Presenting author email address: mrico@the-ahf.org

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# Multi-stakeholder approach to rare disease care in Latin **America: focus on Pompe** and Fabry diseases

Mariana Rico-Restrepo,<sup>1</sup> Carlos Martinez,<sup>2</sup> Richard Salvatierra,<sup>3</sup> Pedro Borga<sup>4</sup>

<sup>1</sup>Americas Health Foundation, Bogota, Colombia; <sup>2</sup>Americas Health Foundation, Tijuana, México; <sup>3</sup>Americas Health Foundation, Washington, DC, USA; <sup>4</sup>Amicus Therapeutics Ltd, Marlow, UK

# Introduction and objectives

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- A varied landscape exists in terms of legislative and regulatory frameworks for rare diseases (RDs) across Latin America. Despite global efforts to improve RD care,<sup>1</sup> patients in the region face challenges in accessing specialized medicines.
- This study aimed to understand the RD care landscape in Argentina, Brazil, Chile, Colombia, Mexico, Peru, and Uruguay. Specifically, to determine patient access to diagnosis and treatments from a multi-stakeholder perspective, identifying cross-regional best practices translatable to RD care strategies among countries, and provide recommendations on addressing challenges.

# **Conclusions/recommendations**

Challenges	Recommendations
The absence of official patient registries leads to underestimated patient counts, impacting accurate resource allocation.	<b>Establish regional or national patient registries</b> that are accurately populated to guide policy, resource allocation, and research.
Health technology assessment (HTA) methodologies are not tailored to RD medicines, affecting accuracy, viability, and efficiency of evaluations.	<b>Implement differentiated HTA Mechanisms</b> tailored to RD treatments to promote accurate treatment evaluation, ensure meaningful representation, and facilitate efficient evaluation processes, prioritizing the patient perspective.
Significant delays in RD diagnosis result in treatment initiation delays, affect quality of life, and negatively impact patient outcomes.	Improve education on RDs for stakeholders, including healthcare providers (especially at the primary care level), payors, regulatory agencies, and the general public. Improve access to newborn screening and genetic counseling to reduce diagnostic delays.
High costs of RD medicines challenge healthcare system budgets and sustainability.	<ul> <li>Leverage negotiation approaches that have proven successful in other countries to secure optimal terms and conditions for treatment procurement, such as Uruguay's portfolio scheme and Argentina's risk-sharing for gene therapy.</li> <li>Establish a specific budget allocation for RDs, with innovative funding mechanisms, to ensure sustained access to RD treatments.</li> <li>Prioritize system sustainability as financial viability is critical to ensuring long-term access to treatments.</li> <li>Digitalize medical records to facilitate information sharing and avoid redundancy in care and resources.</li> </ul>
As RDs are generally not a national priority, changes in government administration affect the continuity of implemented initiatives.	<ul> <li>Prioritize RDs to ensure continuity and sustained implementation of RD initiatives across government transitions.</li> <li>Recognize the importance of PAOs as a stakeholder with valuable knowledge, insights, and inputs to guide policy.</li> </ul>
Absent or outdated clinical practice guidelines (CPGs) for RDs lead to unstandardized care and often limit access.	<b>Develop local guidelines for RD</b> through collaboration with local healthcare authorities and medical societies. <b>Establish physician training programs</b> to foster adherence to guidelines.

<sup>1</sup>This research is aligned with advancing the objectives of the UN resolution on RDs, which underscores the importance of meeting the needs of those living with RDs as crucial to the 2030 Sustainable Development Goals and the UN declaration, which includes RDs in universal health coverage.

