
SUBMISSION BY



RARE
DISEASES
INTERNATIONAL

TO WHO INDEPENDENT HIGH-LEVEL COMMISSION ON NON- COMMUNICABLE DISEASES

May 16, 2018

In response to the draft report dated 1 May 2018 based on the meetings of the WHO Independent High-level Commission on NCDs and a Technical Consultation, Rare Diseases International would like to ensure that in addressing the very high priority of non-communicable diseases, the WHO and Member States do not leave behind significant but often neglected chronic rare diseases, each of which affect relatively small numbers of patients but collectively affect at least 300 million people in all countries of the world causing tremendous suffering as well as economic and societal impact.

Rare diseases are mostly chronic, highly complex, progressive and severely disabling diseases that present multiple comorbidities and affect life expectancy. Characterized by their low prevalence and their heterogeneity, an estimated 6,000 to 7,000 distinct rare diseases exist today, affecting more than 300 million people, both children and adults, across the globe. The lack of scientific knowledge and quality information on these diseases often results in a delay in diagnosis, which also delays timely intervention and opportunities to reduce morbidity, mortality, and incidence. The lack of disease recognition and appropriate quality health care engenders inequalities and difficulties in access to treatment and care, often resulting in significant social and financial burden on patients. Due to the rarity and diversity of rare diseases, research needs to be international to ensure that experts, researchers and clinicians are connected, that clinical trials are multinational and that patients can benefit from the pooling of resources across borders.

Quoting from the Background Paper on Orphan Diseases for the WHO Report on Priority Medicines for Europe and the World (2004) “despite the growing public awareness of rare diseases in the last two decades, there are still many gaps in knowledge related to the development of treatment for rare diseases. Policymakers have to realise that rare diseases are a crucial health issue for about 30 million people in the EU”. And if we extrapolate this figure, we come up with a conservative estimation of 300 million people affected worldwide: **Rare Diseases are an international public health priority that cannot be denied.**

Through this contribution, we take the opportunity to request from the WHO Director-General and the Member States to:

ACKNOWLEDGE the severity of the problem and take immediate action by including rare diseases in the Agenda to be discussed during the upcoming third High-level Meeting of the General Assembly on the Prevention and Control of Non-Communicable Diseases;

PROMOTE national Universal Health Coverage strategies and plans that include the appropriate management of the multiple needs of patients with rare diseases;

ADVOCATE for the adoption of the WHO fair pricing approach by pharmaceuticals in order to guarantee access to safe, available and affordable orphan drugs for all patients with rare diseases;

DEVELOP synergies to increase:

- a) Member State negotiating power for procuring new medicines and health technologies and;
- b) the access of patients to timely, appropriate and effective diagnosis and treatment.

All above requests constitute our consensus position and can be accommodated within the scope and objectives of the Global Action Plan for the Prevention and Control of Non-Communicable Diseases.

Finally, we would like to express our deepest appreciation for the ongoing productive collaboration between our Organizations and the World Health Organization (WHO) and reiterate our firm determination and commitment to support all WHO efforts in attaining its mission that is the “attainment **by all people** of the highest possible level of health” leaving no one behind. As stated by Dr Tedros Adhanom Ghebreyesus, WHO Director-General on the occasion of Rare Disease Day 2018, “Just because a disease affects a small number of people does not make it irrelevant or less important than diseases that affect millions.”

Rare Diseases International is the global alliance of people living with a rare disease of all nationalities across all rare diseases. RDI’s mission is to be a strong common voice on behalf of rare disease patients around the world, to advocate for rare diseases as an international public health priority and to represent its members and enhance their capacities. **RDI brings together national and regional rare disease patient alliances from around the world as well as international rare disease-specific federations to create the global alliance of rare disease patients and families.** RDI has more than 50 member organisations from over 30 countries, that in turn represent rare disease patient groups in more than 100 countries worldwide.