MINISTRY
OF HEALTH

CONCEPT NOTE

Resolution on Rare Diseases titled

"Rare Diseases: A Priority for Global Health Equity and Inclusion"

to be adopted under agenda item 6 **"Universal Health Coverage"** at the 78th World Health Assembly in 2025

The Resolution is initiated by the Arab Republic of Egypt and Spain, currently cosponsored by: (Qatar, Malaysia, France, Panama and Chile)

October 10, 2024

Background

In a world marked by medical and scientific advances, rare diseases (RDs) continue to present a significant challenge for healthcare and public health. 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 2,000 persons living in any World Health Organization-defined region of the world. Although taken one at a time they affect a limited number of people, their global burden and impact is significant, with over 300 million people affected worldwide. More than 80% of rare diseases are of genetic origin, 70% start in childhood and about 95% lack treatments. The average time for an accurate diagnosis is 4 to 8 years and about 30% of the affected children die before 5 years of age. The WHO estimates that there are more than 7,000 rare diseases defined -and this number is steadily increasing-, with the 400 most prevalent affecting an estimated 90% of the population living with a rare disease, a very long tail of extremely rare diseases.[1,2,3]

The general lack of public awareness and expertise constraints have neglected and marginalized the RD population in health systems and in health- and social-care policies.[4]

Adopted in 2015, the UN Sustainable Development Goal (SDG) 3.8 is to "Achieve UHC, including financial risk protection, access to quality essential healthcare services and access to safe, effective, quality and affordable essential medicines and vaccines for all". In 2018, the WHO Director-General, Dr. Tedros Adhanom Ghebreyesus made a statement highlighting the importance of RDs.[5]

[1] The Lancet Global Health. The landscape for rare diseases in 2024. 2024. DOI: https://doi.org/10.1016/S2214-109X(24)00056

[2] Nguengang Wakap, S., Lambert, D. M., Olry, A., Rodwell, C., Gueydan, C., Lanneau, V., ... & Rath, A. (2020). Estimating cumulative point prevalence of rare diseases: analysis of the Orphanet database. European Journal of Human Genetics, 28(2), 165-173. https://doi.org/10.1038/s41431-019-0508-0

[3] Haendel, M., Vasilevsky, N., Unni, D., Bologa, C., Harris, N., Rehm, H., ... & Oprea, T. I. (2020). How many rare diseases are there? Nature reviews drug discovery, 19(2), 77-78.

[4] Chung, Claudia Ching Yan et al. "Rare disease emerging as a global public health priority." Frontiers in public health vol. 10 1028545. 18 Oct. 2022, doi:10.3389/fpubh.2022.1028545

[5] Available on-line: https://www.who.int/news/item/27-02-2018-statement-for-rare-disease-day

In 2019, UN Member States reaffirmed this commitment when they adopted the United Nations Political Declaration on UHC, which includes rare diseases (Resolution A/RES/74/2, 2019).[6] This declaration was renewed in September 2023 with the Political Declaration of the High-Level Meeting on UHC (Resolution A/RES/78/4, 2023).[7] For UHC to be truly universal and to accelerate the progress towards achieving SDG 3.8, health stakeholders around the world need to put rare diseases on the global health agenda.

In December 2021, a major turning point in the global political landscape was achieved when the UN General Assembly unanimously adopted the UN Resolution Addressing the Challenges of Persons Living with a Rare Disease and their Families (Resolution A/RES/76/132, 2022).[8] This UN Resolution paves the way for greater integration of rare diseases into the agenda and priorities of the UN system.

Despite considerable progress, including the recognition of rare diseases within the United Nations Agenda for Sustainable Development 2030, which seeks to promote health equity by addressing the needs of the most vulnerable populations, persons living with rare diseases (PLWRD), people living with undiagnosed rare diseases and their families continue to face significant global challenges. These include delay in diagnosis, difficulties in accessing treatments and services, stigma, financial burdens and social exclusion. A major barrier to improving their inclusion and participation in society is the lack of knowledge and expertise in the field, as well as a general lack of awareness about rare diseases.

Therefore, a health-specific framework for coordinated action with all stakeholders and Member States is needed, in order to solidify commitment and translate these commitments into action to overcome the barriers to accessing diagnosis, treatment, and care for PLWRD.

Adopting a resolution on rare diseases at the 78th WHA will secure rare diseases as a priority on the global health agenda and emphasize to Member States the importance of addressing the social and financial burdens of treating PLWRD in a sustainable and inclusive way to ensure that no one with rare disease is left behind in the pursuit of universal health coverage (UHC).

Objectives of the Resolution

- To promote and enhance universal health coverage to improve public health and social measures needed to support PLWRD, their families and caregivers.
- To strengthen the efforts of Member States and all stakeholders in order to raise awareness, improve diagnostic capabilities and foster research and development of therapies, ensuring that PLWRD have access to affordable, effective, and comprehensive healthcare services, regardless of the rarity of their condition, without facing financial hardship.

