-09435-1

Arza, V., López, A., Montes-Rojas, G., & Pascuini, P. (2023). In the name of TRIPS: The impact of IPR harmonisation on patent activity in Latin America. *Research Policy, 52*(6), 104759. https://doi.org/10.1016/j.respol.2023.104759

Athreye, S., Piscitello, L., & Shadlen, K. C. (2020). Twenty-five years since TRIPS: Patent policy and international business. *Journal of International Business Policy, 3*(4), 315–328. https://doi.org/10.1057/s42214-020-00079-1

Auriol, E., Biancini, S., & Paillacar, R. (2022). Intellectual property rights protection and trade: An empirical analysis (AFD Research Papers No. 240, pp. 1–67). *Éditions AFD*. https://shs.cairn.info/journal-afd-research-papers-2022-240-page-1?lang=en

Auriol, E., Biancini, S., & Paillacar, R. (2019). Universal intellectual property rights: Too much of a good thing? *International Journal of Industrial Organization, 65*, 51–81. https://doi.org/10.1016/j.ijindorg.2019.01.003

Babones, S., Chase-Dunn, C., & Mielants, E. (Eds.). (2012). *Routledge handbook of world-systems analysis.* Routledge.

Baker, B., & Avafia, T. (2011). The evolution of IPRs from humble beginnings to the modern day TRIPS-plus era: Implications for treatment access [Working paper]. Global Commission on HIV and the Law, Technical Advisory Group Meeting.

Balance, R., Pogany, J., & Forstner, H. (1992). *The world's pharmaceutical industries: An international perspective on innovation, competition and policy.* Edward Elgar.

Ballardini, R. M., Mimler, M., Minssen, T., & Rutz, B. (2022). 3D printing, intellectual property rights and medical emergencies: In search of new flexibilities. *IIC - International Review of Intellectual Property and Competition Law, 53*(10), 1149–1173. https://doi.org/10.1007/s40319-022-01235-1

Banerjee, A., & Rewegan, A. (2017). Intensifying relational care: The challenge of dying in long-term residential care. *Journal of Canadian Studies, 50*(2), 396–421. https://doi.org/10.3138/jcs.50.2.396

Beall, R. F., Hardcastle, L., Clement, F., & Hollis, A. (2019). How will recent trade agreements that extend market protections for brand-name prescription pharmaceuticals impact expenditures and generic access in Canada? *Health Policy, 123*(12), 1251–1258. https://doi.org/10.1016/j.healthpol.2019.09.005

Beall, R. F., Nickerson, J. W., Kaplan, W. A., & Attaran, A. (2016). Is patent "evergreening" restricting access to medicine/device combination products? *PLOS ONE, 11*(2), e0148939. https://doi.org/10.1371/journal.pone.0148939

Belzer, L. T., Wright, S. M., Goodwin, E. J., Singh, M. N., & Carter, B. S. (2022). Psychosocial considerations for the child with rare disease: A review with recommendations and calls to action. *Children, 9*(7), 933. https://doi.org/10.3390/children9070933

Berndt, E., & Aitken, M. (2011). Brand loyalty, generic entry, and price competition in pharmaceuticals in the quarter century after the 1984 Waxman-Hatch legislation. *International Journal of the Economics of Business, 18*(2), 177–201. https://doi.org/10.1080/13571516.2011.584423

Bessen, J., & Maskin, E. (2009). Sequential innovation, patents, and imitation. *The RAND Journal of Economics, 40*(4), 611–635. https://doi.org/10.1111/j.1756-2171.2009.00081.x

Boettcher, J., Boettcher, M., Wiegand-Grefe, S., & Zapf, H. (2021). Being the pillar for children with rare diseases—A systematic review on parental quality of life. *International Journal of Environmental Research and Public Health, 18*(9), 4993. https://doi.org/10.3390/ijerph18094993

Bordo, S. (1986). The Cartesian masculinization of thought. *Signs, 11*(3), 439–456. http://www.jstor.org/stable/3174004

Borrell, J.-R., & Watal, J. (2002). Impact of patents on access to HIV/AIDS drugs in developing countries (CID Working Paper No. 92). Center for International Development at Harvard University. https://www.hks.harvard.edu/centers/cid/publications/faculty-working-papers/impact-of-patents-on-access-to-hiv-aids-drugs-in-developing-countries

Brandt, M., Johannsen, L., Inhestern, L., & Bergelt, C. (2022). Parents as informal caregivers of children and adolescents with spinal muscular atrophy: A systematic review of quantitative and qualitative data on the psychosocial situation, caregiver burden, and family needs. *Orphanet Journal of Rare Diseases, 17*(1), 274. https://doi.org/10.1186/s13023-022-02407-5

Branstetter, L., & Saggi, K. (2011). Intellectual property rights, foreign direct investment and industrial development. *The Economic Journal, 121*(555), 1161–1191. https://doi.org/10.1111/j.1468-0297.2011.02440.x

Bröckling, U. (2015). *The entrepreneurial self: Fabricating a new type of subject.* SAGE Publications.

Bryson, V. (2007). *Gender and the politics of time: Feminist theory and contemporary debates* (1st ed.). Bristol University Press. https://doi.org/10.2307/j.ctt9qgwcr

Cadaval Narezo, M. (2022). Methodologies for collaborative, respectful and caring research: Conversations with professional Indigenous women from Mexico. In W. Harcourt, K. van den Berg, C. Dupuis, & J. Gaybor (Eds.), *Feminist methodologies* (pp. 139–163). Palgrave Macmillan. https://doi.org/10.1007/978-3-030-82654-3_7

Cardinali, P., Migliorini, L., & Rania, N. (2019). The caregiving experiences of fathers and mothers of children with rare diseases in Italy: Challenges and social support perceptions. *Frontiers in Psychology, 10*, 1780. https://doi.org/10.3389/fpsyg.2019.01780

Cederström, C. (2019). *State of Nordic fathers.* Nordic Council of Ministers.

Çelik, K. M., Kose, C. C., Kaya, D., Tekin, K., & Silan, F. (2024). Spinal muscular atrophy carrier screening program: Awareness and attitude of healthcare professionals in Turkey. *Journal of Community Genetics, 15*(6), 665–672. https://doi.org/10.1007/s12687-024-00737-4

Chambers, J. D., Silver, M. C., Berklein, F. C., Cohen, J. T., & Neumann, P. J. (2020). Orphan drugs offer larger health gains but less favorable cost-effectiveness than non-orphan drugs. *Journal of General Internal Medicine, 35*(9), 2629–2636. https://doi.org/10.1007/s11606-020-05805-2

Chan, A. Y. L., Chan, V. K. Y., Olsson, S., Fan, M., Jit, M., Gong, M., Zhang, S., Ge, M., Pathadka, S., Chung, C. C. Y., Chung, B. H. Y., Chui, C. S. L., Chan, E. W., Wong, G. H. Y., Lum, T. Y., Wong, I. C. K., Ip, P., & Li, X. (2020). Access and unmet needs of orphan drugs in 194 countries and 6 areas: A global policy review with content analysis. *Value in Health, 23*(12), 1580–1591. https://doi.org/10.1016/j.jval.2020.06.020

Chaudhuri, S. (2013). The pharmaceutical industry in India after TRIPS. In H. Löfgren & O. D. Williams (Eds.), *The new political economy of pharmaceuticals* (pp. 141–158). Palgrave Macmillan. https://doi.org/10.1057/9781137315854_6

Chaudhuri, S., Goldberg, P. K., & Jia, P. (2006). Estimating the effects of global patent protection in pharmaceuticals: A case study of quinolones in India. *American Economic Review, 96*(5), 1477–1514. https://doi.org/10.1257/aer.96.5.1477

Chen, T. H. (2020). New and developing therapies in spinal muscular atrophy: From genotype to phenotype to treatment and where do we stand? *International Journal of Molecular Sciences, 21*(9), 3297. https://doi.org/10.3390/ijms21093297

Chen, Y., & Puttitanun, T. (2005). Intellectual property rights and innovation in developing countries. *Journal of Development Economics, 78*(2), 474–493. https://doi.org/10.1016/j.jdeveco.2004.11.005

Chien, C. V. (2003). Cheap drugs at what price to innovation: Does the compulsory licensing of pharmaceuticals hurt innovation? *Berkeley Technology Law Journal, 18*(3), 853–907. https://digitalcommons.law.scu.edu/facpubs/25

Cho, K., Kim, C., & Shin, J. (2015). Differential effects of intellectual property rights on innovation and economic performance: A cross-industry investigation. *Science and Public Policy, 42*(6), 827–840. https://doi.org/10.1093/scipol/scv009

Chu, A., Cozzi, G., & Galli, S. (2014). Stage-dependent intellectual property rights. *Journal of Development Economics, 106*, 239–249. https://doi.org/10.1016/j.jdeveco.2013.10.005

Chu, A. C., & Peng, S.-K. (2011). International intellectual property rights: Effects on growth, welfare and income inequality. *Journal of Macroeconomics, 33*(2), 276–287.

