



**ORGANISMO ANDINO DE SALUD
CONVENIO HIPÓLITO UNANUE**

▶ **WEBINAR**
Especial

**Enfermedades Raras
y Huérfanas,
hacia su inclusión
como Prioridad
en Salud**



50
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Juntos somos más fuertes





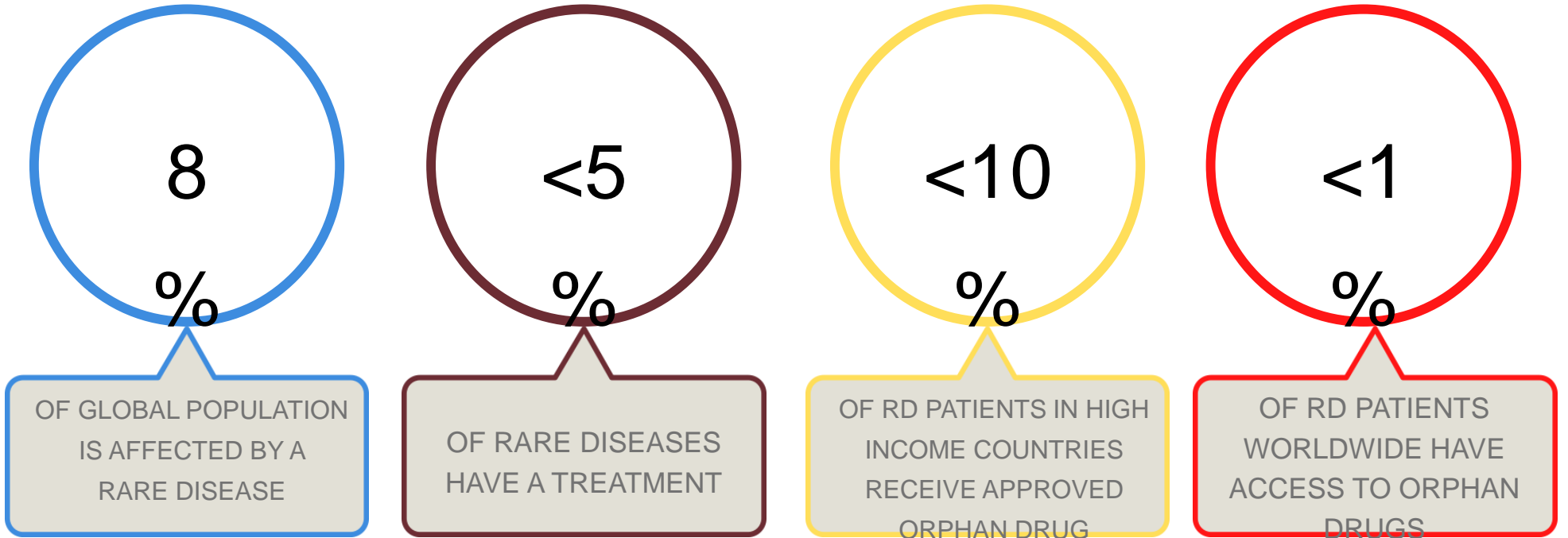
GLOBAL ACCESS: ESSENTIAL RARE DISEASE MEDICINES, BARRIERS & STRATEGIES

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WHY ACCESS STRATEGY IS ESSENTIAL

ONLY SMALL FRACTION OF PATIENTS IN ANY COUNTRY HAVE ACCESS TO APPROVED ORPHAN DRUGS



Approaches to Developing Essential RD Medicines List

1st Approach: Orphan Designated Drugs and Approved RD Drugs

- Database USA FDA Orphan Drug Product Designation
- EMA Data approved Orphan Medicinal Products and RD Medicines
- China's Rare Diseases Catalog and Novel Drugs Approved in Other Jurisdictions

2nd Approach: World Health Organization

- Model List of Essential Medicines – 21st List
- Model List of Essential Medicines for Children – 7th List
- Indicated for rare diseases



