



WHO Essential Medicines and in Vitro Diagnostics Lists: Why, What & How

.....

Yann Le Cam, Chief Executive Officer of EURORDIS-
Rare Diseases Europe and member of RDI Council



RARE
DISEASES
INTERNATIONAL

WHAT ARE WHO PRIORITY LISTS?

- ▶ WHO Priority Lists are up-to-date lists of the most important health technologies a country needs
 - Model List of Essential Medicines (EML)
 - Model List of Essential Medicines for Children (EMLc)
 - Model Essential in Vitro Diagnostics List (EDL)
- ▶ When WHO published the first Model List of Essential Drugs in 1977, it identified 208 individual medicines which together could provide safe, effective treatment for the majority of communicable and non-communicable diseases.
- ▶ In 2019 EML has a total 460 products that include some of the newest and most advanced cancer drugs
- ▶ More than 150 countries currently use the WHO list to work out which medicines best meet their national health contexts and priorities, so they can compile their own national essential medicines lists.
- ▶ Essential medicines are those that satisfy the priority health care needs of a population. They are the medicines that need to be available in a functioning health system at all times, in appropriate dosage forms, of assured quality, and at prices individuals and the community can afford.
- ▶ Lists of essential medicines also guide the procurement and supply of medicines in the public sector, schemes that reimburse medicine costs, medicine donations, and local medicine production.



WHY ARE THEY IMPORTANT?

- ▶ WHO Priority Lists are recognised as essential for advancing universal health coverage, addressing health emergencies and promoting healthier populations -- which are the 3 strategic priorities of 13th WHO General Programme of Work (2019–2023)
- ▶ Expected to provide guidance and serve as a reference for member states that are developing and/or updating national Model Lists for interventions within universal health coverage
- ▶ Inform United Nations agencies and nongovernmental organisations that support selection, procurement, supply, donation or provision of essential medicines and diagnostics
- ▶ Many international organisations, including UNICEF, UNHCR and UNFPA as well as nongovernmental organisations and international non-profit supply agencies, have adopted the essential medicines concept and base their medicine supply system mainly on the Model List
- ▶ The EDL also informs the private medical technology sector about the diagnostic priorities and the IVDs necessary to address global health issues.



CAN HEALTH TECHNOLOGIES FOR RARE DISEASES BE INCLUDED?

- ▶ The concept of essential medicines is forward-looking. It incorporates the need to regularly update medicines selections to **reflect new therapeutic options** and **changing therapeutic needs**; the need to **ensure drug quality**; and the need for **continued development of better medicines**, medicines for **emerging diseases**, and medicines to meet changing resistance patterns.
- ▶ Definition in 1977: Essential drugs as those needed to satisfy the health-care needs of the majority of the population
- ▶ Definition in 2020: Essential medicines are those that satisfy the priority health care needs of a population.
- ▶ BEFORE seen as relevant only in resource-constrained settings, population size
- ▶ NOW seen as equally relevant to high-, middle- and low-income countries, **particularly with the inclusion of new, highly effective and expensive medicines in more recent years.**
- ▶ **Potential curative transformative treatments for rare diseases are changing the perspective and could be considered in the EML, even soon after their approval.**
- ▶ In the case of diagnostics, the Essential In Vitro Diagnostics List (which started in 2018) is open to diagnostic tools for rare diseases



*“If the definition of an essential medicine is to be changed to include medicines needed for people with rare diseases, then the principle of **distributive justice** can be the moral basis for such a change and **cost-effectiveness** analysis can be the method used to select which medicines to include in the Model List.”*

Marcus M Reidenberg

[Rare essentials: drugs for rare diseases as essential medicines.](#)

[Bull World Health Organ. 2006]



*“We must work together to **ensure an equitable and affordable access to quality health services and medicines for everyone, especially people who are most in need, which also includes people living with rare diseases, among others, while ensuring that they do not face financial hardship or fall back into poverty because of their catastrophic medical expenses.**”*

H.E. Vitavas Srivihok,

Ambassador, Permanent Mission of Thailand to the UN
and Rapporteur of the UN Political Declaration on UHC



RARE
DISEASES
INTERNATIONAL

WHO MODEL LIST OF ESSENTIAL MEDICINES (EML)