Cioffi, G., Andreu, P., Karam, J., Child, C., & Chiesi, G. (2021). Evaluation of the societal burden of rare diseases in the United States. *Research Square.* https://doi.org/10.21203/rs.3.rs-936611/v1

Clarke, V., & Braun, V. (2016). Thematic analysis. *The Journal of Positive Psychology, 12*(3), 297–298. https://doi.org/10.1080/17439760.2016.1262613

Clift, C. (2010). Why IPR issues were brought to GATT: A historical perspective on the origins of TRIPS. In C. Correa (Ed.), *Research handbook on the protection of intellectual property under WTO rules* (pp. 3–21). Edward Elgar Publishing. https://doi.org/10.4337/9781849806596.00007

Coates, M. M., Ezzati, M., Robles Aguilar, G., Kwan, G. F., Vigo, D., Mocumbi, A. O., Becker, A. E., Makani, J., Hyder, A. A., Jain, Y., Stefan, D. C., Gupta, N., Marx, A., & Bukhman, G. (2021). Burden of disease among the world's poorest billion people: An expert-informed secondary analysis of Global Burden of Disease estimates. *PLOS ONE, 16*(8), e0253073. https://doi.org/10.1371/journal.pone.0253073

Cockburn, I. M., Lanjouw, J. O., & Schankerman, M. (2016). Patents and the global diffusion of new drugs. *American Economic Review, 106*(1), 136–164. https://doi.org/10.1257/aer.20141482

Coffey, C., Espinoza Revollo, P., Harvey, R., Lawson, M., Parvez Butt, A., Piaget, K., Sarosi, D., & Thekkudan, J. (2020). *Time to care: Unpaid and underpaid care work and the global inequality crisis* (Oxfam Briefing Paper). Oxfam. https://doi.org/10.21201/2020.5419

Correa, C. M. (2000). *Intellectual property rights, the WTO and developing countries: The TRIPS Agreement and policy options.* Zed Books.

Correa, C. M. (2011). Pharmaceutical innovation, incremental patenting and compulsory licensing (Research Paper No. 41). *South Centre.* https://hdl.handle.net/10419/232159

Correa, C. M. (2022). Interpreting the flexibilities under the TRIPS Agreement. In C. M. Correa & R. M. Hilty (Eds.), *Access to medicines and vaccines* (pp. 3–18). Springer. https://doi.org/10.1007/978-3-030-83114-1_1

Craddy, P., Hewitt, C., & Foxon, G. (2023, December). EE700: Are acute therapies and curative drugs more affordable than chronic treatments in rare diseases? An analysis of the top 10 most expensive drugs in the US compared with Germany. *Value in Health, 26*(12, Suppl), S189.

Currie, G., & Szabo, J. (2019). "It would be much easier if we were just quiet and disappeared": Parents silenced in the experience of caring for children with rare diseases. *Health Expectations, 22*(6), 1251–1259. https://doi.org/10.1111/hex.12958

Czech, M., Baran-Kooiker, A., Atikeler, K., Demirtshyan, M., Gaitova, K., Holownia-Voloskova, M., Turcu-Stiolica, A., Kooiker, C., Piniazhko, O., Konstandyan, N., Zalis'ka, O., & Sykut-Cegielska, J. (2020). A review of rare disease policies and orphan drug reimbursement systems in 12 Eurasian countries. *Frontiers in Public Health*, 7, 416. https://doi.org/10.3389/fpubh.2019.00416

Dai, R., & Watal, J. (2021). Product patents and access to innovative medicines. *Social Science & Medicine, 291*, 114479. https://doi.org/10.1016/j.socscimed.2021.114479

Dangouloff, T., & Servais, L. (2019). Clinical evidence supporting early treatment of patients with spinal muscular atrophy: Current perspectives. *Therapeutics and Clinical Risk Management, 15*, 1153–1161. https://doi.org/10.2147/TCRM.S172291

de Sousa Santos, B. (2020). Opening up the canon of knowledge and recognition of difference. In B. de Sousa Santos (Ed.), *Another knowledge is possible: Beyond Northern epistemologies*. Verso.

Deardorff, A. V. (1992). Welfare effects of global patent protection. *Economica, 59*(233), 35–51. https://doi.org/10.2307/2555064

DeCamp, M. W. (2007). Global health: A normative analysis of intellectual property rights and global distributive justice [Doctoral dissertation, Duke University]. *DukeSpace*. https://hdl.handle.net/10161/193

Deere, C. (2008). *The implementation game: The TRIPS Agreement and the global politics of intellectual property reform in developing countries.* Oxford University Press. https://doi.org/10.1093/acprof:oso/9780199550616.001.0001

Deere-Birkbeck, C. (2010). Developing countries in the global IP system before TRIPS: The political context for the TRIPS negotiations. In C. M. Correa (Ed.), *Research handbook on the protection of intellectual property under WTO rules* (Vol. 1, pp. 53–80). Edward Elgar Publishing. https://doi.org/10.4337/9781849806596.00008

Depping, M. K., Uhlenbusch, N., von Kodolitsch, Y., Klose, H. F. E., Mautner, V. F., & Löwe, B. (2021). Supportive care needs of patients with rare chronic diseases: Multi-method, cross-sectional study. *Orphanet Journal of Rare Diseases, 16*(1), 44. https://doi.org/10.1186/s13023-020-01660-w

Dinopoulos, E., & Segerstrom, P. (2010). Intellectual property rights, multinational firms and economic growth. *Journal of Development Economics, 92*(1), 13–27. https://doi.org/10.1016/j.jdeveco.2009.01.007

Diwakar, B., & Sorek, G. (2016). *Dynamics of human capital accumulation, IPR policy, and growth* (Auburn Economics Working Paper Series No. auwp2016-11). Department of Economics, Auburn University.

Diwan, I., & Rodrik, D. (1991). Patents, appropriate technology, and North–South trade. *Journal of International Economics, 30*(1–2), 27–47.

Dodge, J. A., Chigladze, T., Donadieu, J., Grossman, Z., Ramos, F., Serlicorni, A., Siderius, L., Stefanidis, C. J., Tasic, V., Valiulis, A., & Wierzba, J. (2011). The importance of rare diseases: From the gene to society. *Archives of Disease in Childhood, 96*(9), 791–792. https://doi.org/10.1136/adc.2010.193664

Dodson, L. (2009). *The moral underground: How ordinary Americans subvert an unfair economy.* The New Press.

Dorlach, T. (2015). The prospects of egalitarian capitalism in the global South: Turkish social neoliberalism in comparative perspective. *Economy and Society, 44*(4), 519–544. https://doi.org/10.1080/03085147.2015.1090736

Dos Santos, T. (1970). The structure of dependence. *The American Economic Review, 60*(2), 231–236.

Doxzen, K. W., Adair, J. E., Fonseca Bazzo, Y. M., Bukini, D., Cornetta, K., Dalal, V., Guerino-Cunha, R. L., Hongeng, S., Jotwani, G., Kityo-Mutuluuza, C., Lakshmanan, K., Mahlangu, J., Makani, J., Mathews, V., Ozelo, M. C., Rangarajan, S., Scholefield, J., Batista Silva Júnior, J., & McCune, J. M. (2024). The translational gap for gene therapies in low- and middle-income countries. *Science Translational Medicine, 16*(746), eadn1902. https://doi.org/10.1126/scitranslmed.adn1902

Drahos, P. (1996). *A philosophy of intellectual property*. Dartmouth.

Duso, T., Herr, A., & Suppliet, M. (2014). The welfare impact of parallel imports: A structural approach applied to the German market for oral anti-diabetics. *Health Economics, 23*(9), 1036–1057. https://doi.org/10.1002/hec.3068

Dutta, A. (2011). From free entry to patent protection: Welfare implications for the Indian pharmaceutical industry. *The Review of Economics and Statistics, 93*(1), 160–178. https://doi.org/10.1162/REST_a_00056

Dutfield, G. (2017). *Intellectual property rights and the life science industries: A twentieth century history*. Routledge.

