Treating rare diseases with justice: Implications of a human rights-based approach

Dagron, Stéphanie

How to cite

DAGRON, Stéphanie. Treating rare diseases with justice: Implications of a human rights-based approach. In: Revista tecnológica, 2025, vol. 37, n° 1, p. 104–120. doi: 10.37815/rte.v37n1.1282

This publication URL: https://archive-ouverte.unige.ch/unige:186110
Publication DOI: 10.37815/rte.v37n1.1282

https://doi.org/10.37815/rte.v37n1.1282
Original articles

Treating rare diseases with justice: Implications of a human rights-based approach

Tratamiento de las enfermedades raras con justicia: Implicaciones de un enfoque basado en derechos humanos

Stéphanie Dagron[1] https://orcid.org/0000-0001-7075-4447

[1]*Faculty of Law, Faculty of Medicine, Global Studies Institute, University of Geneva*, Geneva, Switzerland
stephanie.dagron@unige.ch

Sent: 2025/02/10
Accepted: 2025/06/03
Published: 2025/06/30

Abstract

The importance of health for social justice has increased since the end of the 20th century, particularly following the recognition in the 1995 Copenhagen Declaration that "people are entitled to a healthy and productive life in harmony with the environment." However, a majority of people worldwide still do not enjoy good health. Numerous efforts have been made by the international community to address this situation in line with the principles of equity and social justice. The agenda 2030 serves as a remarkable example of these efforts, positioning health at the centre of the program, acknowledging the existence of social determinants of health, and affirming the necessity to tackle the causes of health inequities and attend to the needs of disadvantaged groups. Individuals with rare diseases face specific risks and challenges primarily due to unmet health needs stemming from a lack of research and development of suitable medical technologies. In this article, we explore the reasons explaining the injustices faced by these patients, as well as ways in which a human rights-based approach to people with rare diseases can provide arguments in favour of States' action. By considering the right to science as a complementary right to the right to health, we argue that States must promote the science for treating rare diseases and render applications of science accessible and affordable, thereby contributing to the realisation of a just and equitable society.

Keywords: Access to health products, health equity, right to health, right to science, social justice.

Summary: Introduction, Methodology: analysis approach, Findings and Discussion, Conclusions.

How to cite: Dagron, S. (2025). Treating rare diseases with justice: Implications of a human rights-based approach. *Revista Tecnológica - Espol, 37(1), 104-120.* https://rte.espol.edu.ec/index.php/tecnologica/article/view/1282

Resumen

La importancia de la salud para la justicia social ha aumentado desde finales del siglo XX, en particular tras la Declaración de Copenhague de 1995: "las personas tienen derecho a una vida sana y productiva en armonía con el medio ambiente". La mayoría de las personas en el mundo aún no gozan de buena salud. La comunidad internacional ha realizado esfuerzos para abordar esta situación, según los principios de equidad y justicia social. La Agenda 2030 constituye un ejemplo de estos esfuerzos, al situar la salud en el centro del programa, reconocer la existencia de los determinantes sociales de la salud, afirmar la necesidad de abordar las causas de las inequidades en salud y atender las necesidades de los grupos desfavorecidos. Las personas con enfermedades raras enfrentan riesgos y desafíos específicos, principalmente debido a las necesidades de salud insatisfechas derivadas de la falta de investigación y desarrollo de tecnologías médicas adecuadas. En este artículo, se exploran las razones que explican las injusticias a las que se enfrentan estos pacientes, así como las maneras en que un enfoque hacia las personas con enfermedades raras basado en los derechos humanos puede proporcionar argumentos a favor de que los Estados actúen. Al considerar a la ciencia como un derecho complementario del derecho a la salud, se argumenta que los estados deben promover el progreso científico para tratar las enfermedades raras y hacer que las aplicaciones de la ciencia sean accesibles y asequibles, contribuyendo así a la creación de una sociedad justa y equitativa.

Palabras clave: Acceso a productos sanitarios, equidad en salud, derecho a la salud, derecho a la ciencia, justicia social.

Introduction

Rare diseases are "life-threatening or chronically debilitating diseases" (Council Recommendation, 2009, para. 1), resulting in high mortality rates and socioeconomic inequalities (Halley et al., 2023). Historically, the challenges faced by both the patients living with them and their families have rarely been viewed through the lens of social justice. This is surprising, considering the significant impact of such diseases on patients' quality of life and social well-being, as reported by patients themselves (Buckle, 2024), and the fact that the needs of these patients are largely ignored in almost all national health systems. Traditionally, indeed, health systems have prioritised health for all through the development of services and goods designed to address "the main health problems in the community" (Declaration of Alma Ata, 1978). Rare diseases, however, do not feature as one of a community's main health challenges. They are first characterised by a very low prevalence at a country level. In the United States of America (US) and Japan, for example, a rare disease is one that does not affect more than 200,000 (around 6 per 10,000) (Rare Disease Act, 2002) or 50,000 (around 4 per 10,000) (Japan Pharmaceutical Manufacturers Association, 2020) of the total population, respectively; in Europe, a disease is considered rare when it affects no more than five persons per 10,000 people (Regulation (EC) No 141/2000, 1999). Secondly, rare diseases are characterised by a high level of complexity. Most rare diseases are genetically related, but others, such as rare cancers, rare immune-related diseases, and rare poisonings, can be a result of "environmental exposure during pregnancy or later in life" (EESC, 2009, para. 3.1.4), including exposure to hazards in the workplace (Charbotel et al., 2014).

Treating rare diseases with justice means first acknowledging the particularities of the individuals concerned and, secondly, identifying the primary challenges they face and devising ways to address those challenges. The United Nations General Agency (UNGA) in recent years has followed a social justice-based approach for patients with rare diseases, acknowledging the high number of patients globally – indeed, a recent estimation showed that 300 million persons out of a total population of 8 billion are affected by a rare disease (DESA, 2022b), of which

more than half are children. It has also identified the challenges they face in their lives and developed a strategy grounded in human rights and the principle of social justice (UNGA, 2021).

Its first Resolution on rare diseases, adopted in 2021, is firmly rooted in the main international human rights instruments such as the Universal Declaration of Human Rights and the International Covenant on Economic, Social and Cultural Rights (ICESCR), and recognises "the fundamental importance of equity, social justice and social protection mechanisms" (UNGA, 2021). It also acknowledges the specific social and health challenges that affect the quality of life and social well-being of these individuals across various domains, "including but not limited to health, education, employment, and leisure" (UNGA, 2021).

To tackle these challenges, the UNGA proposes a roadmap for States and the international community, grounded in both human rights and the foundational commitment made by all States in 2015 to pursue sustainable development and prioritise those furthest behind. The right to the highest attainable standard of physical and mental health (hereafter referred to as the right to health), the right to an adequate standard of living, and the right to education – especially for children – are explicitly referenced by the General Assembly (UNGA, 2021). Conversely, the right to access, participate in, and benefit from science (hereafter referred to as the right to science), which is also guaranteed by the ICESCR, is not mentioned.