[6] Available on-line: https://documents.un.org/doc/undoc/gen/n19/311/84/pdf/n1931184.pdf
[7] Available on-line: https://documents.un.org/doc/undoc/gen/n23/306/84/pdf/n2330684.pdf
[8] UN General Assembly (76th sess.: 2021-2022). Addressing the challenges of persons living with a rare disease and their families: resolution/ adopted by the General Assembly, A/RES/76/132.

Aim of the Resolution

In order to achieve the main objectives of the Resolution, the primary proposed aim is **to call upon the WHO to develop, in consultation with Member States, and in collaboration with relevant non-State actors, a comprehensive Global Action Plan on Rare Diseases**.

Goals and Components of the Global Action Plan on Rare Diseases

- The Global Action Plan on Rare Diseases will act as a catalyst for systemic change by providing Member States a practical framework for action, tailored to their level of progress. This plan will help in developing national policies and strategies to address the social and financial challenges of treating PLWRD in a sustainable and inclusive manner.
- It will ensure that policies and practices are grounded in the best available evidence, and that rare diseases are integrated into global health priorities like primary healthcare and universal health coverage (UHC). This integration will ensure that rare diseases receive the necessary attention, resources, and strategic planning.
- Additionally, a comprehensive Global Action Plan on rare diseases will provide a clear and detailed roadmap for all stakeholders, promoting interdisciplinary and intersectoral approaches. This plan will establish global targets and strategic goals, along with specific actions and steps to achieve them.

The Global Action Plan will include the following components:

- Global targets and strategic goals to ultimately enhance equitable access to care, social support and improve health outcomes and quality of life for PLWRD;
- Specific actions and measures required to meet these targets and goals at the global,regional,national level;
- Timelines outlining the implementation schedule for the recommended actions and measures;
- Guiding principles for developing and executing rare disease policies and programs;
- An accompanying process for accountability and monitoring to assess and ensure progress in implementation progress.

The guiding principles of this plan will include a common reference point for identifying rare diseases and a commitment to people-centered primary health care, access to expertise and universal health coverage. It will promote an integrated approach to care throughout an individual's lifespan and ensure that policies and practices are based on the best available evidence.

The following elements of the rare disease ecosystem will be addressed by the Global Action Plan:

- The structuring of health systems and care coordination to enhance efficiency and equity within health care systems;
- Cross-sectoral initiatives that empower and actively involve PLWRD and their caregivers;
- Enhanced networking and collaboration across countries, regions, universities, research institutes, centers of expertise and other entities;

- Ensuring timely and accurate diagnosis;
- People-centered universal health coverage (UHC);
- Equitable access to evidence-based treatments;
- Promotion of innovation, research, and technological advancements;
- Provision of comprehensive and high-quality patient care;
- Collaboration among institutions, policymakers, healthcare professionals, scientific societies, and patient organizations;
- Specialized training for medical students and healthcare professionals in managing rare diseases;
- Raising awareness of the individual and global impact of rare diseases; and
- Promote capacity-building support, technical support, and financial assistance of the Ministry of Health.

Key Considerations for Effective Implementation of the Rare Disease Resolution

Global Impact:

It is essential to recognize that rare diseases significantly affect patients, their families, and caregivers, influencing their quality of life and social integration. This impact can be particularly profound if the disease presents early in childhood, potentially affecting an individual's entire life.

National context:

- To consider the economic, social, cultural, and political disparities among countries and regions when devising and implementing measures and policies related to rare diseases.
- To analyze the difficulties in accessing and making available interoperable and quality health data.
- To raise awareness and work with local communities and relevant stakeholders to help the health system recognize the national cases.
- To promote the importance of a multidisciplinary care in patients with rare diseases, adapted to their needs.

Next Steps

Working method

To develop in consultation with cosponsors Member States the zero draft of the resolution text. The main operative paragraphs of the resolution will be to request the WHO Director General to develop, in consultation with Member States, and in collaboration with relevant non-State actors, a comprehensive Global Action Plan on Rare Diseases.

The draft Global Action Plan will be submitted for consideration by the Executive Board at its 160th session, together with a report on the progress achieved in implementing this resolution, with the intention of submitting the draft action plan to the Eightieth World Health Assembly for endorsement.

Expected timeline

- **November 2024:** submission of the zero draft of the resolution before or by November 2nd to the WHO GBS Department.
- **November 2024 – December 2025:** informal consultations with all WHO Member States on the Resolution, and finalization of the resolution for the Executive Board based on the outcome of the consultations.
- **February 2025:** consideration of the resolution by the Executive Board.
- **May 2025:** adoption of the Resolution at the World Health Assembly.

Conclusion

By adopting a WHA resolution on rare diseases, Member States have the opportunity to strengthen their positions as leaders in healthcare innovation and advocacy while reaffirming their commitment to leaving no one behind in the ultimate pursuit of health for all and to contribute their respective expertise and resources to improve the lives of PLWRD not only nationally, but globally.

The WHA resolution, and the resulting global action plan on rare diseases, will be the catalyst that urges Member States to work together towards enhancing policy processes, advancing research, accelerating innovation, and improving access to care for PLWRD worldwide. This will ultimately ensure that PLWRD are included in the critical work being done to advance UHC across the globe.