Eicher, T., & García-Peñalosa, C. (2008). Endogenous strength of intellectual property rights: Implications for economic development and growth. *European Economic Review, 52*(2), 237–258. https://doi.org/10.1016/j.euroecorev.2007.10.003

Eicher, T. S., & Newiak, M. (2013). Intellectual property rights as development determinants. *The Canadian Journal of Economics / Revue canadienne d'économique, 46*(1), 4–22. http://www.jstor.org/stable/42705861

Ekici, B., Bozkurt, B., Tatlı, B., Calışkan, M., Aydınlı, N., & Özmen, M. (2012). Demographic characteristics of SMA type 1 patients at a tertiary center in Turkey. *European Journal of Pediatrics*, 171(3), 549–552. https://doi.org/10.1007/s00431-011-1607-2

Elek, P., Harsányi, A., Zelei, T., Csetneki, K., & Kaló, Z. (2017). Policy objective of generic medicines from the investment perspective: The case of clopidogrel. *Health Policy, 121*(5), 558–565. https://doi.org/10.1016/j.healthpol.2017.02.015

El Said, M. (2022). The impact of 'TRIPS-Plus' rules on the use of TRIPS flexibilities: Dealing with the implementation challenges. In C. M. Correa & R. M. Hilty (Eds.), *Access to medicines and vaccines* (pp. 297–329). Springer. https://doi.org/10.1007/978-3-030-83114-1_11

El-Said, M. (2005). The road from TRIPS-minus, to TRIPS, to TRIPS-plus: Implications of IPRs for the Arab world. *The Journal of World Intellectual Property, 8*(1), 53–65. https://doi.org/10.1111/j.1747-1796.2005.tb00237.x

Emmanuel, A. (1972). *Unequal exchange: A study of the imperialism of trade.* Monthly Review Press.

Eren, I. (2002). The transnationalisation of the Turkish pharmaceutical industry [Doctoral dissertation, University of Sussex].

Eren-Vural, I. (2007a). Domestic contours of global regulation: Understanding the policy changes on pharmaceutical patents in India and Turkey. *Review of International Political Economy, 14*(1), 105–130. https://doi.org/10.1080/09692290601081079

Eren-Vural, I. (2013). Neoliberalism, intellectual property rights and the Turkish pharmaceutical industry in the 2000s. In H. Löfgren & O. D. Williams (Eds.), *The new political economy of pharmaceuticals* (pp. 219–238). Palgrave Macmillan. https://doi.org/10.1057/9781137315854_12

Eren-Vural, İ. (2007b). Pharmaceuticals and intellectual property rights: A political economy of the recent policy changes across the developing world and in Turkey. *ODTÜ Gelişme Dergisi, 34*(2), 337–385. http://www2.feas.metu.edu.tr/metusd/ojs/index.php/metusd/article/view/168

Eriksson, M., Nielsen, H. P., & Paulgaard, G. (2016). The internal other: Reproducing and reworking center and periphery. In M. Eriksson, N. H. P. Nielsen, & G. Paulgaard (Eds.), *Remapping gender, place and mobility* (pp. 37–52). Routledge.

EvaluatePharma. (2023). *Orphan Drugs 2023-2028: A Flattening Curve.* https://www.evaluate.com/thought-leadership/orphan-drug-2023-report/

Evkaya Acar, A., Karadağ Saygı, E., İmamoğlu, S., Öztürk, G., Ünver, O., Ergenekon, P., Gökdemir, Y., Özel, G., & Türkdoğan, D. (2021). The burden of primary caregivers of spinal muscular atrophy patients and their needs. *Turkish Archives of Pediatrics, 56*(4), 366–373. https://doi.org/10.5152/TurkArchPediatr.2021.20117

Falvey, R., Foster, N., & Greenaway, D. (2006). Intellectual property rights and economic growth. *Review of Development Economics, 10*(4), 700–719. https://doi.org/10.1111/j.1467-9361.2006.00343.x

Falvey, R., Foster, N., & Greenaway, D. (2009). Trade, imitative ability and intellectual property rights. *Review of World Economics, 145*(3), 373–404. http://www.jstor.org/stable/40587820

Federici, S. (2012). *Revolution at point zero: Housework, reproduction, and feminist struggle.* PM Press.

Federici, S. (2019). *Re-enchanting the world: Feminism and the politics of the commons.* PM Press.

Fehr, A., & Prütz, F. (2023). Rare diseases: A challenge for medicine and public health. *Journal of Health Monitoring, 8*(4), 3–6. https://doi.org/10.25646/11826

Ferrarese, E. (2017). Precarity of work, precarity of moral dispositions: Concern for others in the era of "emotional" capitalism. *WSQ: Women's Studies Quarterly, 45*(3–4), 176–192. https://doi.org/10.1353/wsq.2017.0055

Flynn, S., Hollis, A., & Palmedo, M. (2009). An economic justification for open access to essential medicine patents in developing countries. *The Journal of Law, Medicine & Ethics, 37*(2), 184–208. https://doi.org/10.1111/j.1748-720X.2009.00365.x

Folbre, N. (1994). *Who pays for the kids? Gender and the structures of constraint* (1st ed.). Routledge. https://doi.org/10.4324/9780203168295

Folbre, N. (2020). *The rise and decline of patriarchal systems: An intersectional political economy.* Verso.

Frank, A. G. (1966). The development of underdevelopment. *Monthly Review, 18*(4), 17–31.

Frank, A. G. (1969). *Capitalism and underdevelopment in Latin America.* Monthly Review Press.

Frank, A. G. (1979). *Dependent accumulation and underdevelopment.* Monthly Review Press.

Fraser, N. (2016). Contradictions of capital and care. *New Left Review, 100*, 99–117.

Frericks, P. (2011). Marketising social protection in Europe: Two distinct paths and their impact on social inequalities. *International Journal of Sociology and Social Policy, 31*(5/6), 319–334. https://doi.org/10.1108/01443331111141282

Friedman, M. (1995). *Foreign economic aid: Means and objectives.* Hoover Institution on War, Revolution, and Peace.

Gaffke, L., Pierzynowska, K., Podlacha, M., Brokowska, J., & Węgrzyn, G. (2021). Changes in cellular processes occurring in mucopolysaccharidoses as underestimated pathomechanisms of these diseases. *Cell Biology International, 45*(3), 498–506. https://doi.org/10.1002/cbin.11275

Galeano, E. (1973). *Open veins of Latin America: Five centuries of the pillage of a continent.* Monthly Review Press.

Gamba, S. (2017). The effect of intellectual property rights on domestic innovation in the pharmaceutical sector. *World Development, 99*, 15–27. https://doi.org/10.1016/j.worlddev.2017.06.003

Gammie, T., Lu, C. Y., & Babar, Z. U. D. (2015). Access to orphan drugs: A comprehensive review of legislations, regulations and policies in 35 countries. *PLOS ONE, 10*(10), e0140002. https://doi.org/10.1371/journal.pone.0140002

German-Soto, V., & Chapa Cantú, J. C. (2018). Structural change in the international patenting level: The TRIPs Agreement's role. *International Journal of Technological Learning, Innovation and Development, 10*(2), 163–182. https://doi.org/10.1504/IJTLID.2018.093161

Gervais, D. (2015). Human rights and the philosophical foundations of intellectual property. In C. Geiger (Ed.), *Research handbook on human rights and intellectual property* (pp. 89–97). Edward Elgar Publishing.

Gill, S. (1991). *American hegemony and the Trilateral Commission.* Cambridge University Press.

Gingrich, J., & Häusermann, S. (2015). The decline of the working-class vote, the reconfiguration of the welfare support coalition and consequences for the welfare state. *Journal of European Social Policy, 25*(1), 50–75. https://doi.org/10.1177/0958928714556970

Giridharadas, A. (2019). *Winners take all: The elite charade of changing the world*. Vintage.