The reading of this Resolution allows us to recognise the importance of access to necessary healthcare services (including products) as a central social determinant of health for people living with a rare disease. This is rather singular concerning the results of public health research in the 21st century, highlighting the fact that, although universal access to adequate and culturally appropriate healthcare services is a source of overall health gains, properly addressing health inequities implies placing more emphasis on addressing social inequalities (Bueno de Mesquita & Forman, 2023). The importance of health care for this group is linked to the complexity of the health-related challenges that are particularly intricate in the case of rare diseases.

However, a number of obstacles, often interrelated, hinder access to healthcare for patients living with a rare disease. The first of these concerns weaknesses in the essential constitutive elements of the health system in the patient's country of origin, including a shortage of specialised healthcare professionals and a lack of information and awareness about rare diseases among those healthcare professionals who are available (Domaradzi & Walkowiak, 2021); financial barriers for accessing goods and services, including long-term care (ILO, 2021); finally, the absence of the requisite proper diagnostic tools and medical treatment options, as shown by analyses revealing that diagnostic tools and special medicines are only available for 5% of rare diseases (EU Commission, 2020). Second, and on a more structural basis, access to healthcare is impeded by the lack of scientific research, which is, in turn, having an impact on the training of specialised healthcare professionals, on the very existence of suitable treatments for the numerous rare diseases already identified (between 5,000 and 7,000), and, finally, on decisions for the coverage of existing treatment costs (Dagron, 2011a; Dagron, 2011b).

Concerning this last point, access to treatment and healthcare services is generally dependent on scientific evidence as the basis for financial allowance decisions. "Orphan drugs" – or drugs developed for patients with rare diseases – are generally very expensive, and access to them may be impossible for patients in countries without appropriate healthcare coverage.

In countries where the costs of at least basic healthcare services and medicines are partially or completely covered by the national healthcare system or health insurance system, access may also be difficult, as reimbursement decisions for medicines are typically based on cost-effectiveness considerations informed by scientific evidence. This is the case of Switzerland, where the mandatory health insurance system reimburses the cost of medicinal products included on a list of pharmaceutical specialities that is constituted based on scientific proof of their effectiveness, therapeutic value, and economic character (Swiss Federal Health Insurance Act, 1994). In some exceptional cases, the proof of a "possibility of improvement" instead of the proof of a "high therapeutic benefit" might be sufficient to allow for coverage (Ordinance on Health Insurance, 1995).

Methodology: analysis approach

Although a full exploration of all challenges encountered by patients suffering from a rare disease and their families – including but not limited to health– would be essential to allow for a complete understanding of their social, economic, or psychological causes, this article will focus solely on the healthcare-related challenges and the main human rights arguments. In the first part, we will explore the limitations of the approaches and strategies adopted so far to address the health needs of people with rare diseases (Part 1). We will then propose an analysis of recent changes at the global level in favour of health equity and justice and analyse the complementarity of specific human rights as a solid basis for patients' requests to their governments concerning the improvement of accessibility to and affordability of innovative tools and treatments (Part 2).

Findings and Discussion

Part 1 - Strategies for rare diseases: existing barriers to health equity

Since the end of the 20th century, only a few States or regions that host leading research-intensive biopharmaceutical companies have addressed rare diseases and the related challenges patients face in accessing healthcare services. More recently, the international community has also paid attention to this issue. The analysis of these efforts reveals that while States are generally becoming more aware of the necessity to aim for the realisation of health equity and justice based on a human rights-based approach to public health issues, the fight against rare diseases has not been linked with such an approach for very specific reasons.

Strategies for rare diseases: the traditionally limited influence of human rights

At national and regional levels, States' interest in combating rare diseases has taken the form of legislative instruments primarily aimed at encouraging the pharmaceutical industries to engage in Research and Development (R&D) for rare diseases. The first legislative act of this kind was the Orphan Drug Act (ODA), adopted by the US in 1983 (Orphan Drug Act, 1983), followed by a revision of Japan's Pharmaceutical Affairs Law in 1993, introducing provisions on the promotion of R&D and priority reviews for orphan drugs (Japan Pharmaceutical Manufacturers Association, 2020), and by the European Union (EU) in 1999, with the Regulation 141/2000 on orphan medicinal products (Regulation (EC) No 141/2000, 1999).

These acts contain similar incentives, including a reduction in fees payable to regulatory authorities for marketing authorisations, market exclusivity (at least for a few years), grants for research promotion, tax reductions, or special procedures for marketing authorisations (e.g., priority reviews for orphan drugs). For example, the US law (Caetano et al., 2021; Fagnan et al., 2014) provides seven years of market exclusivity for a drug designated as an orphan drug (Orphan Drug Act, 1983, § 527(a)(2)), grants and tax credits of up to 50%

for R&D expenses, and a waiver on user fees when applying to the Food and Drug Administration (FDA) (Orphan Drug Act, 1983, § 4(a)). The EU regulation provides a special contribution by the European Community, which allows partially or entirely waived fees for companies seeking to place an orphan drug on the market (Regulation (EC) No 141/2000, 1999). It also grants market exclusivity for 10 years to orphan drugs that have received marketing authorisation (Regulation (EC) No 141/2000, 1999). This market exclusivity effectively prohibits the Community and EU Member States from accepting other applications for marketing authorisation for the same therapeutic purpose (Regulation (EC) No 141/2000, 1999). Furthermore, targeted grants can also be allocated to support research and development (Regulation (EC) No 141/2000, 1999).

These laws have been supplemented by other instruments that address the challenges of conducting research in the context of rare diseases, particularly in cases involving limited numbers of adults and underage patients, by introducing procedures to facilitate the marketing of these specific drugs (Gupta et al., 2016). For instance, the EU introduced accelerated or conditional approval pathways allowing companies to obtain marketing authorisation based on simplified requirements for the quality, safety, and efficacy of these products (Commission Regulation (EC) 507/2006, 2006).

State and regional authorities have historically exercised caution in avoiding references to obligations arising from human rights, whether linked to the right to health or the right to science, as a justification for adopting the aforementioned legislation. While the US Act invokes a "public interest" (Orphan Drug Act, 1983, § 1(6)), the European regulation refers to the principle of equity and considers that "patients suffering from rare conditions should be entitled to the same quality of treatment as other patients" (Regulation (EC) No 141/2000, 1999, at op 2). This approach mirrors the traditional stance of the international community in the field of public health during the latter half of the 20th century and the early 21st century. Interventions by the UNGA in public health have traditionally been scarce up to the present day (Dagron, 2019).

However, the UNGA did intervene on an exceptional basis starting in 2000 and 2001, by including health issues in the Millennium Declaration (UNGA, 2000) and addressing the Acquired Immune Deficiency Syndrome (AIDS) epidemic caused by the Human Immunodeficiency Virus (HIV) (UN, 2001; ECOSOC, 2009). The UNGA has since focused its attention on health problems such as malaria (UNGA, 2001), sickle-cell anaemia (UNGA, 2008), tuberculosis (TB) (UNGA, 2023), non-communicable diseases (NCDs) (UNGA, 2018), rare diseases (UNGA, 2021), and other more general issues, such as global health as a foreign policy matter (UNGA, 2008), health as a central target for the realisation of the 2030 Agenda for Sustainable Development (UNGA, 2015) or universal health coverage (UHC) as an essential instrument for a healthier world for all (UNGA, 2019; UNGA, 2023b).