# Methods

- Five experts from each country were invited to participate in a virtual task force. Participants included patient organization leaders, physicians, payors, regulators, and policymakers. They were recruited through a stakeholder mapping process and compensated for their time, in line with fair market value.
- Experts were provided with a literature search, agenda, and questions as preparation material for the task force. Seven virtual meetings were conducted (one per country) with the experts to discuss and compile data.
- The literature search was conducted using the terms "rare diseases", "access", "Fabry disease", and "Pompe disease" plus each "Latin America", "Argentina", "Brazil", "Chile", "Colombia", "Mexico", "Peru", and "Uruguay". The search included scientific publications, conference proceedings, local websites, and other gray literature.
- Each task force was moderated to ensure all participants were able to provide input, and comprehensive notes were taken. Each meeting lasted approximately 5 hours.
- Following the meetings, country-level reports were developed incorporating the literature search findings and task force insights.

# Results

• A scorecard was developed to rate each country based on ease of access, availability of treatments, and impact of medical societies and patient advocacy organizations (PAOs) on access.

# Figure 1. Regional scorecard

Countries	Ease of access to RD medicines	Contribution of medical societies to access	Contribution of PAO to access
Argentina		•	
Brazil			
Chile	•		
Colombia		•	
Mexico		•	•
Peru			
Wruguay	•		
<ul><li>High</li><li>Medium</li></ul>	Ratings are assigned considering each country's RD legislation and practices. For tr available treatments in the PHS, and sanitary approval for in-country commercializ		

# Argentina

#### *Key challenges*

Low

1. The extent of RD medicine and healthcare coverage is unclear. Sistema Unico de Reintegro por Gestion de Enfermedades (SURGE) is the reimbursement system for disease management. Insurers obtain partial to no reimbursement with long wait terms. Some treatments for Fabry disease and Pompe disease qualify for reimbursement through SURGE.

PHS, public healthcare system.

- 2. Treatment interruptions happen primarily because of lack of reimbursement by the health system to the insurers. Considering Argentina's annual inflation to be around 130%, insurers are reluctant to purchase or maintain treatment.
- . While the Administración Nacional de Medicamentos, Alimentos y Tecnología Médica (regulatory agency) has issued regulations that compel those with marketing authorization certificates to report their product's suggested retail price, there is no pricing regulation in Argentina.
- 4. 58% of the total legal recourses for medicines in the first semester of 2022 were for RD treatments.

# **Brazil**

#### Key challenges

- 1. Marketing authorization does not guarantee swift incorporation into the Sistema Único de Saúde (SUS; PHS). Elaprase's case, approved in 2008 but not incorporated by the Comissão Nacional de Incorporação de Tecnologias no Sistema Único de Saúde (CONITEC; regulatory agency) until 2018, exemplifies the time gap.
- 2. International reference pricing is often used for suggested Câmara de Regulação do Mercado de Medicamentos (CMED; chamber that approves treatment price) prices. This referencing was considered inadequate owing to diverse country budgets and price variations considered in dossier evaluation.
- 3. In 2019, the Ministry of Health spent R\$1.3 billion (~263 million USD) in providing treatments through judicialization, of which R\$1.2 billion were for RD treatments.

### *Key opportunities*

1. Streamline treatment incorporation pathway through collaboration with CONITEC for quicker RD treatment inclusion after approval from the Agência Nacional de Vigilância Sanitária (HTA agency) by using the oral presentation space offered to the industry and participating in public consultations. Engage

## Key opportunities 1. Leverage direct negotiation with Obras Sociales (social security)

- private and public sectors.
- Uruguay and Chile.
- to care.

#### Case examples

- risk-sharing strategy.
- plan, ensuring access.

### stakeholders to emphasize patient urgency for life-transforming treatments during incorporation processes.

- support initiatives.
- affect patient outcomes.

### Case examples

- also led to SUS incorporation.
- time to access.



treatments, rating is based on disease recognition, zation.

and Private Insurance and strengthen collaboration between the 2. Engage in regional price negotiations with neighboring countries

3. Develop additional support services, such as patient support

programs, therapeutic adherence monitoring, and complementary added-value services to improve patient access

#### • Argentina guaranteed access to onasemnogene abeparvovec, a gene therapy for spinal muscular atrophy (SMA), regardless of patient health insurance, negotiated under an innovative

• The Cystic Fibrosis Law 27.552 was approved in 2020, stating that medications in CPGs will be added to the Bank of Special Drugs

2. The government should engage industry stakeholders to align on more comprehensive contributions to patient access and diagnostic/patient support programs. Consider regulatory mechanisms to encourage industry commitment to access and

#### 3. CMED could use pricing benchmarks from countries that are more economically similar to Brazil for more accurate assessments (including other Latin American countries).