Glenn, E. N. (2010). *Forced to care: Coercion and caregiving in America.* Harvard University Press. https://doi.org/10.2307/j.ctv1p6hnw0

Gold, E. R., Morin, J.-F., & Shadeed, E. (2019). Does intellectual property lead to economic growth? Insights from a novel IP dataset. *Regulation & Governance, 13*(1), 107–124. https://doi.org/10.1111/rego.12165

Gopakumar, K. M. (2015). Twenty years of TRIPS Agreement and access to medicine: A development perspective. *Indian Journal of International Law, 55*(3), 367–404. https://doi.org/10.1007/s40901-016-0022-7

Granlund, D., & Köksal-Ayhan, M. Y. (2015). Parallel imports and a mandatory substitution reform: A kick or a muff for price competition in pharmaceuticals? *The European Journal of Health Economics, 16*(9), 969–983. https://doi.org/10.1007/s10198-014-0646-9

Grootendorst, P., & Hollis, A. (2011). The 2011 Canada-European Union Comprehensive Economic and Trade Agreement: An economic impact assessment of the EU's proposed pharmaceutical intellectual property provisions. *Journal of Generic Medicines, 8*(2), 81–103. https://doi.org/10.1177/1741134311408275

Grossman, G. M., & Lai, E. L.-C. (2004). International protection of intellectual property. *American Economic Review, 94*(5), 1635–1653. https://doi.org/10.1257/0002828043052312

Gutiérrez-Rodríguez, E. (2014). Domestic work–affective labor: On feminization and the coloniality of labor. *Women's Studies International Forum, 46*, 45–53. https://doi.org/10.1016/j.wsif.2014.03.005

Hammami, S. (2021). The effect of intellectual property protection on innovation: Empirical analysis of developing countries panel. *African Journal of Science, Technology, Innovation and Development, 13*(4), 397–405. https://doi.org/10.1080/20421338.2020.1824608

Hayek, F. A. (1966). The principles of a liberal social order. *Il Politico, 31*(4), 601–618. http://www.jstor.org/stable/43206528

Held, V. (2005). *The ethics of care: Personal, political, and global.* Oxford University Press.

Heller, M. A. (1998). The tragedy of the anticommons: Property in the transition from Marx to markets. *Harvard Law Review, 111*(3), 621–688.

Hellerstein, R. (2012). What do drug monopolies cost consumers in developing countries? *Economics Letters, 116*(1), 108–111. https://doi.org/10.1016/j.econlet.2011.12.114

Hickel, J., Dorninger, C., Wieland, H., & Suwandi, I. (2022). Imperialist appropriation in the world economy: Drain from the global South through unequal exchange, 1990–2015. *Global Environmental Change, 73*, 102467. https://doi.org/10.1016/j.gloenvcha.2022.102467

Hill, A., Redd, C., Gotham, D., Erbacher, I., Meldrum, J., & Harada, R. (2017). Estimated generic prices of cancer medicines deemed cost-ineffective in England: A cost estimation analysis. *BMJ Open, 7*(1), e011965. https://doi.org/10.1136/bmjopen-2016-011965

Hobbes, T. (2008). *Leviathan* (J. C. A. Gaskin, Ed.). Oxford University Press. (Original work published 1651)

Hoy, S. M. (2019). Onasemnogene abeparvovec: First global approval. *Drugs, 79*(11), 1255–1262. https://doi.org/10.1007/s40265-019-01162-5

Hsieh, C. R. (2013). Pharmaceuticals, health policy and intellectual property rights in China. In H. Löfgren & O. D. Williams (Eds.), *The new political economy of pharmaceuticals* (pp. 48–69). Palgrave Macmillan. https://doi.org/10.1057/9781137315854_3

Hu, A. G. Z., & Png, I. P. L. (2013). Patent rights and economic growth: Evidence from cross-country panels of manufacturing industries. *Oxford Economic Papers, 65*(3), 675–698. https://doi.org/10.1093/oep/gpt011

Hudson, J., & Minea, A. (2013). Innovation, intellectual property rights, and economic development: A unified empirical investigation. *World Development, 46*, 66–78. https://doi.org/10.1016/j.worlddev.2013.01.023

Hughes, J. (1988). The philosophy of intellectual property. *Georgetown Law Journal, 77*, 287–359.

Industrial Property Code [Law No. 6769 of December 22, 2016]. (2016). *WIPO Lex*. https://www.wipo.int/wipolex/en/legislation/details/16609

Inkeles, A., & Smith, D. H. (1974). *Becoming modern: Individual change in six developing countries.* Harvard University Press.

Iskander, D. (2013). TRIPS and access to medicines in Egypt. In H. Löfgren & O. D. Williams (Eds.), *The new political economy of pharmaceuticals* (pp. 91–110). Palgrave Macmillan. https://doi.org/10.1057/9781137315854_5

Islam, M. D., Kaplan, W. A., Trachtenberg, D., & Uribe, M. V. (2019). Impacts of intellectual property provisions in trade treaties on access to medicine in low- and middle-income countries: A systematic review. *Global Health, 15*, 88. https://doi.org/10.1186/s12992-019-0528-0

Jaeger, G., Röjvik, A., & Berglund, B. (2015). Participation in society for people with a rare diagnosis. *Disability and Health Journal, 8*(1), 44–50. https://doi.org/10.1016/j.dhjo.2014.07.004

Jayasundara, K., Hollis, A., Krahn, M., Mamdani, M., Hoch, J. S., & Grootendorst, P. (2019). Estimating the clinical cost of drug development for orphan versus non-orphan drugs. *Orphanet Journal of Rare Diseases, 14*(1), 12. https://doi.org/10.1186/s13023-018-0990-4

Jung, Y., & Kwon, S. (2015). The effects of intellectual property rights on access to medicines and catastrophic expenditure. *International Journal of Health Services, 45*(3), 507–529. http://www.jstor.org/stable/45140511

Kesselheim, A. S., Fischer, M. A., & Avorn, J. (2006). Extensions of intellectual property rights and delayed adoption of generic drugs: Effects on Medicaid spending. *Health Affairs, 25*(6), 1637–1647. https://doi.org/10.1377/hlthaff.25.6.1637

Kesselheim, A. S., & Solomon, D. H. (2010). Incentives for drug development—the curious case of colchicine. *The New England Journal of Medicine, 362*(22), 2045–2047. https://doi.org/10.1056/NEJMp1003126

Kessomboon, N., Limpananont, J., Kulsomboon, V., Maleewong, U., Eksaengsri, A., & Paothong, P. (2010). *Impact on access to medicines from TRIPS-Plus: A case study of Thai–US FTA*. *The Southeast Asian Journal of Tropical Medicine and Public Health, 41*(3), 667–677.

Keynes, J. M. (1936). *The general theory of employment, interest and money.* Harcourt Brace.

Kiger, M. E., & Varpio, L. (2020). Thematic analysis of qualitative data: AMEE Guide No. 131. *Medical Teacher, 42*(8), 846–854. https://doi.org/10.1080/0142159X.2020.1755030

Kim, Y. K., Lee, K., Park, W., & Choo, K. (2012). Appropriate intellectual property protection and economic growth in countries at different levels of development. *Research Policy, 41*(2), 358–375. https://doi.org/10.1016/j.respol.2011.09.003

Kılıç, P., Koçkaya, G., Yemşen, Ö., Tan, C., Öztunca, F. H., Aksungur, P., & Kerman, S. (2013). Orphan drug regulations in Turkey. *Journal of Pharmaceutical Health Services Research, 4*(3), 151–153. https://doi.org/10.1111/jphs.12018

Kittay, E. F. (2019). *Love's labor: Essays on women, equality and dependency* (2nd ed.). Routledge. https://doi.org/10.4324/9781315108926

Koçkaya, G., Atalay, S., Oğuzhan, G., Kurnaz, M., Ökçün, S., Sar Gedik, Ç., Şaylan, M., & Şencan, N. (2021). Analysis of patient access to orphan drugs in Turkey. *Orphanet Journal of Rare Diseases, 16*(1), 68. https://doi.org/10.1186/s13023-021-01718-3

Koçkaya, G., Oguzhan, G., Ökçün, S., & Kurnaz, M. (2023). Out-of-pocket healthcare expenditures of Turkish households living with rare diseases. *Frontiers in Public Health, 11*, 1051851. https://doi.org/10.3389/fpubh.2023.1051851

Korzeniewicz, R. P., & Moran, T. P. (2009). *Unveiling inequality: A world-historical perspective.* Russell Sage Foundation. http://www.jstor.org/stable/10.7758/9781610446587

Krishna, V. (2022). *Phantom plague: How tuberculosis shaped history.* Penguin Random House India Private Limited.