Except for HIV, only the more recent resolutions on TB, NCDs and UHC expressly frame the health issues at stake as human rights concerns. They also encourage States to adopt a strategy that relies on a human rights-based approach (HRBA) (London, 2008; Bustreo & Doebbler, 2020). The more recent Resolution on TB interestingly recognises the importance of the right to health in association with the right to science as essential human rights to be respected and promoted in this field (UNGA, 2023a).

Traditional barriers to the implementation of an HRBA for rare diseases

There are multiple reasons behind the absence of express references to human rights in general and the rights to health and science, more specifically, in international strategies and

national and regional legislative acts concerning rare diseases. The first reason is linked to the State's capacity to implement the numerous obligations related to both. Indeed, it should be understood that an obligation to promote these rights does not lead to the same consequences for all States; the existing socioeconomic differences between them render their promotion arguably a controversial goal since pharmaceutical R&D capacities are still scarce in some parts of the world (Vieira et al., 2023; Khan, 2021). This is even more true regarding the right to science, as underlined in 2011 by Chapman (Chapman, 2009). She considered that "poor countries and many middle-income countries lack the capacity to conduct scientific and technological research, to translate findings into useful applications, to evaluate and regulate their potentially harmful effects, to distribute them widely, and to make them affordable and accessible across geographic and populations boundaries" (Chapman, 2009, p. 31). The reality today is that only some countries host large R&D-based pharmaceutical companies (Schuhmacher et al., 2021).

This might change in the future, following the example of the successful development of capacities to produce the COVID-19 vaccine in South Africa, which resulted from global efforts to develop R&D in various regions of the world (Paremoer & Pollock, 2022). For the time being, however, only a few countries have the necessary capacities to conduct research for rare diseases and develop pharmaceutical products. Moreover, these capacities are protected through the global framework applicable to the protection of intellectual property rights, which has a significant impact on the availability and accessibility of all medicines (Hestermeyer, 2007). The complexity of the relationships between intellectual property regimes, human rights and the right to science, in particular, has been deconstructed by the Committee on Economic, Social and Cultural Rights (CESCR, 2020) as well as in recent literature (Yu, 2022; Plomer, 2021).

More importantly, a central barrier to the use of human rights arguments in favour of people with rare diseases and their specific healthcare needs is linked to the interpretations given in 2000 (CESCR, 2000) and 2020 (CESCR, 2020) to the rights to health and science by the CESCR, as well as by the Committee on the Rights of the Child (CRC) in charge of the implementation of the Convention on the Rights of the Child and of the right to health guaranteed in art. 24 (CRC, 2013).

The rights to health and science are intrinsically linked. It was only relatively recently that the right to science was developed (Mitchell, 2021) and its content concretely formalised (CESCR, 2020), while its implications in the context of health remain the subject of ongoing discussions and research (Donders, 2011; Chapman, 2009; Knoppers, 2024). Regarding its content, it is necessary to understand that this right may be interpreted as offering limited utility in the context of rare diseases, at least when considering the core obligations identified in the literature and the CESCR (CESCR, 2020). Indeed, the right to science is neither unlimited nor absolute (Donders, 2011; CESCR, 2020). This is particularly true in the field of health. There is indeed no general obligation for States to utilise all their resources to support every form of health-related research or to make all medical products and technologies available and accessible to address every health need under this right. On the contrary, and in line with the CESCR's interpretation, States must make choices and focus on priority healthcare needs, thereby prioritising prevalent diseases, those more commonly found within the population in terms of total number of cases. According to the CESCR, States must make sure that they use all available resources to "ensure access to those applications of scientific progress that are critical to the enjoyment of the right to health" (CESCR, 2020, para. 52). They also have to "ensure that in the allocation of public resources, priority is given to research in areas where there is the greatest need for scientific progress in health" (CESCR, 2020, para. 52).

The objective of fulfilling the priority healthcare needs of the population, which by definition excludes the specific needs of patients with (non-prevalent) rare disease, also clearly emerges from the interpretation given to the right to health by both the CESCR (Donders, 2011) and the CRC.

Under the CESCR in GC 14, the right enshrined in Art. 12.2 (d) ICESCR to health facilities, goods and services is to be understood as a right to equal and timely access to "appropriate treatment of prevalent diseases and illnesses" (CESCR, 2000, para. 17). Consequently, the "critical elements" for the enjoyment of the right to health are guided by this central objective. This is evident in how health technologies are identified: the Committee requires States to ensure the provision of the so-called "essential drugs" (CESCR, 2000, para. 17), defined by the WHO Action Programme on Essential Medicines as "those that satisfy the priority healthcare needs of a population (…) selected with due regard to disease prevalence and public health relevance, evidence of efficacy and safety and comparative cost-effectiveness" (WHO, 2021). This same rationale applies to the State's core obligations resulting from the right to health, which are related to the population's priority needs. For instance, the Committee explicitly states the obligation "to provide essential drugs" (CESCR, 2000, para. 43 (d)), "to adopt and implement a national public health strategy and plan of action, on the basis of epidemiological evidence, addressing the health concerns of the whole population" (CESCR, 2000, para. 43 (f)), "to provide immunisation against the major infectious diseases occurring in the community" (CESCR, 2000, para. 43 (b)), and "to take measures to prevent, treat and control epidemic and endemic diseases" (CESCR, 2000, para. 43 (c)).

The CRC, in its GC 15, adopts the same interpretation of States' obligations in relation to the right to health of the child, as guaranteed by Art. 24 of the Convention on the Rights of the Child (CRC, 2013). The CRC proposes a list of appropriate measures for the full implementation of children's right to health, specifically addressing prevalent health issues. The CRC, art. 24, paragraph 2 requires States to "put in place a process for identifying and addressing (…) issues relevant to children's right to health" (CRC, 2013, para. 32). States are therefore asked to conduct "an in-depth analysis of the current situation in terms of priority health problems and responses" and identify and implement "evidence-based interventions and policies that respond to key determinants and health problems" (CRC, 2013, para. 32).

Moreover, this approach frames the obligation to "take appropriate measures (…) to diminish infant and child mortality" enshrined in Art. 24, paragraph 2 (a), as an obligation to pay particular attention to specific causes of mortality for children under five, such as pneumonia, diarrhoeal disease and malaria (CRC, 2013, paras. 33-35). Similarly, the obligation "to ensure the provision of necessary medical assistance and health care to all children with an emphasis on the development of primary healthcare," enshrined in Art. 24, paragraph 2 (c), is interpreted as imposing an obligation upon States to "prioritise universal access for children to primary healthcare services" (CRC, 2013, para. 36), and to "make all essential medicines on the WHO Model Lists of Essential Medicines (…) available, accessible and affordable" (CRC, 2013, para. 37). Finally, art. 24, paragraph 2 (d) also includes an obligation that does specifically address the specific needs of patients with rare diseases: the obligation for States to "combat disease (…) through the application of readily available technology" (Convention on the Rights of the Child, 1989, para. 24 (2)(c)). This point is reiterated in the CRC reference regarding technologies for "immunisation against the common childhood diseases" or "essential antibiotics and antiviral drugs" (CRC, 2013, para. 41).