4. The Ministry of Health should ensure continuity of medicine procurement to address treatment delays and interruptions that

### • Price tends to be the main point of reference when there is not enough evidence to justify the expense during the CONITEC review. Price reductions of treatments for Fabry disease, infantile hemangioma, and an SGLT2 inhibitor for type 2 diabetes mellitus

• High-cost Gaucher treatments provided by RD reference center: with adequate vial dosage and storage conditions, patients no longer have to travel to main hospitals to receive treatment, reducing the peripheral access cost to patients and expediting

# Chile

### *Key challenges*

1. Ricarte Soto law was implemented in 2015 to manag provision of high-cost medications. As of 2023, the la 27 diseases, and the decree through which new dise technologies will be incorporated has been delayed

- 2. There is no suitable alternative in place for RD treatment reimbursement by the health system. RD treatments the Ricarte Soto law are often denied unless indicate by judicialization.
- 3. Despite no negotiation limitations or restrictions, the often take place. Industry has presented many strate (over 80) to both sectors, public and private, without
- 4. Strengthen HTA framework to improve the assessme

# Colombia

### *Key challenges*

- 1. The current government proposed a health reform a restructuring the system to eliminate the private Ent Promotora de Salud (EPS; health insurers of the publ and centralizing healthcare provision and financing. was revoked by the Senate, there is uncertainty and confidence in the healthcare system and its payors.
- . Despite having one of the region's most robust legisla frameworks for RDs, implementation issues leave a between what the law states and the reality.
- 3. Institución Prestadora de Salud (IPS; care-providing i have limited ability to finance high-cost treatments b reimbursement terms with the EPS average >200 day often even more delayed.

### *Key opportunities*

1. Collaborate with governmental bodies, medical socie PAOs to bridge the gap between legislation and imple establish working groups to address challenges, strea processes, and ensure effective execution of RD-focu

# Mexico

### Key challenges:

- 1. Mexico lacks specific legislation for RD. The General outlines the approval process for treatments, without RD differentiation.
- 2. As of 2015, genetic diseases are not covered through insurance, impeding patient access to treatments. Pa a significant financial burden.
- Varying clinical practice guidelines by each institution unstandardized care.
- 4. The closure of the Negotiating Commission in 2019 le the price negotiation process. Under the current adm a "base price" is now established, and institutions mu individually negotiate prices.

# Peru

### *Key challenges*

1. The absence of standardized processes to access hig treatments raises challenges in availability and afford

- 2. High importation taxes, particularly the 36% rate imp 2001, create significant commercial barriers.
- 3. The RD law requires the establishment of Advisory C by the Instituciones Administradoras de Fondos de Aseguramiento en Salud (health insurers) and by eac department to evaluate RD diagnosis and treatment However, only 19 out of 24 committees have been cr only two active in Lima.
- 4. Medicine prices significantly impact treatment contin Advisory Committees may grant approval, institution medicines because of budget constraints, even within Social de Salud (ESSALUD; social security).

# 

### *Key challenges*

- 1. There are no specific laws for RD coverage in Urugua decision-making process for treatment reimburseme Fondo Nacional de Recursos (FNR; national resource lacks clarity.
- 2. The FNR requires patients to complete testing to gua continuous access to treatment. If these tests are no treatment is interrupted.

### Key opportunities

1. FNR is open to negotiations with industry and is the c purchaser of high-priced medications in the country, several\$innovative purchasing mechanisms have bee implemented, including volume-based, portfolio-based risk-sharing agreements.

## Acknowledgments and disclosures

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