Kwan, Y. K., & Lai, E. L.-C. (2003). Intellectual property rights protection and endogenous economic growth. *Journal of Economic Dynamics and Control, 27*(5), 853–873. https://doi.org/10.1016/S0165-1889(02)00018-0

Kyle, M. K., & McGahan, A. M. (2012). *Investments in pharmaceuticals before and after TRIPS*. *The Review of Economics and Statistics, 94*(4), 1157–1172. https://www.jstor.org/stable/23355347

Kyle, M., & Qian, Y. (2014). Intellectual property rights and access to innovation: Evidence from TRIPS (NBER Working Paper No. 20799). *National Bureau of Economic Research*. https://www.nber.org/papers/w20799

Oksala, J. (2016). Affective labor and feminist politics. *Signs: Journal of Women in Culture and Society, 41*(2), 281–302. https://doi.org/10.1086/682920

Özveren, E. (2000). The scope of Turkish "semiperipherality". *Ekonomik Yaklaşım, 11*(36), 1–24.

Lai, E. L.-C. (1998). International intellectual property rights protection and the rate of product innovation. *Journal of Development Economics, 55*(1), 133–153. https://doi.org/10.1016/S0304-3878(97)00059-X

Lefebvre, S., Bürglen, L., Reboullet, S., Clermont, O., Burlet, P., Viollet, L., Benichou, B., Cruaud, C., Millasseau, P., & Zeviani, M. (1995). Identification and characterization of a spinal muscular atrophy-determining gene. *Cell, 80*(1), 155–165. https://doi.org/10.1016/0092-8674(95)90460-3

Léger, A. (2005). Intellectual property rights in Mexico: Do they play a role? *World Development, 33*(11), 1865–1879. https://doi.org/10.1016/j.worlddev.2005.07.003

Lerner, D. (1958). *The passing of traditional society: Modernizing the Middle East.* Free Press.

Lexchin, J., & Gagnon, M. A. (2014). CETA and pharmaceuticals: Impact of the trade agreement between Europe and Canada on the costs of prescription drugs. *Global Health, 10*(1), 30. https://doi.org/10.1186/1744-8603-10-30

Li, H., Yang, Y., Hong, W., Huang, M., Wu, M., & Zhao, X. (2020). Applications of genome editing technology in the targeted therapy of human diseases: Mechanisms, advances and prospects. *Signal Transduction and Targeted Therapy, 5*(1), Article 1. https://doi.org/10.1038/s41392-019-0089-y

Liu, M., & La Croix, S. (2014). *The impact of stronger property rights in pharmaceuticals on innovation in developed and developing countries* (Working Paper No. 201407). University of Hawaii at Manoa, Department of Economics. https://www.economics.hawaii.edu/research/workingpapers/WP_14-7.pdf

Lopez Gousset, V., & Bolz-Johnson, M. (2021). Global needs assessment study extract: Common needs of the rare disease population identified globally. https://www.rarediseasesinternational.org/wp-content/uploads/2021/10/final-Common-Needs-of-the-Rare-Disease-Population-Identified-Globally-report-extract.pdf

Löfgren, H., & Williams, O. D. (2013). The new political economy of pharmaceuticals: Conformity and resistance in the global South. In H. Löfgren & O. D. Williams (Eds.), *The new political economy of pharmaceuticals* (pp. 1–28). Palgrave Macmillan. https://doi.org/10.1057/9781137315854_1

Lukács, G. (1972). *History and class consciousness: Studies in Marxist dialectics* (R. Livingstone, Trans.). MIT Press. (Original work published 1923)

Luzzatto, L., Hyry, H. I., Schieppati, A., Costa, E., Simoens, S., Schaefer, F., Roos, J. C. P., Merlini, G., Kääriäinen, H., Garattini, S., Hollak, C. E., & Remuzzi, G. (2018). Outrageous prices of orphan drugs: A call for collaboration. *The Lancet, 392*(10149), 791–794. https://doi.org/10.1016/S0140-6736(18)31069-9

Lynch, K. (2021). *Care and capitalism.* John Wiley & Sons.

Lynch, K., & Kalaitzake, M. (2018). Affective and calculative solidarity: The impact of individualism and neoliberal capitalism. *European Journal of Social Theory, 23*(2), 238–257. https://doi.org/10.1177/1368431018786379

Lynch, K., Baker, J., & Lyons, M. (2016). *Affective equality: Love, care and injustice.* Springer.

Macdonald, I. (2011). Cold, cold, warm: Autonomy, intimacy and maturity in Adorno. *Philosophy & Social Criticism, 37*(6), 669–689. https://doi.org/10.1177/0191453711402940

Macpherson, C. B. (1962). *The political theory of possessive individualism.* Oxford University Press.

Mahadew, B. (2024). Reconciling patent rights and the human right to access to essential medicines: A critical review. *Emerging Perspectives: Human Rights, 2*(1), Article 1766. https://doi.org/10.59400/ephr1766

Malpani, R. (2009). All costs, no benefits: How the US-Jordan free trade agreement affects access to medicines. *Journal of Generic Medicines, 6*(3), 206–217. https://doi.org/10.1057/jgm.2009.13

Manu, T. (2017). Interpretation of TRIPS provisions in a manner consistent with human rights instruments: A policy option for the exploration of South–South judicial cooperation. *Oxford University Commonwealth Law Journal*, 17(1), 1–44. https://doi.org/10.1080/14729342.2017.1322850

Market Data Forecast. (2023). *Gene therapy market.* https://marketdataforecast.com/market-reports/gene-therapy-market

Marwaha, S., Knowles, J. W., & Ashley, E. A. (2022). A guide for the diagnosis of rare and undiagnosed disease: Beyond the exome. *Genome Medicine, 14*(1), 23. https://doi.org/10.1186/s13073-022-01026-w

Maskus, K. E. (2000). Intellectual property rights and foreign direct investment. *SSRN Electronic Journal.* https://doi.org/10.2139/ssrn.231122

Maskus, K. (2014). The new globalisation of intellectual property rights: What's new this time? *Australian Economic History Review, 54*(3), 262–284. https://doi.org/10.1111/aehr.12049

Mau, S. (2015). *Inequality, marketization and the majority class: Why did the European middle classes accept neo-liberalism?* Springer.

Mbiti, J. (1969). *African religions and philosophy.* Heinemann.

McClelland, D. C. (1976). *The achieving society.* Irvington. (Original work published 1961)

Melnikova, I. (2012). Rare diseases and orphan drugs. *Nature Reviews Drug Discovery, 11*(4), 267–268. https://doi.org/10.1038/nrd3654

Méndez, S. J. (2018). Parallel trade of pharmaceuticals: The Danish market for statins. *Health Economics, 27*(2), 333–356. https://doi.org/10.1002/hec.3551

Mielants, E. (2012). The great transition debate and world-systems analysis. In S. Babones, C. Chase-Dunn, & E. Mielants (Eds.), *Routledge handbook of world-systems analysis* (pp. 56–61). Routledge.

Miller, J. E., Mello, M. M., Wallach, J. D., Gudbranson, E. M., Bohlig, B., Ross, J. S., Gross, C. P., & Bach, P. B. (2021). Evaluation of drug trials in high-, middle-, and low-income countries and local commercial availability of newly approved drugs. *JAMA Network Open, 4*(5), e217075. https://doi.org/10.1001/jamanetworkopen.2021.7075

Mises, L. von. (2010). *The theory of money and credit.* Liberty Fund. https://oll.libertyfund.org/title/mises-the-theory-of-money-and-credit (Original work published 1912)

Moir, H. V. J., Tenni, B., Gleeson, D., & Lopert, R. (2016). *The Trans Pacific Partnership Agreement and access to HIV treatment in Vietnam*. *Global Public Health, 13*(4), 400–413. https://doi.org/10.1080/17441692.2016.1256418

Moon, S., Bermudez, J., & 't Hoen, E. (2012). Innovation and access to medicines for neglected populations: Could a treaty address a broken pharmaceutical R&D system? *PLOS Medicine, 9*(5), e1001218. https://doi.org/10.1371/journal.pmed.1001218

Moore, J. W. (2010). "Amsterdam is standing on Norway" part II: The global north Atlantic in the ecological revolution of the long seventeenth century. *Journal of Agrarian Change, 10*(2), 188–227.