World Health Organization
Model List of Essential Medicines

21st List
2019

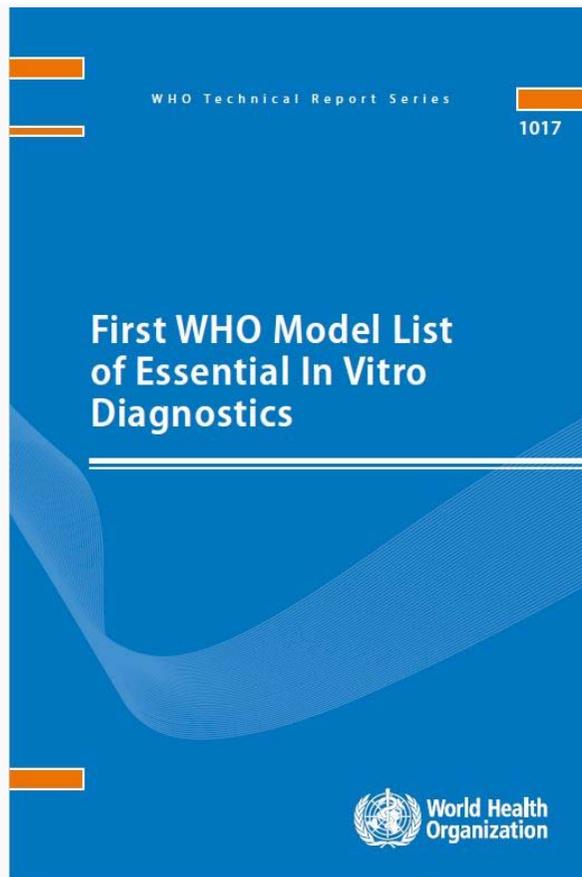


- Model Lists of Essential Medicines has been updated every two years since 1977
- Updated by a group of experts : WHO Expert Committee on the Selection and Use of Essential Medicines
- Includes information about available dose forms and strengths; specifications as to who should take a medicine; what conditions a medicine can treat and how it should be taken, as well as details about the clinical evidence supporting recommendations being made.
- The current versions have been updated in June 2019
 - 21st WHO Essential Medicines List (EML)
 - 7th WHO Essential Medicines List for Children (EMLc)
- Meeting of 23rd WHO Expert Committee will be held at WHO Headquarters, Geneva on 22 – 26 March 2021 (TBC)
- The application period is now open until 30 November 2020



RARE
DISEASES
INTERNATIONAL

WHO MODEL LIST OF ESSENTIAL IN VITRO DIAGNOSTICS (EDL)



- First WHO Model List of Essential In Vitro Diagnostics (EDL) published in May 2018
- Limited scope covering high priority infectious diseases concentrating on a limited number of priority diseases – HIV, malaria, tuberculosis, and hepatitis
- + 60 types of in vitro diagnostic tests for use at different levels of the healthcare system, including disease-specific tests as well as general laboratory tests
- The second edition of the EDL was launched in July 2019
- Includes general tests for non-communicable diseases ie. sickle cell
- + 40 new tests, a new anatomical pathology section as well as several edits to test categories in the first edition.
- The third edition of the EDL was to be reviewed in March 2020 (postponed to later date TBC)
- 4th Cycle of submissions: July/ August 2020 (TBC)

WHO MODEL LIST OF ESSENTIAL IN VITRO DIAGNOSTICS (EDL)

- ▶ Like the EML, the EDL lists IVDs that are recommended by WHO for use in countries. .
- ▶ The EDL is expected to provide guidance and serve as a reference for Member States (programme managers, laboratory managers, procurement officers and reimbursement officers) that are developing and/or updating national EDLs for interventions within universal health coverage and for selecting and using IVDs.
- ▶ Meant to be informative for United Nations agencies and nongovernmental organizations that support selection, procurement, supply, donation or provision of IVDs and will inform the private medical technology sector about the diagnostic priorities and the IVDs necessary to address global health issues.
- ▶ Not intended to be prescriptive with respect to the IVDs listed or the levels at which they can or should be used; rather, countries should decide which IVDs to select and where to use them, depending on their epidemiology, human resources and infrastructure
- ▶ Updated/expanded once a year, based on yearly calls for submission of applications
- ▶ The WHO Strategic Advisory Group of Experts on In Vitro Diagnostics (SAGE IVD) oversees the process



RDI ACTION PLAN 2020-2021

Objective: Inclusion of more in vitro diagnostics and therapies for RD, considered essential by relevant disease communities, into the WHO Essential List

Method: in liaison with WHO EDL, EMP, Qualification units and building on RDI members:

- Promote the opportunity to all potential applicants (ie. patient groups, clinical leads, hospitals and company market holders).
- Present and explain EML and EDL programme, application and processes to RDI members at large

Activities 2020:

- 2 webinars with WHO EDL and EML Secretariat
- 2 webinars with case studies / patient groups with experience
- Progressively involve relevant Expert Faculty members into analysis of current essential lists, guidance to RDI members, peer to peer learning, liaison with WHO
- Attendance by RDI members and Expert Faculty of EDL and EML Expert Committees open to stakeholders

Coordination with IRDIRC Rare Disease Treatment Access Working Group



EXAMPLES

- ▶ Thalassaemia: Red cells and deferoxamine (DFO) in EML
- ▶ Sickle Cell Disease: Hydroxycarbamide (hydroxyurea) in EML
- ▶ Haemophilia: factor VIII and factor IX in EML
- ▶ Primary immunodeficiency (PID) products in EML
- ▶ In 2018 attempt by Osteogenesis Imperfecta community to include bisphosphonates for injection on the Essential Medicines List for Children (WHO EMLc)
- ▶ In 2019 inclusion of essential primary immunodeficiency (PID) diagnostic tests in [2nd WHO Model List of Essential In Vitro Diagnostics](#)