Part 2 - Time for a change of paradigm: implications of equity and justice for rare diseases

Despite the considerable number of patients living with a rare disease worldwide, they are not defined as people who suffer from a disease commonly found within a population. Consequently, they have so far been deprived of the full and equal enjoyment of their rights to health and to science, rights that are particularly relevant for the achievement of health equity and social justice. In this section, we argue that this should change due to an evolving interpretation of States' obligations towards patients with a rare disease, resulting from a strong emphasis on social justice at the international level in recent years. An approach that links the right to health and the right to science has strong implications for these patients' future.

Recognising Rare Disease Patients as a Vulnerable Group in Human Rights Law: an important step towards social justice

Human rights instruments are designed to protect all individuals, particularly those who are more vulnerable to violations of their fundamental rights. To date, patients with a rare disease have not been recognised by human rights bodies or courts as a group requiring particular attention. In its GC 15, the CRC only considers children belonging to "the poorest parts of the population and in developing States" as being in a vulnerable situation (CRC, 2013, para. 86). Here, the Committee aligns with the literature and the identification of disadvantaged individuals as being "those at the bottom of the economical [sic] and social scale" (Chapman, 2009, p. 14), evidently subject to multiple social, economic, environmental, legal and political risk factors, and not those who are disadvantaged due to a genetic disorder or other specific disease. In the same vein, the CESCR, in its GC 14, identifies women, children, older persons, persons with disabilities and Indigenous persons as the ones who should receive special protection (CESCR, 2000).

However, today, many arguments support a change of paradigm. The first is based on the evolution of discrimination over time. In its 2009 2009 General Comment on the principles of non-discrimination and equality as fundamental components of international human rights law, the CESCR stated that "the nature of discrimination varies according to context and evolves over time" (CESCR, 2009, para. 27). Accordingly, the lists of grounds developed in its general comments and concluding observations are not exhaustive, and other possible prohibited grounds – or sources of discrimination and marginalisation – can be recognised, and new groups requiring special protection may be identified. For example, individuals with HIV status have been progressively recognised as such a group due to the multiple restrictions imposed on their civil and political rights, as well as their economic, social and cultural rights (CESCR, 2009). Another example is given by the GC 25 with the recognition of lesbian, gay, bisexual, transgender, queer and intersex (LGBTQI) persons (Holzhacker, 2014) as a group deserving special protection, as have women, persons with disabilities, Indigenous people and persons – more particularly children – living in poverty (CESCR, 2009).

Patients living with a rare disease experience numerous forms of discrimination that are difficult to consider as "systemic" due to the rarity and specificity of their conditions at the national level. However, the lack of available and accessible diagnostic tools and medicines constitutes a considerable barrier to both their right to health and their right to science. This amounts to discrimination, which arguably justifies the recognition of these patients as requiring special protection, as well as the development of special procedures to facilitate the protection and promotion of their rights based on the procedures already mandated by the Human Rights Council on specific groups (children, Indigenous people, older people, etc.).

The second argument is linked to the recent attention given by the international community to the principle of social justice and the achievement of equity in health. While the

concept of equity in health has been at the centre of discussions conducted by the International Negotiating Body in charge of the development of a new pandemic legal instrument (WHO Collaborating Center, 2023) since 2022, the principle of social justice has been a cornerstone of the Sustainable Development Agenda 2030, adopted in 2015 by the UNGA (UNGA, 2015),. The so-called 2030 Agenda does not expressly refer to either international human rights norms or the rights to health or science. However, it sets out the strategies to be jointly followed by each nation and the international community, strategies inspired by international human rights instruments (Ebbeson & Hey, 2022), to achieve a common vision of a "just, equitable, tolerant, open and socially inclusive world in which the needs of the most vulnerable are met" (UNGA, 2015, para. 8).

It is within this framework that patients with rare diseases have been recognised as a special group, whose specific needs must be taken seriously. More precisely, rare diseases must be taken into account for the full realisation of UHC, which is also a central component of the 3rd Sustainable Development Goal on health and well-being (Dagron, 2022). In 2019, the UNGA expressly acknowledged an obligation for States to address rare diseases as part of UHC (UNGA, 2019, para 34). It also defined UHC as ensuring "that all people have access, without discrimination, to nationally determined sets of the needed promotive, preventive, curative, rehabilitative and palliative essential health services, and essential, safe affordable, effective and quality medicines and vaccines, while ensuring that the use of these services does not expose the users to financial hardship, with a special emphasis on the poor, vulnerable, and marginalised segments of the population" (UNGA, 2019, para. 8).

Moreover, for the European Union, patients with rare diseases belong to the vulnerable "segments of the population" deserving special treatment. For instance, the strong link between solidarity, Universal Health Coverage (UHC), and rare diseases is evident in the EU's public health strategies following the COVID-19 pandemic (EU Parliament, 2020; EU Commission, 2021). The same is true for the High Commissioner for Human Rights (HCHR) in a report on the contribution of human rights to the conceptualisation of UHC (HCHR, 2019). The HCHR notes that "even when access to some form of health care exists, coverage usually mirrors health issues as experienced by the general population, with little or no attention paid to the specific needs and rights of persons living with rare diseases" (HCHR, 2019, para. 29). The HCHR also highlights that "the paucity of medical and scientific knowledge about rare diseases drives [the]marginalisation [of the patients], with the result that many people remain undiagnosed and therapies are difficult to develop" (HCHR, 2019, para. 29) Finally, according to the HCHR, "rare diseases often attract stigma and discrimination, and many persons living with a rare disease find themselves excluded from participation in employment and from integrating fully and productively into society." (HCHR, 2019, para. 29) As a consequence, the HCHR encourages States to scale up their efforts to achieve UHC by 2030, including "efforts to address (…) rare diseases (…) as part of universal health coverage" (HCHR, 2019, para. 34).

It is worth noting that the designation of groups as requiring special attention or as being "vulnerable" has been recognised as potentially leading to stigmatisation, misplaced paternalism, or even essentialism (Peroni & Timmer, 2013). This risk, inherent in the concept of vulnerability, must be taken seriously. However, in the case of rare diseases, such risks do not appear to be prominent. The primary source of discrimination stems from the lack of medical and scientific knowledge rather than the specific and personal attributes of each patient. Their particularities are rooted in the complexity and severity of the disease they suffer from, not in characteristics such as gender, socioeconomic status or nationality (recognised as forbidden sources of discrimination) (ICCPR, 1966, art. 26; ICESCR, 1966, art. 2) – although they may also experience discrimination related to these characteristics. Moreover, these

patients are not deprived of their independence and have shown strong capacities to address their vulnerabilities, both as individuals asserting their rights in court (Dagron, 2011b) and collectively as a group. For instance, lobbying efforts from grassroots patient groups in advancing human rights-based approaches were the driving force behind the legislative efforts analysed above. The advocacy of these groups has helped hold their governments accountable for protecting patients' rights to life and health (Dunkle et al., 2010). A change of paradigm in how the needs of patients with rare diseases are comprehensively addressed through a human rights lens constitutes a remarkable step forward. With the implementation of the right to science, specific actions are no longer seen as political choices but as legal obligations for States.

