Challenges and Opportunities for Rare Diseases and Orphan Drugs in Latin America: An Industry Perspective

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Our purpose
We enable people with life-altering conditions to lead better lives.
Overview of presentation

Impact and Challenges of Rare Diseases

Opportunities and Common Goals

A Rare Disease Patient’s Journey
The impact of rare diseases is enormous

Nearly 7000 rare diseases are recognized\(^1\)

Effective treatments are available for only 1% of rare diseases\(^2\)

75% of those diagnosed with rare diseases are children\(^3\)

1/3 of children born with a rare disease are unlikely to see their 5th birthday\(^3\)
Additional challenges patients face in Latin America

- Few specialist centers exist for rare diseases in most countries
- Patients often have to travel long distances to get to the doctor and treatment
- National policies or plans for RDs very recent in most countries
- Funding of available treatments is a challenge for governments and patients alike
- Time to access for treatments can vary significantly in the region
The rare disease patient’s journey

- Challenges in getting to the right doctor
- Challenges with getting the right diagnosis
- Difficulty in accessing available treatments
Challenges in getting to the right doctor

Limited physician knowledge of the signs and symptoms of a rare disease

Rare diseases often hide behind the symptoms of other diseases.
Challenges in getting the right diagnosis

Patients make 8 doctor visits on average before getting a diagnosis

4.8 years is the mean average length of time from symptom onset to accurate diagnosis

40% of rare disease patients are misdiagnosed at least once
Difficulties in accessing treatment in Latin America

Once diagnosed, access to treatment is hugely variable.

There is a lack of dedicated processes to assess and fund orphan drugs.

Often the route to accessing treatment is judicial (Brazil and Columbia).

In Mexico, there are age thresholds for treatment.

We need to learn from global best-practices and create a special regulatory regimen for rare disease treatment & orphan drugs.
What are the opportunities for helping the patient’s on their journey?
The common goal is to improve the lives of patients with rare diseases.
We will only be successful if we all work together

We need **focused** and **united** efforts from all parties involved in the rare diseases space.

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Speaking with **One Voice**

Committed to **Change**

With shared **Goals And Values**
United effort to increase awareness of rare diseases – Mexico case study

• Photography exhibition held in the Mexican Congress on February 24, 2015 to celebrate Rare Disease Day
• Attended by patient associations, doctors, Mexican celebrities who support the cause, political and health authorities and media
• Extensive media coverage about the state of rare diseases in Mexico
Working together to elevate rare diseases to a national priority in Mexico

- Followed by a Legislative Forum sponsored by Congresswoman Beatriz Yamamoto held on February 25 with testimonials of patient associations
- Charter released outlining priorities for the next four years:
  - Establishment of National Registry
  - Formation of a National Council

Chart for Rare Disease 2015-18

Physicians

Patient Advocacy Groups
Because people with rare diseases never give up, and neither should we