P4H SOCIAL HEALTH PROTECTION NETWORK

World Health Organization

RARE DISEASES INTERNATIONAL

# Webinar on Health Financing & Social Health Protection for Rare Diseases

Wednesday 5 November 2025

# WEBINAR OBJECTIVES

- Introduce the study on health financing and social health protection for **rare diseases across 11 countries** and its objectives.
- Share the overall preliminary findings as well as **three country case studies (China, France, Kazakhstan)** to illustrate different models and challenges.
- Facilitate discussion on the significance of the results among **experts in health financing and social health protection**.
- Gather feedback and questions from participants to **refine recommendations before publishing a report** in the coming months.
- Inform the implementation of the recent **World Health Assembly Resolution on Rare Diseases** and the forthcoming **Global Action Plan**.

# KEY TAKEAWAYS

**Integrating rare diseases into health financing and social health protection is critical to prioritize equity and the build sustainable, inclusive systems.**

**Equity by Design**: This means ensuring that rare diseases are not an afterthought but a structural component of universal health coverage.

**Innovative & Alternative Health Financing Solutions:** National funds, national risk pooling, and national or pooled (multi-country) procurement are promising models to further explore.

**Stronger Global Coordination & Accountability:** Stronger collaboration and data-driven strategies are essential to ensure sustainable progress. We hope the upcoming Global Action Plan on Rare Diseases will deliver on this.

# DISCUSSION SUMMARY

- Preliminary findings from 11 countries showed **varied definitions, financing models, and coverage for rare diseases**. Key challenges include **high medicine costs, limited financial protection, and lack of available data on rare disease expenditures**.

- The three country case studies highlighted **unique and common challenges and successes in health financing & social health protection for rare diseases.**

- The panel discussion focused **first on equity & priority setting**, with some panelists stressing the need for explicit frameworks and registries, especially in low-income countries, as well as regional collaboration and starting with the most tractable diseases.

- The panel then discussed **innovation & sustainability**, as panelists highlighted differential pricing, pooled procurement, and local production as promising strategies. Integrating health and non-health needs and avoiding disease-specific funds in favor of broader risk pooling was also suggested.

- **All panelists agreed on the importance of standardized data, registries, and global and regional collaboration**, with caution about donor dependence and the need for sustainable, locally-driven solutions.

# OPENING REMARKS

**Claude Meyer**
Senior Adviser, P4H Network
Department of Performance,
Financing and Delivery (PFD)
World Health Organization (WHO)

> People living with rare diseases are often among the most vulnerable, and **it is our mission within the P4H network - comprising 24 institutions - to find sustainable solutions that improve their financial protection**. This webinar is especially timely, as we face a global health financing emergency marked by widespread budget cuts in many countries.

> By addressing the needs of persons living with rare diseases, we **make equity measurable, strengthen systems for everyone, and bring patients and communities to the center of governance**. The project highlights both the challenges and the innovative pathways being tested to bridge coverage gaps and make Universal Health Coverage truly universal.

**Alexandra Heumber Perry**
CEO
Rare Diseases International

# OVERALL FINDINGS OF THE ANALYSIS OF 11 COUNTRIES

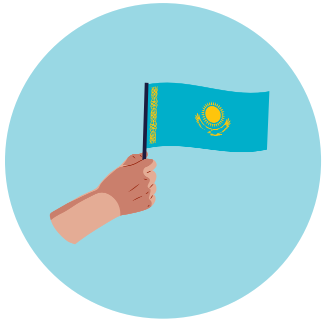
## The study reviews health financing arrangements and social health protection schemes for rare diseases across 11 countries, focusing on treatment and medicines.

**Aungsumalee Pholpark**
P4H Network

- *Key Findings*:
  - Social health protection schemes exist in most countries, but coverage ranges from comprehensive (e.g., Australia, France) to minimal or absent (e.g., Costa Rica, Tanzania).
  - Financing sources are mixed-government revenue and insurance dominate, but out-of-pocket payments remain high in several low-income countries.
- *Mechanisms & Coverage*:
  - Pooling for rare diseases is rare.
  - Purchasing models include capitation, fee-for-service, DRGs, and central procurement for high-cost medicines in select countries.
  - Benefit packages vary significantly, with some countries covering all rare diseases and others only specific conditions or offering one-time grants.
- *Challenges*:
  - High cost of medicines, sustainability of funding, and lack of inclusion in national drug lists.
  - Data gaps on financial burden and catastrophic health expenditures for rare diseases.

# CASE STUDIES: CHINA, FRANCE AND KAZAKHSTAN

**China** implements a **multi-layered financing system** combining national health insurance, local pilots, commercial health insurance, and philanthropy, with significant price reductions for some drugs but persistent gaps for high-cost treatments.



In **Kazakhstan**, the system encompasses **a Social Health Insurance Fund, a nationl charitable fund, and local government budgets** for uncovered treatments. There have been recent efforts to coordinate care and data, but fiscal constraints and financial sustainability remain challenges.

A **comprehensive national plan** is in place in **France**, with strong institutional networks, and comprehensive insurance coverage, but there are ongoing challenges in diagnosis, data use, and research.

# CASE STUDIES: CHINA, FRANCE AND KAZAKHSTAN

**Finn Guo**
Illness Challenge Foundation
*China*

> In **China**, the primary challenge for patients with rare diseases remains **financial risk protection**. Through coordinated financing, the patient out-of-pocket expense has reduced from $150,000 to  $2,600 USD, less than 2% of the total cost. The very important aspect here is a paradigm shift: from cost-effectiveness to social equity and public good governance, and not just the focus on the price of the medication. In the future, **we need to enhance financing coordination from different parties** like the public insurance, commercial plans, and philanthropy.

