



**WHO Expert Committee on the Selection and Use of Essential Medicines - Open Session
24 April 2023, Geneva**

**Rare Diseases International (RDI) Statement
Durhane Wong-Rieger, Chair of the RDI Council**

Rare Diseases International, RDI, is very pleased to have the opportunity to address the WHO Expert Committee. While rare diseases affect a small percentage of the population, there are over 6,000 known rare diseases, with an estimated 300 to 450 million persons worldwide living with a rare disease. Thanks to global support, including the United Nations General Assembly Resolution “Addressing the challenges of persons living with a rare disease and their families” and RDI-WHO collaboration to improve access to essential medicines and diagnostics, our communities have significantly greater impact in calling upon Member States to accelerate efforts on Universal Health Coverage and be more inclusive of persons living with a rare disease toward access for quality, affordable essential medicines and diagnostics.

Only 5% of rare diseases have approved therapies, some of which provide disease management and supportive care but many others truly slow, stop, or prevent serious symptoms and some new ones that transform lives by addressing the underlying cause of the disease. In 2021, the International Rare Disease Research Consortium (IRDiRC) Access Working Group published the first “essential list of medicinal products for rare diseases”: all are approved by one or more major regulator, but none are accessible everywhere and some are accessible in very few places to few eligible patients. This leads to why we are here today to consider how these can be included in the WHO Model List of Essential Medicines. I have three key points.

- A mapping exercise performed by the EML Secretariat revealed that approximately 15% of medicines of EML and EML for children could be relevant for rare diseases. But not all of these are clearly annotated so as to be clearly visible to users and policy makers. As a consequence, these medicines are not included in healthcare decision-making, not at the individual patient-provider level, not at health plan coverage/reimbursement levels, and not at policy and regulator levels. One feasible path to improve annotation within EML is to associate medicines to the corresponding diseases using the International Classification of Diseases 11th Revision.
- We are encouraged by the number of applications for medicines for rare diseases that are under review. The inclusion of medicines for rare diseases in the EML signals to countries and health authorities that drugs indicated for a small number of patients can nevertheless “satisfy the priority needs of a population.” Moreover, inclusion of these therapies on the EML will support patients and healthcare providers in their efforts to gain access not only to the medicines but also to essential diagnostic, specialist and comprehensive care, and complementary support services.
- RDI acknowledges the WHO selection of essential medicines for inclusion in the EML “with due regard to disease prevalence and public health relevance, evidence of efficacy and safety and comparative cost-effectiveness.” Moreover, the EML is intended as a guide for health authorities to “improved access through streamlined procurement and distribution of quality-assured



medicines, support more rational or appropriate prescribing and use and lower costs for both health care systems and for patients.” To these ends, we urge the Expert Committee to appropriately apply the criteria and selection process to the realities of rare diseases and rare disease medicines, where small patient numbers can mean higher per-patient costs but low budget impact, slower development of replacement generics or biosimilars, and despite some orphan drug laws, relatively few incentives to develop medicines with the result that only 5% of rare diseases have approved therapies and most ultra-rare diseases have none.

In conclusion, the inclusion of rare disease essential medicines in the WHO Model List of Essential Medicines is of critical importance to supporting appropriate patient access in high, middle, and low income countries. We in the rare disease community are committed to working with national and local authorities to helping develop the infrastructure for effective diagnosing, specialist and supportive care and long-term monitoring to assure medicines are used effectively to deliver the best outcomes to patients and society. Rare Diseases International appreciates the opportunities to collaborate with the WHO on all these steps.