Advancing equity in health for rare disease patients through human rights: what implications today?

The recognition of patients living with a rare disease as a group with specific risks and needs creates positive (legal) obligations for States to address all sources of risks and promote (substantive) equality. In light of the UNGA Resolutions on rare diseases, TB or NCDs, the CESCR interpretation of the right to science (CESCR, 2020), and academic discussions on invoking this right in specific contexts (Frick & Dang, 2021), the call to address the health needs of patients with rare diseases through a human rights lens has significant implications.

Although complementary, these implications differ depending on whether we consider the implementation of the right to health in conjunction with the right to adequate standards of living or the implementation of the right to science. The UNGA 2021 Resolution on rare diseases expressly refers to the first set of rights and identifies a two-fold, complementary goal for States addressing rare diseases.

Concerning the right to health, the UNGA emphasises that States must strengthen their health systems to ensure access to healthcare services that are safe, of the highest attainable quality, accessible, available and affordable, timely, and both clinically and financially integrated (UNGA, 2021).

Regarding the right to an adequate standard of living, the Resolution considers that States must address the root causes of social inequality and discrimination. These include specific social challenges related to education, employment, and leisure; achieving gender equality, particularly in access to healthcare services, education, and decent work; and ensuring access to social protection and assistance, including universal and equitable access to quality health services without financial hardship (UNGA, 2021).

When considering the implementation of the right to science, States must adopt a more systematic approach that combines measures designed to guide, support and enable scientific progress. First, States must guide research toward achieving specific goals for patients living with rare diseases. This involves addressing the role of private companies in decision-making related to research and accessibility. Although the challenges faced by patients with rare diseases are primarily the result of biological specificities, they are also exacerbated by a research structure that prioritises market objectives over healthcare needs. The pharmaceutical industry tends to define its research priorities based on the potential marketability of new products, which in turn depends on the number of potential future consumers, a country's economic purchasing power, and the capacity of health systems to deliver treatments (Trouiller et al., 2002).

Given the central role of the pharmaceutical industry in the research and development of technologies and treatments for rare diseases, it seems more necessary than ever to advocate

for the greater recognition of the human rights-related responsibilities of pharmaceutical companies, as mentioned in the "UN Guiding principles on business and human rights" adopted in 2011 by the Human Rights Council (UN Human Rights Council, Resolution 17/4). In 2008, the UN Special Rapporteur on the right to health, relying on the principles of equality and non-discrimination as expressions of social justice, had already urged private companies to "give particular attention to the needs of disadvantaged individuals, communities and populations, such as children, the elderly and those living in poverty" (UN Special Rapporteur, 2008, guideline 5). Although his focus at the time was on neglected diseases, the reasoning applies equally to rare diseases today.

Second, States must support the research and development of diagnostic tools and treatments. Such support can be the result of legislative instruments, as previously mentioned, that promote the research and development of orphan drugs. It can also be the result of promoting international contacts and cooperation in the scientific field, as required by Art.15 (4) of the ICESCR. As scientific progress becomes increasingly dependent on data and information exchange between countries and regions, States should promote cross-border scientific collaborations, which should also include the sharing of research results, as well as cooperation between the private and public sectors and among national, regional, and international authorities involved in rare diseases strategies. Art. 15 (4) of the ICESCR expressly refers to the benefits derived from international cooperation in the scientific field, and the CRC emphasises that the realisation of the child's right to health, especially in developing countries, depends on such collaboration (CRC, 2013).

Notably, several collaborative efforts are already in place. These include platforms such as *Orphanet,* created in 1997 through a cooperative initiative involving eight countries, including France, Germany and Switzerland and supported by the European Union (Orphanet, 1997; Kolkhir, 2023), and the *International Rare Diseases Research Consortium* launched in 2011 (International Rare Disease Research Consortium, 2011). Both initiatives are dedicated to collecting data and disseminating information on rare diseases, making significant contributions to addressing the diagnosis and treatment of affected patients.

Finally, States should adopt measures that enable patients not only to access the applications of scientific research (diagnostic tools and treatments) and the related knowledge and information (including research findings and data) (CESCR, 2020, paras. 16-17; CRC, 2013, paras. 5, 28; Frick & Dang, 2021), but also to be directly involved. This inclusion is part of the "participation" dimension of patients' rights (Frick & Dang, 2021). Patients living with rare diseases – as well as persons with disabilities, as understood by the CESCR, or citizens more generally (CESCR, 2020) – have the right to participate in the decision-making process, including those related to research priorities and broader public health strategies concerning access to the benefits of research.

The combination of these sets of measures is essential. To date, support for research and development alone, as recommended by the 2021 UNGA resolution (UNGA, 2021), has not ensured that people living with rare diseases can fully benefit from scientific progress. This has been demonstrated in the US and the EU, where legislation on rare diseases primarily addresses the costs of R&D (through financial incentives and extended intellectual property rights) while still allowing the industry's freedom to define which research and diseases to address.

On the one hand, evaluations in both regions reveal a certain degree of commitment to supporting patients living with rare diseases. For instance, analyses of US legislation have

shown that the adoption of the ODA encouraged research into rare diseases, as evidenced by a notable increase in approvals for rare disease products (Miller & Lanthier, 2018). Furthermore, a 2020 analysis by the European Commission has shown that since the 1999 Orphan Regulation, there has been a significant increase in the availability of orphan medicines and investments in rare disease research and development (R&D) (EU Commission, 2020).

On the other hand, these same analyses highlight that this increase in the number of available drugs has not translated into broader coverage of specific patient needs (Miller & Lanthier, 2018). According to the European Commission's evaluations of regulations on rare diseases and paediatric research, "unmet medical need[s]" have not been properly addressed, as for the large majority of rare diseases, there still is no treatment available (EU Commission, 2020, p. 34). The report concluded that the legislative instruments "have not done enough to direct the development [of drugs] in [these] areas" (EU Commission, 2020, p. 34); it also noted that "product development tends to cluster around certain (more profitable) therapeutic areas." As a result, "the number of treatment options is expanding for some conditions", such as certain cancers (EU Commission, 2020, p. 41). In response, in 2022, the European Economic and Social Committee expressed its disappointment in the progress made and called "for a comprehensive European approach that takes into account all the needs of people with rare diseases" (EESC, 2022, para. 1.1).

Conclusions

Patients living with a rare disease face not only fundamental discrimination, manifested in the lack of diagnostic tools and treatments and the resulting inability to enjoy their right to health and benefit from scientific progress, but also additional forms of discrimination based on socioeconomic status, gender, age, and other factors. They are also subjected to a deep injustice stemming from the specific characteristics of their condition.