Moore, P., & Dannreuther, C. (2009). Turkey in the world system and the new orientalism. In O. Worth & P. Moore (Eds.), *Globalization and the 'new' semi-peripheries* (pp. 138–156). Palgrave Macmillan. https://doi.org/10.1057/9780230245167_9

Moultrie, F., Chiverton, L., Hatami, I., Lilien, C., & Servais, L. (2025). Pushing the boundaries: Future directions in the management of spinal muscular atrophy. *Trends in Molecular Medicine, 31*(4), 307–318. https://doi.org/10.1016/j.molmed.2024.12.006

Muzaka, V. (2011). The politics of intellectual property rights and access to medicines: Some empirical and theoretical issues. In *The politics of intellectual property rights and access to medicines* (pp. 1–17). Palgrave Macmillan. https://doi.org/10.1057/9780230306158_1

Müller, B. (2019). The careless society—Dependency and care work in capitalist societies. *Frontiers in Sociology, 3*, Article 44. https://doi.org/10.3389/fsoc.2018.00044

Nelson, R. E., McAdam-Marx, C., Evans, M. L., Ward, R., Campbell, B., Brixner, D., & Lafleur, J. (2011). Patent extension policy for paediatric indications: An evaluation of the impact within three drug classes in a state Medicaid programme. *Applied Health Economics and Health Policy, 9*(3), 171–181. https://doi.org/10.2165/11539060-000000000-00000

Neves, P. C., Afonso, O., Silva, D., & Sochirca, E. (2021). The link between intellectual property rights, innovation, and growth: A meta-analysis. *Economic Modelling, 97*, 196–209. https://doi.org/10.1016/j.econmod.2021.01.019

Nguengang Wakap, S., Lambert, D. M., Olry, A., Rodwell, C., Gueydan, C., Lanneau, V., Murphy, D., Le Cam, Y., & Rath, A. (2020). Estimating cumulative point prevalence of rare diseases: Analysis of the Orphanet database. *European Journal of Human Genetics, 28*, 165–173. https://doi.org/10.1038/s41431-019-0508-0

Novartis. (2024). *First quarter 2024: Condensed interim financial report – Supplementary data.* https://www.novartis.com/sites/novartis_com/files/2024-04-interim-financial-report-en.pdf

Nuijten, M. (2022). Pricing Zolgensma—the world's most expensive drug. *Journal of Market Access & Health Policy, 10*(1), 2022353. https://doi.org/10.1080/20016689.2021.2022353

Nussbaum, M. C. (1995). Emotions and women's capabilities. In M. C. Nussbaum & J. Glover (Eds.), *Women, culture, and development: A study of human capabilities* (pp. 360–395). Oxford University Press. https://doi.org/10.1093/0198289642.003.0015

Odagiri, H., Goto, A., Sunami, A., & Nelson, R. R. (Eds.). (2010). *Intellectual property rights, development, and catch-up: An international comparative study.* Oxford University Press. https://doi.org/10.1093/acprof:oso/9780199574759.001.0001

Okin, S. M. (1989). *Justice, gender, and the family.* Basic Books.

Öniş, Z. (1998). *State and market: The political economy of Turkey in comparative perspective.* Boğaziçi University Press.

Özdemir, Y., Arısoy, R., Semiz, A., Şanlıkan, F., Akar, G., & Çağ, M. (2022). Carrier frequency of spinal muscular atrophy in Turkish population. *Perinatal Journal, 30*(1), 57–60. https://doi.org/10.2399/prn.22.0301012

Özkan, M. S. (2022). Constitution of values and subjects in personalized medicine: The case of spinal muscular atrophy [Master's thesis, Technical University of Munich]. mediaTUM. https://mediatum.ub.tum.de/1693330

Öztürk, F., Okçun, S., Tibet, B., & Koçkaya, G. (2023). EE495: Cost of spinal muscular atrophy (SMA) management in Türkiye. *Value in Health, 26*(12, Suppl), S146–S147. https://doi.org/10.1016/j.jval.2023.09.760

Palley, T. I. (2005). From Keynesianism to neoliberalism: Shifting paradigms in economics. In A. Saad-Filho & D. Johnston (Eds.), *Neoliberalism: A critical reader* (pp. 20–29). Pluto Press.

Papageorgiadis, N., & Sharma, A. (2016). Intellectual property rights and innovation: A panel analysis. *Economics Letters*, 141, 70–72. https://doi.org/10.1016/j.econlet.2016.01.003

Parello, C. P. (2008). A North–South model of intellectual property rights protection and skill accumulation. *Journal of Development Economics, 85*(1–2), 253–281. https://doi.org/10.1016/j.jdeveco.2006.08.001

Parsons, T. (2005). *The social system* (B. S. Turner, Preface). Routledge. (Original work published 1951)

Patel, R., & Moore, J. W. (2018). *A history of the world in seven cheap things: A guide to capitalism, nature and the future of the planet.* Verso.

Pedrique, B., Strub-Wourgaft, N., Some, C., Olliaro, P., Trouiller, P., Ford, N., Pécoul, B., & Bradol, J. H. (2013). The drug and vaccine landscape for neglected diseases (2000–11): A systematic assessment. *The Lancet Global Health, 1*(6), e371–e379. https://doi.org/10.1016/S2214-109X(13)70078-0

Peet, R., & Hartwick, E. R. (2009). *Theories of development: Contentions, arguments, alternatives* (2nd ed.). Guilford Press.

Pelentsov, L. J., Laws, T. A., & Esterman, A. J. (2015). The supportive care needs of parents caring for a child with a rare disease: A scoping review. *Disability and Health Journal, 8*(4), 475–491. https://doi.org/10.1016/j.dhjo.2015.03.009

Perelman, M. (1998). *Class warfare in the information age*. St. Martin's Press.

Peukert, A. (2017). Intellectual property and development—Narratives and their empirical validity. *The Journal of World Intellectual Property, 20*(1–2), 2–23. https://doi.org/10.1111/jwip.12072

Pierzynowska, K., Kamińska, T., & Węgrzyn, G. (2020). One drug to treat many diseases: Unlocking the economic trap of rare diseases. *Metabolic Brain Disease, 35*(8), 1237–1240. https://doi.org/10.1007/s11011-020-00617-z

Plahte, J., & Reid-Henry, S. (2013). Immunity to TRIPS? Vaccine production and the biotechnology industry in Cuba. In H. Löfgren & O. D. Williams (Eds.), *The new political economy of pharmaceuticals* (pp. 61–81). Palgrave Macmillan. https://doi.org/10.1057/9781137315854_4

Pogge, T. (2023). Pharmaceutical patents and economic inequality. *Health and Human Rights, 25*(2), 199–204.

Postma, M. J., Noone, D., Rozenbaum, M. H., Carter, J. A., Botteman, M. F., Fenwick, E., & Garrison, L. P. (2022). Assessing the value of orphan drugs using conventional cost-effectiveness analysis: Is it fit for purpose? *Orphanet Journal of Rare Diseases, 17*, 157. https://doi.org/10.1186/s13023-022-02283-z

Qian, Y. (2007). Do national patent laws stimulate domestic innovation in a global patenting environment? A cross-country analysis of pharmaceutical patent protection, 1978–2002. *Review of Economics and Statistics, 89*(3), 436–453. https://doi.org/10.1162/rest.89.3.436

Rahmatian, A. (2010). Neo-colonial aspects of global intellectual property protection. *The Journal of World Intellectual Property, 12*(1), 40–74. https://doi.org/10.1111/j.1747-1796.2008.00349.x

Raley, S., Bianchi, S. M., & Wang, W. (2012). When do fathers care? Mothers' economic contribution and fathers' involvement in child care. *American Journal of Sociology, 117*(5), 1422–1459. https://doi.org/10.1086/663354

Rare Diseases International. (2022). *Operational description of rare diseases.* https://www.rarediseasesinternational.org/wp-content/uploads/2022/08/INFOGRAPHIC-OPERATIONAL-DESCRIPTION-OF-RARE-DISEASES.pdf

Republic of Turkey Ministry of Health. (2023a). *Spinal muscular atrophy carrier screening program field guide.* https://hsgm.saglik.gov.tr/depo/birimler/cocuk-ergen-sagligi-db/Programlar/EO_SMA_TASIYICI_TARAMA_PROGRAMI_SAHA_REHBERI.pdf