RDТА WG ACTION PLAN -2

- RDТА WG **List of Medicines** considered **essential for Rare Diseases** based on approvals by key regulatory agencies (US, EU, China) plus WHO Essential Lists
 - **Databases** of medicines designated as OMPs or approved for Rare Disease indications;
 - **WHO Model Lists** of Essential Medicines and of Essential Medicines for Children
- Collated List of **204 Essential Medicinal Products**: efficacious, safe and have significant impact on quality or duration of life.
- Organised into **seven disease categories** as initial iteration of a “**living document**” to be updated periodically.



List of Essential Rare Disease Medicines

List: 204 Essential Medicines for Rare Diseases

- Does not include medicines for rare cancers

Seven Disease Categories

- Metabolic, neurologic, hematologic, anti-inflammatory, endocrine, pulmonary, immunologic, miscellaneous

Types of Drugs

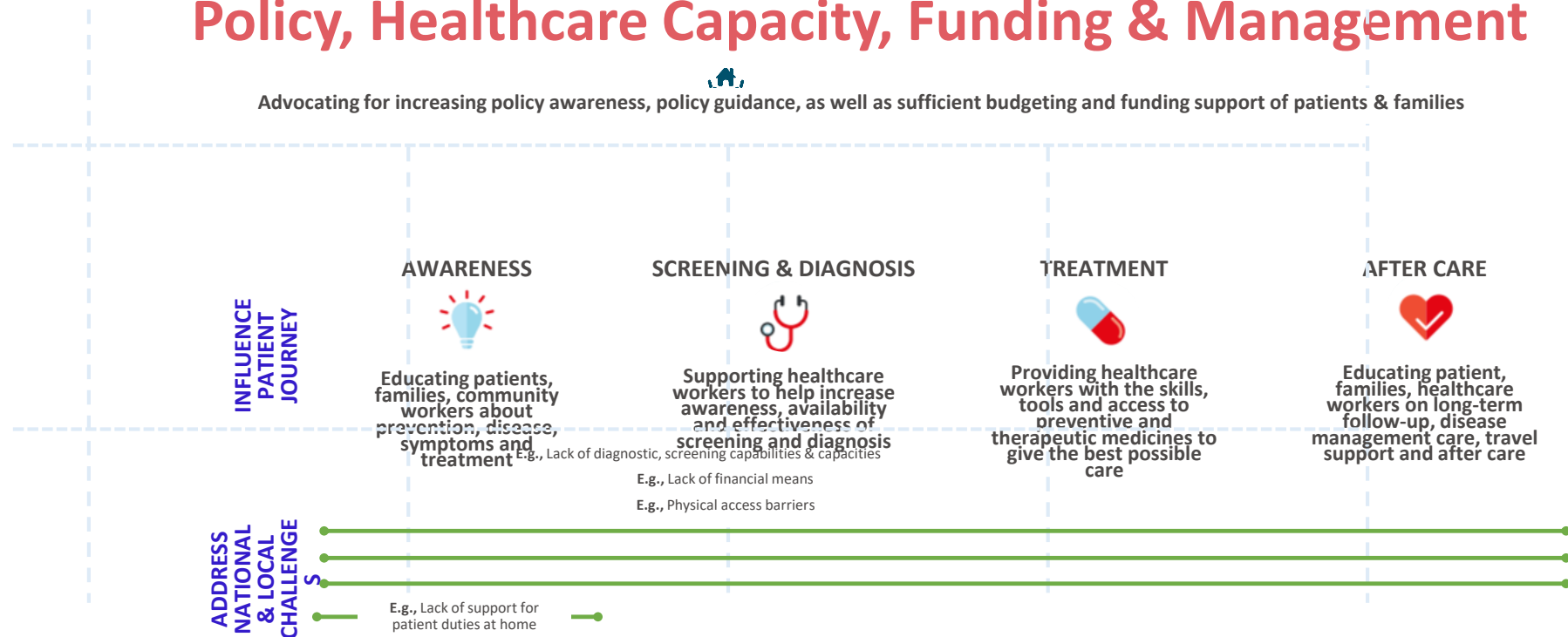
- 125 chemical drugs
- 79 biologics (polyclonal and monoclonal antibodies, cell and nucleic acid therapies)