> **France** is a pioneer in the field of rare diseases at the European level as **the first country in Europe to have developed and implemented a National Plan for Rare Diseases**. Over the past 20-25 years, this effort has created a structured framework with 23 healthcare networks, 122 coordinating rare disease centers composed of 603 centers of reference, and more than 1,700 competence centers at the local level, **ensuring that each patient can be matched with an expert in their specific rare disease**.

**Salma Khalfi**
National Fund for Health Insurance (CNAM)
*France*

# CASE STUDIES: CHINA, FRANCE AND KAZAKHSTAN

**Ainur Aiypkhanova**
Scientific-Technical Council of the Ministry of Health
*Kazakhstan*

> “In Kazakhstan, one of the biggest challenges in healthcare today is the **limited fiscal space**. The Ministry of Finance has struggled to allocate promised funds to the Ministry of Health and other agencies, leading to delays in transfers and a lack of revenue for healthcare providers. This affects the sustainability of services and the ability to plan future purchases. To address this, we need continuous monitoring, stronger governance, and mechanisms like national funds or charity foundations to ensure access to essential medicines and treatments for people living with rare diseases.

# PANEL DISCUSSION: BRIDGING SYSTEMIC GAPS IN RARE DISEASE CARE

**Joseph B. Babigumira**
GHE Consulting

> You can't retrofit equity - it has to be a design principle. In countries with no rare disease financing, like Tanzania and Côte d'Ivoire, trying to build specialised coverage on broken foundations won't work. We need explicit priority-setting frameworks, structural reform, and progressive financing through general taxation to move forward.

> Medical innovation is a global public good in the technical sense. The knowledge can be shared around the world without using it up. It doesn’t mean everybody gets it, but in principle, it could. To realize that, we need systems that are ready - analytics, infrastructure, and equitable access across countries.

**Lou Garrison**
The Comparative Health Outcomes, Policy, and Economics (CHOICE) Institute
Department of Pharmacy, University of Washington, Office of Health Economics

# PANEL DISCUSSION: BRIDGING SYSTEMIC GAPS IN RARE DISEASE CARE

**Susan Horton**
University of Waterloo

> "Small countries need to collaborate and pool their resources to build centres of excellence that can serve a region. Otherwise, they will be left behind. You have to walk before you run - start slow, choose the most tacklable diseases, and build from there.

> "Equity means spreading the risk across the largest number of people. Instead of setting up separate funds to pay for the health expenditures of rare diseases, we should integrate financing into national systems to ensure sustainability. A global fund accessible to low-income countries could be a step toward equitable solutions.

**Claude Meyer**
World Health Organization

# PANEL DISCUSSION: UNLOCKING INNOVATION FOR SUSTAINABLE ACCESS

> This whole idea of health risk protection means that we are willing to pay more in rare catastrophic situations than we would in other situations. But when you think about production of medicines over time, you would expect that there would be diminishing returns and we expect that the costs of these innovation is rising in time. But they can benefit 8 billion people on earth so we need to find a way for everyone to contribute according to their ability.

**Lou Garrison**
The Comparative Health Outcomes, Policy, and Economics (CHOICE) Institute, Department of Pharmacy, University of Washington, Office of Health Economics

**Susan Horton**
University of Waterloo

> I think GAVI the Vaccines initiative tells us that we are willing to have the lowest prices for the poorest countries, and that as they graduate and become more able to afford it, then they can pay higher prices. So I think the tiered pricing pooled procurement is very important.

# PANEL DISCUSSION: UNLOCKING INNOVATION FOR SUSTAINABLE ACCESS

> “Health systems experts tend to forget that the needs of a person living with a rare disease are complex and diverse. You have of course the health expenditure needs. But you also have needs in terms of transportation, social care, housing, and of course to factor all those different needs into the situation of one specific person and to organize a social protection system around them. This is also something that is very often forgotten.

**Claude Meyer**
World Health Organization

> “Pooled procurement is important with regard to the monopsonist position. Governments can pool together like ECOWAS [Economic Community of West African States] because for a drug like hydroxyurea for sickle cell, the amount of money needed to treat hundreds of thousands of Africans is the same amount of money needed to treat a few hundred or so with a really innovative new drug. And if you have a monopsonist player like ECOWAS, then that can move a long way.

**Joseph B. Babigumira**
GHE Consulting

# PANEL DISCUSSION: RECOMMENDATIONS

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“We need **data about financial hardship** that people living with rare diseases are facing. We also need to explore the WHA Resolution on the Economics of Health for All that also tackles dimensions of public policy that are not in the health sector, but that answer to health related issues.

“Without data, there is no accountability. I would mandate **national rare disease registries** with standardized data and I think that’s what the GAPRD will need to do.

“We've had orphan diseases for a long time and I don't have a good sense of what the access lag is, what the cost is and so I'd want to run the simulation. **We do need more information**.

“Firstly, pay what you can - tiered pricing is crucial. Second, **leave no country behind**, not just poor countries, but small countries. Regional collaboration is essential.

# CONCLUSION

**Alexandra Heumber Perry**
CEO Rare Diseases International

> Rare disease is about a **holistic approach**. It's not only about looking at access to treatment but it's also looking at data, knowledge, access to diagnosis, newborn screening, etc. Today's discussion is a step toward a global consensus on financing rare disease care in a sustainable and fair way. We are looking forward to continuing this dialogue and building a world where people living with rare diseases are better included in every national health and social protection system - even on small islands.

## What's Next?

A joint report by P4H and RDI will follow, contributing to the forthcoming Global Action Plan on Rare Diseases.