Taking action to fulfil their human rights obligations means that States must intervene to strengthen their health systems, address the root causes of inequality and discrimination, and direct scientific research towards unmet needs. This includes supporting research and development, particularly through the strengthening of international cooperation, and ensuring patients' access to diagnostic tools and treatment alongside their participation in the decision-making process.

Acting in accordance with the principles of social justice demands that States prioritise the needs of patients with rare diseases, even when doing so may challenge cost-efficiency metrics used in broader public health policies. Access to quality health services and essential medicinal products without financial hardship is a significant challenge for a large part of the world's population today (DESA, 2022a; ILO, 2021). Even in high-income countries, trade-offs are always present in healthcare, as no system operates with unlimited resources. Until now, States have favoured those whose needs they consider the most pressing based on epidemiological information and central objectives, such as maternal and child health, health security, and epidemic control (CESCR, 2000). However, when guided by specific values such as solidarity, health equity and social justice, adopting an HRBA to healthcare introduces additional obligations into the equation. The specific healthcare needs of patients with rare diseases must be given the same level of priority as those of any other group whose needs have traditionally been viewed as most urgent. In light of ongoing global health reforms, it is time to integrate rare disease justice into mainstream health and human rights strategies.

Acknowledgements

The author wishes to express her gratitude to the participants and organisers of the Symposium on the Right to Science with a Focus on Health (in particular to Samantha Besson, Gérard Escher and Bartha Knopper), which took place at the Brocher Foundation in Switzerland in December 2021, for their extensive discussions on the right to science. Special thanks to Patrick Taran for his insightful contributions to social justice and to Pauline Roy and Elizabeth Joanne Schwarczer for their bibliographic research and language editing support.

References

Buckle N., Rogers Y., O'Toole D., McNulty S., Kroll T., Gibbs L., Somanadhan S. (2024). A qualitative exploration of children's lives with rare diseases. *Child Care Health Dev.,* 50(4), p. 1-11, https://doi.org/10.1111/cch.13294

Bueno de Mesquita, J., & Forman, L. (2023). Human Rights, Equity and Social Justice in Global Health. In L. O. Gostin, B. M. Meier (Eds.), *Global Health Law & Policy, Ensuring Justice for a Healthier* World (pp. 91-118). Oxford University Press.

Bustreo, F., & Doebbler, C. F. J. (2020). The Rights-Based Approach to Health. In L. O. Gostin, B. M. Meier (Eds.), *Foundations of Global Health & Human Rights* (pp. 89-110). Oxford University Press.

Caetano, R., Correa, M. C. D. V., Villardi P., Almeida Rodrigues P. H., & Osorio-de-Castro, C. G. S. (2021). Dynamics of patents, orphan drug designation, licensing, and revenues from drugs for rare diseases: The market expansion of eculizumab. *PLOS ONE,* 16(3), Article e0247853. https://doi.org/10.1371/journal.pone.0247853

Chapman, A. (2009). Towards an Understanding of the Right to Enjoy the Benefits of Scientific Progress and its Applications. *Journal of Human Rights,* 8(1), 1-36. http://dx.doi.org/10.1080/14754830802701200

CESCR, *General Comment No. 14: The Right of the Highest Attainable Standard of Health (Art. 12)*, adopted 11 August 2000, UN Doc E/C.12/2000/4. https://www.ohchr.org/sites/default/files/Documents/Issues/Women/WRGS/Health/GC14.pdf

CESCR, *General comment No. 25 (2020) on science and economic, social and cultural rights (article 15 (1)(b), (2),(3) and (4) of the International Covenant on Economic, Social and Cultural Rights*, adopted 20 April 2020, UN Doc E/C.12/GC/25. https://documents.un.org/doc/undoc/gen/g20/108/12/pdf/g2010812.pdf

Charbotel, B., Fervers, B., Droz, J. P. (2014). Occupational exposures in rare cancers: A critical review of the literature. *Critical Reviews in Oncology/Hematology*, 90, 99-134.

Committee on Economic, Social and Cultural Rights. (2009, July 2). *General Comment No. 20: Non-Discrimination in Economic Social and Cultural Rights (art. 2, para 2 of the International Covenant of Economic Social and Cultural Rights)*, (E.C.12/GC/20). https://digitallibrary.un.org/record/659980?v=pdf

Convention on the Rights of the Child, UN Doc A/RES/44/25 (1989). https://www.ohchr.org/sites/default/files/crc.pdf

Council of the European Union. (2009, June 8). *Council Recommendation of 8 June 2009 on an action in the field of rare diseases* (2009/C 151/07). Official Journal of European Union. https://eur-lex.europa.eu/LexUriServ/LexUriServ.do?uri=OJ:C:2009:151:0007:0010:EN:PDF

CRC, *General Comment No. 15 (2013) on the right of the child to the enjoyment of the highest attainable standard of health (art. 24)*, adopted 17 April 2013, UN Doc CRC/C/GC/15. https://documents.un.org/doc/undoc/gen/g13/428/14/pdf/g1342814.pdf

Dagron, S. (2011). Droit à la santé et jurisprudence constitutionnelle : les enseignements de l'expérience allemande. In Faculté de droit et des sciences sociales de Poitiers, *Les voyages du droit : Mélanges en l'honneur de Dominique Breillat* (pp. 155-169). LGDJ.

Dagron, S. (2011). Maladies rares et financement des traitements médicamenteux innovants en Suisse entre rationalisation et rationnement : commentaire de l'arrêt du Tribunal fédéral du 23 novembre 2010 (Myozym*). Bioethica Forum* 3, 110-111.

Dagron, S. (2019). Le droit à la santé, un droit internationalement protégé. In Société française pour le droit international (Ed.), *Santé et droit international* (pp. 51-72). Pedone.

Dagron, S. (2022). SDG 3: Ensure Healthy Lives and Promote Well-Being for All at All Ages. In E. Hey, & J. Ebbesson (Eds.), *Cambridge Handbook of The Sustainable Development Goals and International Law*. (pp. 95-116). Cambridge University Press.

DESA. (2022a). The Sustainable Development Goals Report. https://unstats.un.org/sdgs/report/2022/The-Sustainable-Development-Goals-Report-2022.pdf

DESA. (2022b). World Population Prospects 2022, Summary of Results (New York: United Nations, 2022). https://www.un.org/development/desa/pd/sites/www.un.org.development.desa.pd/files/wpp2022_summary_of_results.pdf

Domaradzi, J., & Walkowiak, D. (2021, May 28). Knowledge and Attitudes of Future Healthcare Professionals Toward Rare Diseases. *Frontiers in Genetics*, 12, Article 639610. https://doi.org/10.3389/fgene.2021.639610

Donders, Y. (2011). The Right to Enjoy the Benefits of Scientific Progress: in Search of State Obligations in Relation to Health. *Medicine, Health Care and Philosophy*, 14(4), 371-378. https://doi.org/10.1007/s11019-011-9327-y

Dunkle M., Pines W., & L. Saltonstall P. L. (2010). Advocacy Groups and Their Role in Rare Diseases Research. In M. P. de la Paz and S. C. Groft (Eds.). *Rare Diseases Epidemiology: Advances in Experimental Medicine and Biology* (pp. 515-525). Springer.

