Patient-Centred Healthcare: Rare Diseases and Personalized Medicines

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IAPO-LATAM Regional Meeting
August 24, 2015
How Rare Disease Policy Happens

- (Almost) every rare disease/orphan drug policy was the result of patient group advocacy; the history of rare disease policy is the history of patient organizations

- Most rare disease patient groups are started by patients and parents; some are started when a new orphan drug is developed

- History of rare disease groups is also history of orphan drug discovery and pharmaceutical industry support

- Rare disease groups are (even more) impactful when they work in partnership with other groups and with other stakeholders

- Regional and international rare disease alliances will be even more important in the future
Patient Group-Orphan Drug-Industry Relationship

- Patient Advocacy Group
- Orphan Drug Policy
- New Orphan Drug
- PAG Industry Support
- Rare Disease Programs
Developments in Orphan Drug Regulation

1983: Orphan Drug Act
1993: EU Council Resolution on Orphan Drugs
1995: Australia Orphan Drug Policy
1998: EU Regulation on Orphan Medicinal Products
1999: Brazil Regulation 199
2014: Japan Orphan Drug Regulation

USA Orphan Drug Act: A Mother, a Congressman, an Actor

Harmonized Orphan Drugs/Rare Diseases Policy - Latin America

May 2015
Decade prior to 1983: only 10 new drugs for rare diseases

1980: Abbey Meyers’ son with Tourette Syndrome was receiving drug through clinical trial; CT halted and drug no longer available

Young man with Tourette’s; mother contacted Congressman Waxman, who held Congressional hearing; but no drug companies testified

Jack Klugman featured story on his TV show deploring lack of economic incentives for orphan drug development

1982: Another TV show, another congressional hearing, this time with drug companies supporting call for Orphan Drugs

1983: USA passes world’s 1st Orphan Drug Act providing economic incentives for orphan drug development

2015: FDA approves 500th Orphan drug; OD Act has benefitted more than 15 million patients
Countries in Latin America Represent a Range Of National Plan (NP) Policy Scenarios Which Inform the Rare Disease (RD) Global Marketplace

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<th>Country</th>
<th>Status</th>
<th>Details</th>
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<tbody>
<tr>
<td>Mexico</td>
<td>In development: RD NP passed</td>
<td>- N.P. proposed but stalled</td>
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<td>- No specific budget for Orphan Drugs</td>
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<td>- A small number of rare diseases are covered by public insurance but access is very limited</td>
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<td>- Collation of groups push for RD patient needs; fragmented databases</td>
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<td>Brazil</td>
<td>Under development: RD NP adopted</td>
<td>- N.P. passed and being implemented</td>
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<td>- MOH to allocate resources for special health centers</td>
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<td>- No special coverage for OD; patients go to court to access to treatments</td>
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<td>- Patients groups influence rare disease policy; no national registry</td>
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<td>Argentina</td>
<td>Early stage: RD NP proposed</td>
<td>- N.P. adopted but not implemented</td>
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<td>- Centralized funding in place (SUR); care plan under development</td>
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<td>- N.P. calls for coverage of RD treatments; no early access programs in place</td>
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<td>- Patients organizations help provide education; N.P. calls for national patient registry</td>
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Argentina: National Plan & Policy Framework

- No specific regulations regarding early authorization process for orphan drugs

- National plan requires coverage of RD treatment; fund exists for certain diseases.

- Networks of coordinated care and special diagnosis programs proposed in national plan; not yet implemented.

- National rare disease registry proposed in national plan; not yet implemented
Brazil: National Plan & Policy Framework

- No specific regulations regarding early approval process for orphan drugs; fast track available, but timeline varies.

- No special funds or evaluation considerations for access to ODs; new protocols for rare diseases in development.

- Networks of coordinated care and special diagnosis programs proposed in national plan; not yet implemented.

- No registry proposed in national plan; Sao Paolo registry program (DORA) in development.
Chile: National Plan & Policy Framework

- No specific regulations regarding early authorization process for orphan drugs

- **Jan 2015 Law for financing of rare disease drugs. Investment of 200 billion pesos.**

- No national plan for diagnosis and treatment centres.

- National rare disease registry proposed in national plan; not yet implemented
2010 Orphan Disease Law recognizes orphan diseases as significant. Rules for protection.

2013 Ministry of Health (Colombian Fund for High Cost Diseases) conducted census of patients with orphan diseases, reporting 13,168 cases.

- No Networks of coordinated care and special diagnosis.

No registry proposed in national plan; Sao Paolo registry program (DORA) in development.
Fast track approval process for orphan drugs in place through “letter of recognition”

No special funds or considerations for access to orphan drugs; use of catastrophic illness fund for certain diseases.

Networks of coordinated care proposed in draft national plan; prioritization of resources for diagnosis & screening.

No rare disease registry proposed in draft national plan; separate initiatives to collect data exist.
Peru: National Plan & Policy Framework

- 2011: Legislation promoting treatment and national strategy for RDs
- No special funds or considerations for access to orphan drugs; use of catastrophic illness fund for certain diseases.
- Proposed diagnosis, surveillance, & prevention.
- No rare disease registry proposed in draft national plan; separate initiatives to collect data exist.
Lifecycle Approach with Patient Input and Industry & Real-World Data

- **Patient Input**
  - Regulator: Approval on Benefits-Risks-Uncertainties; Use & Real-World Monitoring
  - HTA: Comparison Benefits, Risks, Cost w/Alternatives; Place in Therapy

- **Industry Data**
  - Payer: Budget Impact; Access Criteria; R-W Data Collection
  - Researcher/Clinician: Disease Knowledge; Drug Discovery; Treatment Guidelines
  - Company: Clinical Trials & Outcome Measures; biomedical, clinical, PROs, Real-World Impact

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Incremental Value Added (effectiveness, side effects, tolerability, improved quality of life) may not equal incremental costs.

Pricing criteria may not be established, and willingness to pay may have little impact on pricing.

Medicines for rare and unmet needs tend to have high R&D, high uncertainty, high cost.

Reimbursement strategies may be directed toward reducing uncertainty in safety, effectiveness, appropriate use, and budget impact.

Managed access schemes include registries, CED, prior authorization, limited use, $ capitation.
Innovative (combination, breakthrough) medicines tend to be much more expensive than older standard treatments.

Personalized therapy may have no standard comparator.

Personalized therapies (for serious, life-threatening) conditions may be approved with less definitive outcomes (surrogate, biomarkers, short-term measures).

Targeted patient population may be small (rare conditions, subtypes) so outcomes are less robust.

Accuracy of test x multiple factors influencing outcomes => high uncertainty of PM impact.

Cost of testing unaffected patients => low overall ROI.

Do PMs justify higher $/QALY?
Why Managed Access Programs

- Arrangement between manufacturer and payer that enables payment for a drug under specified conditions
- AKA risk-sharing agreements
- High uncertainty in safety and effectiveness
- Uncertainty of patient numbers (diagnosis, eligibility)
- Uncertainty of long-term benefit vs. harms and health outcomes (QoL, survival)
- High cost (individual and total budget impact)
Innovative Pricing Models: Relevance to Personalized Medicines?

- Outcomes based / treatment response (individual)
- Value based pricing (sub populations)
- Achievement of overall treatment (population)

- Price & volume agreements
- Capitation agreements (patient or population)
- Portfolio agreements (patient or population)
- Portfolio trade-offs

- Value added services

- Differential pricing
Thank You!

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