Total of 134 diseases



Transform Health System to Transform Treatment Access

Policy, Healthcare Capacity, Funding & Management



WHAT ARE SYSTEM-WIDE GAPS?
WHAT ARE DISEASE-SPECIFIC BARRIERS?



Challenges to Access to RD Medicines

- Lack diagnosis and access to expert care
- Only 25 on List in WHO Model List of Essential Medicines
- Many without regulatory approval across jurisdictions
- Many approved but not authorized or reimbursed for rare condition
- Some applicable to only fraction of patients within disease category
- LMICs: no access to essential medicines for All diseases
 - Lack of (universal) healthcare
 - Feasibility of manufacturing and delivery chains
- High prices and lack of transparency in pricing
- HTA or values-based assessment not suitable rare disease therapies
- Indicated for rare diseases



Barriers to Medicines Access in Low- and-Middle Income Countries

INTERNATIONAL LEVEL

- Lack of commitment (by developed countries) to building optimized and efficient health systems (public health for all)
- Reduced international focus on Non-communicable diseases

HEALTH SYSTEMS LEVEL

- Availability of (common) medicines (2/3 in (higher priced) developed countries; only 1/3 in (public sector priced) LMIC)
- Affordability: lower national income, less healthcare coverage, higher out-of-pocket expenses

PHARMACEUTICAL LEVEL

- Limiting Intellectual property (higher prices)
- Competition and generics

INDIVIDUAL LEVEL

- Lack of literacy, education and awareness of drug and access options
- Lack of or false diagnosis
- Lack of (accessible) healthcare (poor use of public health services)
- Out-of-pocket expenses
- Poor adherence