Ebbesson, J., Hey, E. (2022). Introduction: The Sustainable Development Goals, Agenda 2030 and international law. In E. Hey, & J. Ebbesson (Eds.), *Cambridge Handbook of The Sustainable Development Goals and International Law* (pp. 1-49). Cambridge University Press.

Economic and Social Council, *Report of the United Nations High Commissioner for Human Rights*, 2019 session, adopted 15 May 2019, UN Doc E/2019/52 (15 May 2019). https://documents.un.org/doc/undoc/gen/g19/139/41/pdf/g1913941.pdf

Economic and Social Council, *Resolution 2009/6: Joint United Nations Programme on HIV/AIDS (UNAIDS)*, adopted 24 July 2009, UN Doc E/RES/2009/6). https://www.un.org/en/ecosoc/docs/2009/resolution%202009-6.pdf

European Commission. (2006). *Commission Regulation (EC) 507/2006 of 29 March 2006 on the conditional marketing authorisation for medicinal products for human use falling within the scope of Regulation (EC) No 726/2004 of the European Parliament and of the Council*. Official Journal of the European Union. https://eur-lex.europa.eu/LexUriServ/LexUriServ.do?uri=OJ:L:2006:092:0006:0009:EN:PDF

European Commission. (2020, August 11), *Commission staff working document: Joint evaluation of Regulation (EC) No 1901/2006 of the European Parliament and of the Council of 12 December 2006 on medicinal products for paediatric use and Regulation (EC) No 141/2000 of the European Parliament and of the Council of 16 December 1999 on orphan medicinal products* (SWD (2020) 164 final, part 1/6). https://eur-lex.europa.eu/resource.html?uri=cellar:e9a9fff0-dbd9-11ea-adf7-01aa75ed71a1.0001.02/DOC_1&format=PDF

European Commission. (2021, March 4). *The European pillar of social Rights Action Plan* (SWD (2021) 46 final). https://eur-lex.europa.eu/legal-content/EN/TXT/HTML/?uri=CELEX:52021DC0102

European Economic and Social Committee. (2009, September 11). *Opinion on the Proposal for a Council Recommendation on a European action in the field of rare diseases* (C 218/18). Official Journal of the European Union. https://eur-lex.europa.eu/legal-content/EN/TXT/?uri=CELEX:52009AE0346

European Economic and Social Committee. (2022, October 26). *Opinion: Ensuring strong European solidarity for rare disease patients* (SOC/729). https://www.eesc.europa.eu/en/our-work/opinions-information-reports/opinions/ensuring-strong-european-solidarity-rare-disease-patients

European Parliament. (2020, July 10). *Resolution on the EU's public health strategy post-COVID-19* (2021/C 371/13). Official Journal of the European Union. https://eur-lex.europa.eu/legal-content/EN/TXT/PDF/?uri=CELEX:52020IP0205

European Parliament and Council of the European Union (1999, December 16). *Regulation (EC) No 141/2000 of the European Parliament and of the Council of 16 December 1999 on orphan medicinal products*, L 18/1. Official Journal of the European Communities. https://eur-lex.europa.eu/legal-content/EN/TXT/HTML/?uri=CELEX:32000R0141

Fagnan, D. E., Gromatzky A. A., Stein R. M., Fernandez J.-M., & Lo A. W. (2014). Financing drug discovery for orphan diseases. *Drug Discovery Today*, 19(5), 533-538. https://doi.org/10.1016/j.drudis.2013.11.009

Frick M., & Dang G. (2021). The Right to Science: A Practical Tool for Advancing Global Health Equity. In H. Porsdam, & S. Porsdam Mann (Eds.), *The Right to Science, Then and Now* (pp. 246-267). Cambridge University Press.

Halley, M. C., Halverson C. M. E., Tabor H. K., & Goldenberg A. J. (2023). Rare Disease, Advocacy and Justice: Intersecting Disparities in Research and Clinical Care. *The American Journal of Bioethics*, 23(7), 17-26. https://doi.org/10.1080/15265161.2023.2207500

Hestermeyer, H. (2007). *Human Rights and the WTO: the Case of Patents and Access to Medicines*. Oxford University Press.

Holzhacker, R. (2014). Gay Rights are Human Rights: The Framing of New Interpretations of International Human Rights Norms. In G. Andreopoulos, & Z. F. K. Arat (Eds.), *The Uses and Abuses of Human Rights: A Critical Approach to Advocacy* (pp. 29-64). Palgrave Macmillan.

International Covenant on Civil and Political Rights, December 16, 1966. https://www.ohchr.org/sites/default/files/ccpr.pdf

International Covenant on Economic, Social and Cultural Rights, December 16, 1966. https://www.ohchr.org/sites/default/files/cescr.pdf

International Labour Organization (2021). *World Social Protection Report 2020-2022: Social protection at the crossroads – in pursuit of a better future*. Geneva, United Nations. https://www.ilo.org/sites/default/files/wcmsp5/groups/public/@ed_protect/@soc_sec/documents/publication/wcms_817572.pdf

International Rare Disease Research Consortium (n.d.). *Who we are*. Retrieved February 6, 2025, from https://irdirc.org/who-we-are

Japan Pharmaceutical Manufacturers Association. (2020). *Information on Japanese Regulatory Affairs, Pharmaceutical Administration and Regulations in Japan*. https://www.jpma.or.jp/english/about/parj/eki4g600000078c0-att/2020.pdf

Khan, U. (2021). Decolonizing Tuberculosis Care: A Perspective From The Global South On World TB Day. *Health Policy Watch*. https://healthpolicy-watch.news/84121-2/

Knoppers, B. M., Beauvais, M. J. (2024), Implementing the human right to science in the context of health: introduction to the special issue. *Journal of Law and the Biosciences*, 11(2), 1-8. https://doi.org/10.1093/jlb/lsae018

Kolkhir, P. (2023). An EU task force to assess the burden of rare diseases. *Nature Medicine,* 29(3), 516-517. https://doi.org/10.1038/s41591-023-02207-9

London, L. (2008). What is a Human-Rights Based Approach to Health and Does It Matter? *Health and Human Rights Journal*, 10(1), 65-80. http://dx.doi.org/10.2307/20460088

Miller, K., & Lanthier M. (2018). Investigating the Landscape of US Orphan Product Approvals. *Orphanet Journal of Rare Diseases*, 13(1), 183. https://doi.org/10.1186/s13023-018-0930-3

Mitchell, C. (2021). Epilogue: Tensions in the Right to Science Then and Now. In H. Porsdam, S. Porsdam Mann (Eds.), *The Right to Science, Then and Now* (pp. 286-297). Cambridge University Press.

