Leverage Data to Improve Access to Essential Medicines for Rare Diseases

Chiu-Hui Mary Wang, Chiara Tovani, Sara Brambilla, on behalf of RDI Essential Medicines Working Group

Rare Diseases International ✉ mary.wang@rarediseasesint.org

Access to Essential Medicines

Access to essential medicines is a critical component of global health policy, guided by the **WHO's Essential Medicines List (EML)**. The WHO EML is a register of **minimum medicines considered to be the most effective, safe and cost-effective**, to meet the needs for every healthcare system[1].

What is the WHO EML:

It is divided in two lists, EML **for adults** which contains **502** (23rd version), and one for **Children** (EMLc) composed by **361** medicines (9th version).

The EML is, also divided in two sections:

- **Core list:** for basic health-care needs.
- **Complementary list:** specialist medical care needs.

The EML is an **open-access resource** that supports **national public health planning**. Applications for inclusion or modification can be submitted by any stakeholder.

[1] Purgato, & Barbui, (2012). What is the WHO essential medicines list?

Objectives

- **Promote** the **understanding** of the Essential Medicines Model Lists.
- **Analyze** relevant **successful and failed** rare disease medicine **applications** to the EML.
- Identify **pitfalls and lessons**.
- **Advocate** for appropriate assessment of essential rare disease medicines.

Rare Diseases International Essential Medicines Working Group

The Working Group was established by **patient representatives** from **RDI member organizations** and their members.

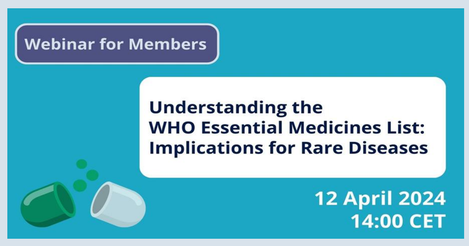
Ana de Carmo Campos	RD Portugal, Istituto Nacional De Saude Dr. Ricardo Jorge, Unidad Local de Saúde Santa Maria
Andrea Verónica Fraschina	Federacion Argentina de Enfermedades Poco Frecuentes (FADEPOF)
Arpana A Iyengar	Organization for Rare Diseases India
Christina Raj	Center for Ichthyosis Related Members (CIRM) Foundation, Global Skin
Emmylou Casanova	Psoriasis Philippines, Global Skin
Manzi Ndamukunze	Centre-Alliance for Rare Disease in Rwanda
Robelle Mae Tanangunan	Rheumatology Educational Trust Foundation Inc, GlobalSkin
Roberta Anido	Federacion Argentina de Enfermedades Poco Frecuentes (FADEPOF)
Salome Mekhuzla	World Federation of Hemophilia
Sudheendra Rao	Organization for Rare Diseases India
Trudy Nyakambangwe	Child and Youth Care Zimbabwe
Virginie Hivert	EURORDIS - Rare Diseases Europe

Methods

- Compiled a **list of stakeholders** who had made applications or used the EML for rare diseases, using the electronic EML and community contacts as information sources.
- **Identified over 40 potential stakeholders for interviews**, including: patient organizations, academia, non-profit organizations and pharmaceutical manufacturers.
- Developed an **interview guide to capture the experiences** of stakeholders.
- **Conducted** 12 semi-structured **interviews with different stakeholders** to **gather data** from different sources.
- Identification of **common challenges** among stakeholders

Capacity Building Activities

- **Webinar** for RDI Members on WHO EML with **speakers** from **WHO EML Secretariat** to present:
  - purpose and composition of the Model list and the application process
  - presence of rare diseases medicines in the list



- **Factsheet** to facilitate the understanding of the EML and its relevance for rare diseases.

English: Spanish:

Community Experience Driven Insights

Application Process

Good practices to consider for preparing an application:

- Having a **structured methodology**
- Identification of the **right medicine**, considering feasibility across different global contexts, medical need and accessibility
- Gather support from the community via inclusion of **support letters**
- Data from **multiple regions** of the world, including Low- and Middle- Income Countries
- Comparative effectiveness vs the current Standard of Care.

Challenges Specific for Rare Diseases

- **Quality of the evidence**: RD often lack large-scale and randomized controlled clinical trials; long-term data may be unavailable or inexistent due to high mortality.
- **Balancing value and uncertainties**: it is crucial to distinguish between evidence assessment and value assessment, because even with weak evidence, the value of a medicine can be substantial.
- **High cost** of medicines for RD presents a major challenge.

Impact and Value of EML

- It is a useful tool for **advocacy** to increase access and improve the assessment of essential rare disease medicines.
- In LMICs the **inclusion** of a medicine in the WHO EML is crucial at a **national level** for advocates, doctors and patients.
- Once in the list the treatment is recognized as a **Standard of Care** (SoC).
- **Dynamic and evolving resource**, allowing for the inclusion of new treatments as well as older that, while more affordable, offer equal effectiveness.

Data for an Application

- International nonproprietary names
- Treatment details
- Disease epidemiological information
- Disease burden
- Target populations
- Alternative medicines already in the EML
- Clinical evidence of the medicine
- Safety data
- Cost
- Cost-effectiveness studies
- Regulatory status and market availability
- References

Conclusions & Next Steps

To enhance access to healthcare for rare diseases, the EMWG aims to gather lessons learned, address gaps in the WHO EML, and develop recommendations.

Understanding the EML's implications will help stakeholders build capacity and **improve outcomes** for affected individuals.

About RDI

RDI is the global alliance of people living with a rare disease of all nationalities across all rare diseases. Our mission is to be a strong common voice on behalf of RD patients around the world.

www.rarediseasesinternational.org

@Rare Diseases International

RARE DISEASES INTERNATIONAL