Increasing Access to Essential Medicines in Developing Countries



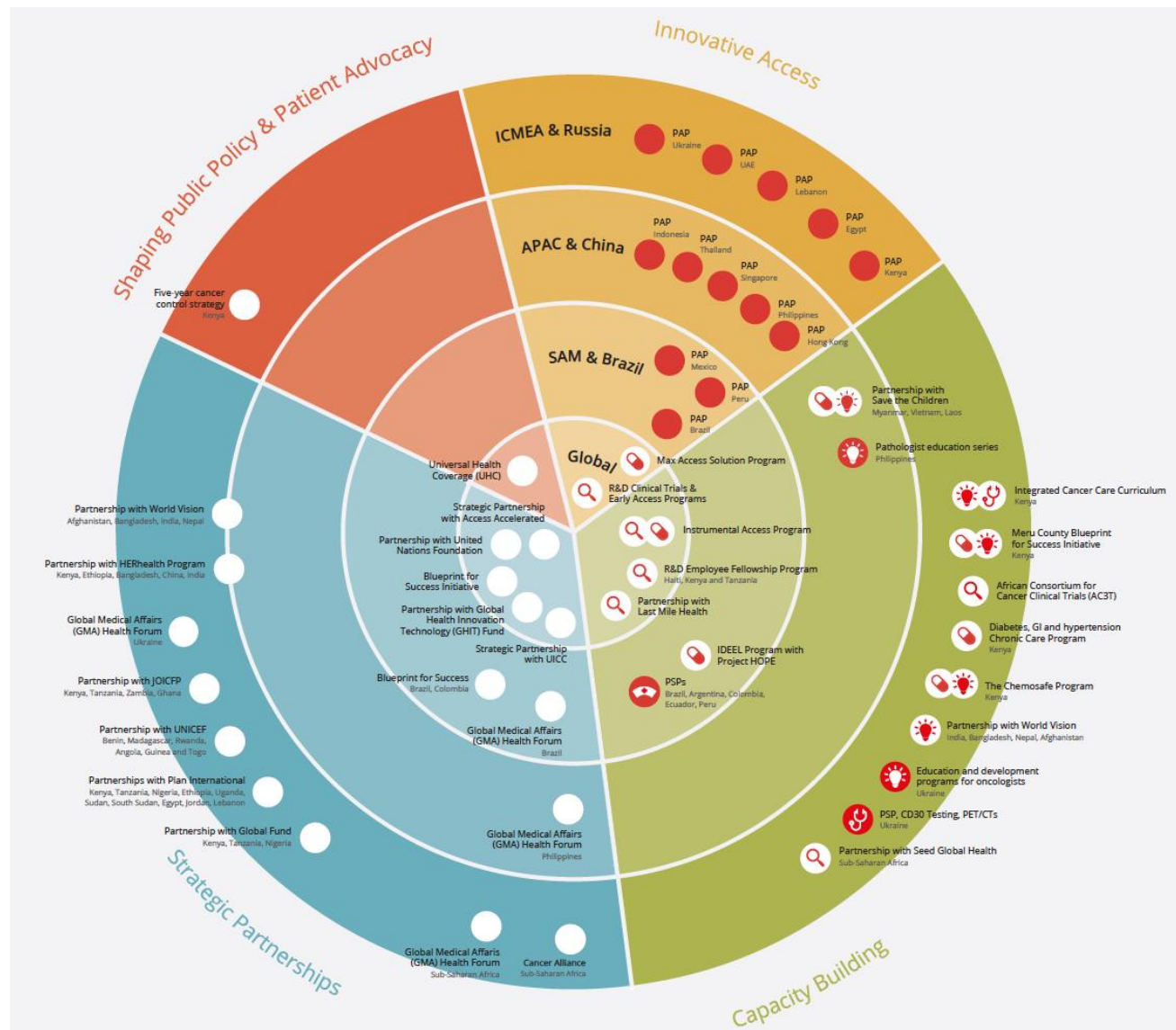
- **Regulatory Harmonization**
- **Health System Capacity**
 - Diagnosis, referral and registries
 - Expert centres and clinical development
- **Affordability**
 - Differential pricing, extending disease scope but ensure conditions do not cause undue burden on country's economics
 - Avoid leakage (different packaging)
- **Research, Monitoring and Reporting**
 - Private-public partnerships
 - Patient group development and sustainability



Initiatives for Access

- **WHO Essential medicines**
- **Vaccines (GAVI, Gates Foundation)**
- **Patient Advocacy Group Programs**
 - World Federation of Hemophilia “Redistribution Program
 - International Gaucher Alliance Humanitarian Aid
- **Donated medicines programs**
 - Americares
 - The MAX Foundation
- **Global Health Initiatives**
 - Partnership for Quality Medical Donations
 - MAP International
- **Multi-stakeholder development partnership**
 - UNITAID MAP (Medical Assistance Program)
 - AID for AIDS

Enfermedades Raras y Huérfanas, hacia su inclusión como Prioridad en Salud



Framework for Access to Medicines*



Transition WG to RDI - Next Steps

Use Collated List collated to **stimulate interactions** among patient organisations, healthcare providers, industry and government agencies to **improve standards of care and promote access to treatments.**

Integrate IRDiRC **RDTA Working Group within RDI** and align activities with priorities of RDI and Members;

Capacity-building to enable Members to use WHO Essential Medicines List & In-Vitro Diagnostics List; apply for inclusion to the Essential Lists.

Launch and expand Expert Faculty to grow knowledge base and support capacity-building activities on access.



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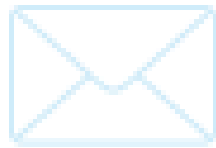
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