Republic of Turkey Ministry of Health. (2023b). *SMA Bilim Kurulu Toplantısına İlişkin Açıklama.* https://www.saglik.gov.tr/TR-94662/sma-bilim-kurulu-toplantisina-iliskin-aciklama.html

Ricardo, D. (2010). *On the principles of political economy, and taxation.* Project Gutenberg. https://www.gutenberg.org/ebooks/33310 (Original work published 1817)

Richards, D. G. (2015). *Intellectual property rights and global capitalism: The political economy of the TRIPS Agreement.* Routledge. (Original work published 2004)

Richter, T., Nestler-Parr, S., Babela, R., Khan, Z. M., Tesoro, T., Molsen, E., Hughes, D. A., & International Society for Pharmacoeconomics and Outcomes Research Rare Disease Special Interest Group. (2015). Rare disease terminology and definitions—A systematic global review: Report of the ISPOR Rare Disease Special Interest Group. *Value in Health, 18*(6), 906–914. https://doi.org/10.1016/j.jval.2015.05.008

Romero, M., & Pérez, N. (2016). Conceptualizing the foundation of inequalities in care work. *American Behavioral Scientist, 60*(2), 172–188. https://doi.org/10.1177/0002764215607572

Sabatini, M. T., Xia, T., & Chalmers, M. (2022). Pricing and market access challenges in the era of one-time administration cell and gene therapies. *Pharmaceutical Medicine, 36*(5), 265–274. https://doi.org/10.1007/s40290-022-00443-x

Sachs, J. (1992). The economic transformation of Eastern Europe: The case of Poland. *Economics of Planning, 25*, 5–19. https://doi.org/10.1007/BF00366287

Sagonowski, E. (2020, August 7). Roche gets a green light to challenge Biogen, Novartis with SMA med Evrysdi. *Fierce Pharma.* https://www.fiercepharma.com/pharma/roche-set-to-take-biogen-novartis-sma-evrysdi-approval

Sampat, B., & Williams, H. L. (2019). *How do patents affect follow-on innovation? Evidence from the human genome*. *The American Economic Review, 109*(1), 203–236. https://doi.org/10.1257/aer.20151398

Sandilands, K., Williams, A., & Rylands, A. J. (2022). Carer burden in rare inherited diseases: A literature review and conceptual model. *Orphanet Journal of Rare Diseases, 17*(1), 428. https://doi.org/10.1186/s13023-022-02561-w

Schafheutle, E. I., Hassell, K., Noyce, P. R., & Weiss, M. C. (2002). Access to medicines: Cost as an influence on the views and behaviour of patients. *Health & Social Care in the Community, 10*(3), 187–195. https://doi.org/10.1046/j.1365-2524.2002.00356.x

Scherer, F. M. (2010). Pharmaceutical innovation. In B. H. Hall & N. Rosenberg (Eds.), *Handbook of the economics of innovation* (Vol. 1, pp. 539–574). Elsevier. https://doi.org/10.1016/S0169-7218(10)01012-9

Scholz, R. (2009). Patriarchy and commodity society: Gender without the body. *Mediations, 27*(1–2), 123–142. http://www.mediationsjournal.org/articles/patriarchy-and-commodity-society

Schumpeter, J. A. (1934). *The theory of economic development: An inquiry into profits, capital, credit, interest, and the business cycle* (R. Opie, Trans.). Harvard University Press. (Original work published 1911)

Scopel, C. T., & Chaves, G. C. (2016). Initiatives to challenge patent barriers and their relationship with the price of medicines procured by the Brazilian Unified National Health System. *Cadernos de Saúde Pública, 32*(11), Article e00113815. https://doi.org/10.1590/0102-311X00113815

Sell, S. K. (2003). *Private power, public law: The globalization of intellectual property rights* (Vol. 88). Cambridge University Press. https://doi.org/10.1017/CBO9780511491665

Sell, S. K. (2007). TRIPS-Plus free trade agreements and access to medicines. *Liverpool Law Review, 28*(1), 41–75. https://doi.org/10.1007/s10991-007-9011-8

Sell, S. K. (2011). TRIPS was never enough: Vertical forum shifting, FTAs, ACTA, and TTP. *Journal of Intellectual Property Law, 18*(2), 447–478. https://digitalcommons.law.uga.edu/jipl/vol18/iss2/5

Semin, S., & Güldal, D. (2008). Globalization of the pharmaceutical industry and the growing dependency of developing countries: The case of Turkey. *International Journal of Health Services: Planning, Administration, Evaluation, 38*(2), 379–398. https://doi.org/10.2190/HS.38.2.i

Sevenhuijsen, S. (1998). *Citizenship and the ethics of care: Feminist considerations on justice, morality and politics*. Routledge.

Shadlen, K. C., Sampat, B. N., & Kapczynski, A. (2019). Patents, trade and medicines: Past, present and future. *Review of International Political Economy, 27*(1), 75–97. https://doi.org/10.1080/09692290.2019.1624295

Shaffer, E. R., & Brenner, J. E. (2009). A trade agreement's impact on access to generic drugs. *Health Affairs, 28*(5), w957–w968. https://doi.org/10.1377/hlthaff.28.5.w957

Şimşek, D. C., & Çetin, K. D. K. (2025). Emotions experienced by parents whose children have spinal muscular atrophy: A qualitative research. *Journal of Pediatric Nursing, 80*, e111–e119. https://doi.org/10.1016/j.pedn.2024.11.027

Smith, A. (2010). *The theory of moral sentiments.* Penguin. (Original work published 1759)

Smith, A. (1937). *The wealth of nations* (M. Lerner, Ed.). Modern Library. (Original work published 1776)

Sonderholm, J. (2010). Intellectual property rights and the TRIPS Agreement: An overview of ethical problems and some proposed solutions (Policy Research Working Paper No. 5228). World Bank. https://doi.org/10.1596/1813-9450-5228

Stevens, D., Claborn, M. K., Gildon, B. L., Kessler, T. L., & Walker, C. (2020). Onasemnogene abeparvovec-xioi: Gene therapy for spinal muscular atrophy. *The Annals of Pharmacotherapy, 54*(10), 1001–1009. https://doi.org/10.1177/1060028020914274

Sweet, C. M. (2013). The political economy of pharmaceutical production in Brazil. In H. Löfgren & O. D. Williams (Eds.), *The new political economy of pharmaceuticals* (pp. 29–47). Palgrave Macmillan. https://doi.org/10.1057/9781137315854_2

Sweet, C., & Eterovic Maggio, D. S. (2015). Do stronger intellectual property rights increase innovation? *World Development, 66*, 665–677. https://doi.org/10.1016/j.worlddev.2014.08.025

Tabb, W. K. (2004). *Economic governance in the age of globalization.* Columbia University Press.

Tenni, B., Moir, H. V. J., Townsend, B., Gleeson, D., Lopert, R., & Moir, H. (2022). What is the impact of intellectual property rules on access to medicines? A systematic review. *Global Health, 18*, 40. https://doi.org/10.1186/s12992-022-00826-4

The Lancet Global Health. (2021). Now and then: Lessons from the rollout of ART. *The Lancet Global Health, 9*(6), e721. https://doi.org/10.1016/S2214-109X(21)00222-9

Thielen, F. W., Heine, R. J. S. D., van den Berg, S., ter Ham, R. M. T. T., & de Groot, C. A. U. (2022). Towards sustainability and affordability of expensive cell and gene therapies? Applying a cost-based pricing model to estimate prices for Libmeldy and Zolgensma. *Cytotherapy, 24*(12), 1245–1258. https://doi.org/10.1016/j.jcyt.2022.09.002

't Hoen, E. (2009). *The global politics of pharmaceutical monopoly power: Drug patents, access, innovation and the application of the WTO Doha Declaration on TRIPS and public health.* AMB.

't Hoen, E., Berger, J., Calmy, A., & Moon, S. (2011). Driving a decade of change: HIV/AIDS, patents and access to medicines for all. *Journal of the International AIDS Society, 14*(15), 1–12. https://doi.org/10.1186/1758-2652-14-15

't Hoen, E. F., Veraldi, J., Toebes, B., & Hogerzeil, H. V. (2018). Medicine procurement and the use of flexibilities in the Agreement on Trade-Related Aspects of Intellectual Property Rights, 2001–2016. *Bulletin of the World Health Organization, 96*(3), 185–193. https://doi.org/10.2471/BLT.17.199364

Timmermann, C. A., & van den Belt, H. (2013). Intellectual property and global health: From corporate social responsibility to the access to knowledge movement. *Liverpool Law Review, 34*(1), 47–73. https://doi.org/10.1007/s10991-013-9129-9

Trachtenberg, D., Kaplan, W. A., Wirtz, V. J., & Gallagher, K. P. (2019). The effects of trade agreements on imports of biologics: Evidence from Chile. *Journal of Globalization and Development, 10*(2), 1–21. https://doi.org/10.1515/jgd-2019-0030

Tronto, J. C. (1993). *Moral boundaries: A political argument for an ethic of care.* Routledge.