Ordinance of 27 June 1995 on Health Insurance, SR 832.102 (1995). https://www.fedlex.admin.ch/eli/cc/1995/3867_3867_3867/fr

Orphan Drug Act of 1983, H.R. 5238, 97th Congress (1983). https://www.congress.gov/bill/97th-congress/house-bill/5238/text

Orphanet (1997). *The portal for rare diseases and orphan drugs*. Retrieved February 6, 2025, from https://www.orpha.net/consor/cgi-bin/index.php.

Paremoer, L., & Pollock, A. (2022). "A passion to change the landscape and drive a renaissance": The mRNA Hub at Afrigen as decolonial aspiration. *Frontiers in Public Health*, 10. https://doi.org/10.3389/fpubh.2022.1065993

Peroni, L., & Timmer, A. (2013). Vulnerable groups: The promise of an emerging concept in European Human Rights Convention law. *International Journal of Constitutional Law*, 11(4), 1056–1085. https://doi.org/10.1093/icon/mot042

Plomer, A. (2021). IP Rights and Human Rights, What History Tells Us and Why It Matters. In H. Porsdam, S. Porsdam Mann (Eds.), *The Right to Science, Then and Now* (pp. 54-75). Cambridge University Press.

Rare Disease Act of 2002, H.R. 4013, 107th Congress (2002). https://www.congress.gov/107/plaws/publ280/PLAW-107publ280.pdf

Schuhmacher, A., Wilisch L., Kuss M., Kandelbauer A., Hinder M., & Gassmann O. (2021). R&D efficiency of leading pharmaceutical companies – a 20-year analysis. *Drug Discovery Today*, 26(8), 1784-1789. https://doi.org/10.1016/j.drudis.2021.05.005

Swiss Federal Health Insurance Act of 18 March 1994, RS 832.10 (1994). https://www.fedlex.admin.ch/eli/cc/1995/1328_1328_1328/fr

Trouiller, P., Olliaro, P., Torreele, E., Orbinski, J., Laing, R., & Ford, N. (2002). Drug Development for Neglected Diseases: a Deficient Market and a Public-Health Policy Failure. *The Lancet*, 359(9324), 2188-2194. https://doi.org/10.1016/S0140-6736(02)09096-7

UN, *Declaration of Commitment on HIV/AIDS*, 26th Sess, adopted 27 June 2001. https://www.ohchr.org/en/instruments-mechanisms/instruments/declaration-commitment-hivaids

UNGA, *2001-2010 Decade to Roll Back Malaria in Developing Countries, Particularly in Africa*, 55th Sess, adopted 28 September 2001, UN Doc A/RES/55/284). https://digitallibrary.un.org/record/448002?ln=ru&v=pdf

UNGA, *Addressing the challenges of persons living with a rare disease and their families*., 76th Sess, adopted 16 December 2021, UN Doc A/RES/76/132. https://digitallibrary.un.org/record/3953765?v=pdf

UNGA, *Declaration on the Elimination of Discrimination against Women*, 22nd Sess, adopted 7 November 1967, UN Doc A/RES/22/2263. https://www.eods.eu/library/UNGA_Declaration%20Women_1967_en.pdf

UNGA, *Global Health and Foreign Policy*, 63rd Sess, adopted 26 November 2008, UN Doc A/RES/63/33. https://digitallibrary.un.org/record/642456?ln=fr&v=pdf

UNGA, *Political Declaration of the Third High-Level Meeting of General Assembly on the Prevention and Control of non-communicable diseases*, 73rd Sess, adopted 10 October 2018, UN Doc A/RES/73/2. https://digitallibrary.un.org/record/1648984?ln=fr&v=pdf

UNGA, *Political Declaration of the High-Level Meeting of the General Assembly on the Fight Against Tuberculosis*, 78th Sess, adopted 5 October 2023, UN Doc A/RES/78/5. https://digitallibrary.un.org/record/4025280?ln=fr&v=pdf

UNGA, *Political Declaration of the High-Level Meeting on Universal Health Coverage*, 74th Sess, adopted 10 October 2019, UN Doc A/RES/74/2. https://digitallibrary.un.org/record/3833350?ln=fr&v=pdf

UNGA, *Political Declaration of the High-Level Meeting on the Fight against Tuberculosis*, 78th Sess, adopted 16 October 2023 (2023a), UN Doc A/RES/78/5. https://documents.un.org/doc/undoc/gen/n23/306/91/pdf/n2330691.pdf

UNGA, *Political Declaration of the High-Level Meeting on Universal Health coverage*, 78th Sess, adopted 16 October 2023 (2023b), UN Doc A/RES/78/4. https://documents.un.org/doc/undoc/gen/n23/306/84/pdf/n2330684.pdf

UNGA, *Recognition of Sickle-Cell Anemia as a Public Health Problem*, 63rd Sess, adopted 22 December 2008, UN Doc A/RES/63/237. https://digitallibrary.un.org/record/644334/files/A_RES_63_237-EN.pdf

UNGA, *Transforming Our World: The 2030 Agenda for Sustainable Development*, 70th sess, adopted 25 September 2015, UN Doc A/RES/70/1. https://www.un.org/en/development/desa/population/migration/generalassembly/docs/globalcompact/A_RES_70_1_E.pdf

UNGA, *United Nations Millennium Declaration*, 55th Sess, adopted 18 September 2000, UN Doc A/RES/55/2. https://www.un.org/en/development/desa/population/migration/generalassembly/docs/globalcompact/A_RES_55_2.pdf

UN Human Rights Council, *Resolution 17/4 endorsing the "Guiding Principles on Business and Human Rights"*, 16 June 2011

UN Special Rapporteur on the right to the highest attainable standard of health, *Human Rights Guidelines for Pharmaceutical Companies in relation to Access to Medicines*, 11 August 2008, UN Doc A/63/263. https://docs.un.org/en/A/63/263

Vieira, M., Andia, T., Karim, O., Srishti, S. A., Pineda, S. A., Ruiz, A. A., Large, K., Liu, Y., Moon, S., Naher, N., Siddiqui, A., & Ahmed, S. M. (2023). Rising pharmaceutical innovation in the Global South: a landscape study. *Journal of Pharmaceutical Policy and Practice*, 16(155), 1-19. https://doi.org/10.1186/s40545-023-00669-3

World Health Organization. (2021). *Model List of Essential Medicines, 22nd List* (WHO/MHP/HPS/EML/2021.02). https://iris.who.int/bitstream/handle/10665/345533/WHO-MHP-HPS-EML-2021.02-eng.pdf

World Health Organization Collaborating Center. (2023). *Global Consultation on Equity Models for a Pandemic Agreement in support of the World Health Organization and the Intergovernmental Negotiating Body: Advancing a World Together Equitably* (Geneva, United Nations,). https://oneill.law.georgetown.edu/wp-content/uploads/2023/02/P7_ONL_Pandemic_Equity_Models_-1.pdf

Yu, P. K. (2022). Can the Right to Science Reduce the Tensions Between Intellectual Property and Human Rights. *Texas A&M University School of Law,* Legal Studies Research Paper No 22-56. https://papers.ssrn.com/sol3/papers.cfm?abstract_id=4273521