Tronto, J. C. (2017). There is an alternative: Homines curans and the limits of neoliberalism. *International Journal of Care and Caring, 1*(1), 27–43. https://doi.org/10.1332/239788217X14866281687583

Ünver, O., Öztürk, G., Güneş, A., Özçelik, A., Genç, H., Saltık, S., Akbeyaz, İ., Tekin Orgun, L., Karakayalı, B., Arslan, E., Cırdı, G., Ceylan, A. C., Gökdemir, Y., Akkaya, A., Erdem, E., Saygı, E., Karadağ, B., Kara, B., Topaloğlu, H., & Türkdoğan, D. (2024). 171P: Newborn spinal muscular atrophy screening: 2-year results of multi-center experience in Turkey. *Neuromuscular Disorders, 43*, 104441. https://doi.org/10.1016/j.nmd.2024.07.615

Uvey, D., Gokce, A. N., & Basagaoglu, I. (2004). Pharmaceutical industry in Turkey. *JISHIM: Journal of the International Society for the History of Islamic Medicine, 3*(5), 46–49.

Vanni, N. (2016). Narratives and counter-narratives in pharmaceutical patent law making: Experiences from 3 developing countries (Doctoral dissertation, University of Warwick).

Vawda, Y. A. (2022). Compulsory licenses and government use: Challenges and opportunities. In C. M. Correa & R. M. Hilty (Eds.), *Access to medicines and vaccines* (pp. 73–104). Springer. https://doi.org/10.1007/978-3-030-83114-1_3

Velásquez, G. (2013). Trade agreements, intellectual property and access to medicines: An introduction. In *Intellectual property and access to medicines: Papers and perspectives* (pp. 1–11). South Centre. https://www.southcentre.int/wp-content/uploads/2016/05/Bk_2013_IP-and-Access-to-Medicines_EN.pdf (Original work published 2010)

Verhaart, I. E. C., Robertson, A., Wilson, I. J., Aartsma-Rus, A., Cameron, S., Jones, C. C., Cook, S. F., & Lochmüller, H. (2017). Prevalence, incidence and carrier frequency of 5q-linked spinal muscular atrophy: A literature review. *Orphanet Journal of Rare Diseases, 12*(1), 124. https://doi.org/10.1186/s13023-017-0671-8

Vill, K., Schwartz, O., Blaschek, A., Gläser, D., Nennstiel, U., Wirth, B., Burggraf, S., Röschinger, W., Becker, M., Czibere, L., Durner, J., Eggermann, K., Olgemöller, B., Harms, E., Schara, U., Kölbel, H., & Müller-Felber, W. (2021). Newborn screening for spinal muscular atrophy in Germany: Clinical results after 2 years. *Orphanet Journal of Rare Diseases, 16*(1), 153. https://doi.org/10.1186/s13023-021-01783-8

Wallerstein, I. (1974). The rise and future demise of the world capitalist system: Concepts for comparative analysis. *Comparative Studies in Society and History, 16*(4), 387–415. https://doi.org/10.1017/S0010417500007520

Wallerstein, I. (2011). *The modern world-system I: Capitalist agriculture and the origins of the European world-economy in the sixteenth century.* University of California Press. https://www.jstor.org/stable/10.1525/j.ctt1pnrj9 (Original work published 1974)

Walter, M. C., Chiriboga, C., Duong, T., Goemans, N., Mayhew, A., Ouillade, L., Oskoui, M., Quinlivan, R., Vázquez-Costa, J. F., Vissing, J., & Servais, L. (2021). Improving care and empowering adults living with SMA: A call to action in the new treatment era. *Journal of Neuromuscular Diseases, 8*(4), 543–551. https://doi.org/10.3233/JND-20061

Wang, C. M., Whiting, A. H., Rath, A., Anido, R., Ardigò, D., Baynam, G., Dawkins, H., Hamosh, A., Le Cam, Y., Malherbe, H., Molster, C. M., Monaco, L., Padilla, C. D., Pariser, A. R., Robinson, P. N., Rodwell, C., Schaefer, F., Weber, S., & Macchia, F. (2024). Operational description of rare diseases: A reference to improve the recognition and visibility of rare diseases. *Orphanet Journal of Rare Diseases, 19*, 334. https://doi.org/10.1186/s13023-024-03322-7

Watal, J. (2001). *Intellectual property rights in the WTO and developing countries*. Kluwer Law International.

Watal, J. (2015). Patents: An Indian perspective. In J. Watal & A. Taubman (Eds.), *The making of the TRIPS Agreement: Personal insights from the Uruguay Round negotiations* (pp. 81–90). World Trade Organization.

Weber, M. (1978). *Selections in translation* (W. G. Runciman, Ed.; E. Matthews, Trans.). Cambridge University Press.

Weber, M. (2013). *The Protestant ethic and the spirit of capitalism* (S. Kalberg, Trans. & Ed.). Routledge. (Original work published 1905)

Wilkinson, R. G., & Pickett, K. (2018). *The inner level: How more equal societies reduce stress, restore sanity and improve everybody's wellbeing*. Allen Lane.

Williams, H. L. (2013). Intellectual property rights and innovation: Evidence from the human genome. *Journal of Political Economy, 121*(1), 1–27. https://doi.org/10.1086/669706

Williams, M., & Moser, T. (2019). The art of coding and thematic exploration in qualitative research. *International Management Review, 15*(1), 45–55.

Woo, S., Jang, P., & Kim, Y. (2015). Effects of intellectual property rights and patented knowledge in innovation and industry value added: A multinational empirical analysis of different industries. *Technovation, 43–44*, 49–63. https://doi.org/10.1016/j.technovation.2015.03.003

World Economic Forum. (2020). *Global data access for solving rare disease—A health economics value framework.* https://www3.weforum.org/docs/WEF_Global_Data_Access_for_Solving_Rare_Disease_Report_2020.pdf

World Trade Organization. (2001). *Declaration on the TRIPS agreement and public health* (WT/MIN(01)/DEC/2). https://www.wto.org/english/thewto_e/minist_e/min01_e/mindecl_trips_e.htm

World Trade Organization. (1994). *Agreement on Trade-Related Aspects of Intellectual Property Rights (TRIPS).* https://www.wto.org/english/docs_e/legal_e/27-trips.pdf

Yamabhai, I., Mohara, A., Tantivess, S., Chaisiri, K., Teerawattananon, Y., & Thavorncharoensap, M. (2011). Government use licenses in Thailand: An assessment of the health and economic impacts. *Global Health*, 7, 28. https://doi.org/10.1186/1744-8603-7-28

Yılmaz, V. (2022). The contemporary politics of health in Turkey: Diverse actors, competing frames, and uneven policies. In G. M. Tezcür (Ed.), *The Oxford handbook of Turkish politics* (pp. 249–272). Oxford University Press. https://doi.org/10.1093/oxfordhb/9780190064891.013.37

Yin, N. (2023). Pharmaceuticals, incremental innovation and market exclusivity. *International Journal of Industrial Organization, 87*, 102922. https://doi.org/10.1016/j.ijindorg.2023.102922

Young, A., Menon, D., Street, J., Al-Hertani, W., & Stafinski, T. (2017). Exploring patient and family involvement in the lifecycle of an orphan drug: A scoping review. *Orphanet Journal of Rare Diseases, 12*(1), 188. https://doi.org/10.1186/s13023-017-0738-6

Yu, P. K. (2011). Are developing countries playing a better TRIPS game? *UCLA Journal of International Law and Foreign Affairs, 16*(2), 311–343. https://scholarship.law.tamu.edu/facscholar/394

Zamora, B., Maignen, F., O'Neill, P., Mestre-Ferrandiz, J., & Garau, M. (2019). Comparing access to orphan medicinal products in Europe. Orphanet Journal of Rare Diseases, 14, 95. https://doi.org/10.1186/s13023-019-1078-5