Patient-Centred Healthcare: Rare Diseases and Personalized Medicines

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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 others groups and with other stakeholders
- Regional and international rare disease alliances will be even more important in the future

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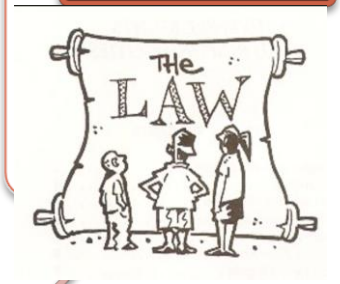
Patient Group-Orphan Drug-Industry Relationship

Patient Advocacy Group

Orphan Drug Policy

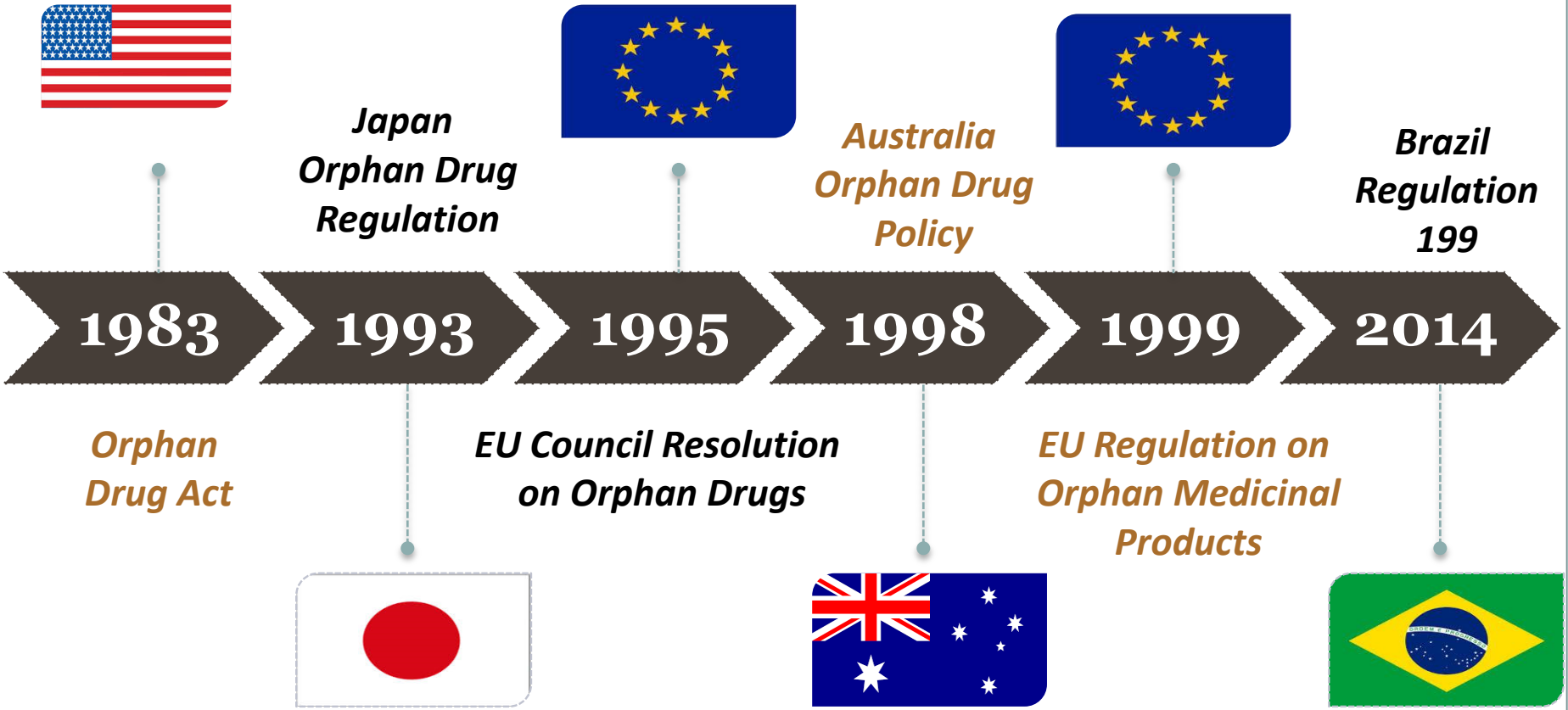


New Orphan Drug

PAG Industry Support

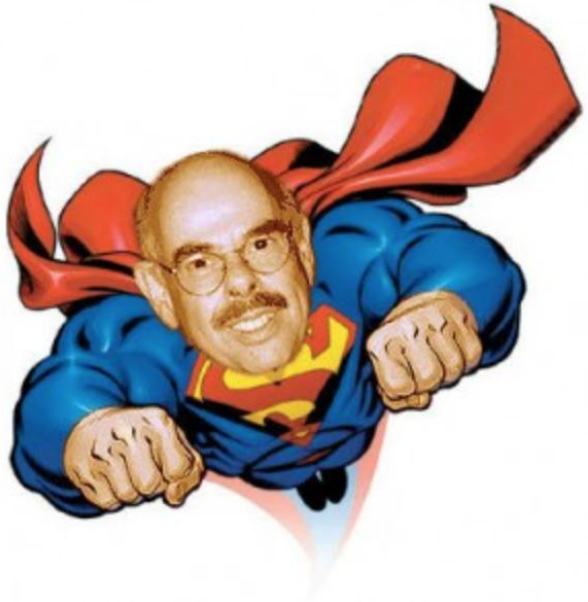
Rare Disease Programs

Developments in Orphan Drug Regulation



- Source: Orphanet Global Policy. Accessed April, 2015. <http://www.orpha.net/consor/cgi-bin/Education_AboutOrphanDrugs.php?lng=EN>

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



Canadian Organization for Rare Disorders

USA Orphan Drug Act: How Did It Happen?

- 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

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Countries in Latin America Represent a Range Of National Plan (NP) Policy Scenarios Which Inform the Rare Disease (RD) Global Marketplace

- Early stage: RD NP proposed
- In development: RD NP passed
- Under development: RD NP adopted

Mexico

- N.P. proposed but stalled
- No specific budget for Orphan Drugs
- A small number of rare diseases are covered by public insurance but access is very limited
- Collation of groups push for RD patient needs; fragmented databases

Brazil

- N.P. passed and being implemented
- MOH to allocate resources for special health centers
- No special coverage for OD; patients go to court to access to treatments
- Patients groups influence rare disease policy; no national registry

Argentina

- N.P. adopted but not implemented
- Centralized funding in place (SUR); care plan under development
- N.P. calls for coverage of RD treatments; no early access programs in place
- Patients organizations help provide education; N.P. calls for national patient registry

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***

Columbia: National Plan & Policy Framework

- ✓ ***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.***

Mexico: National Plan & Policy Framework

- ✓ ***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

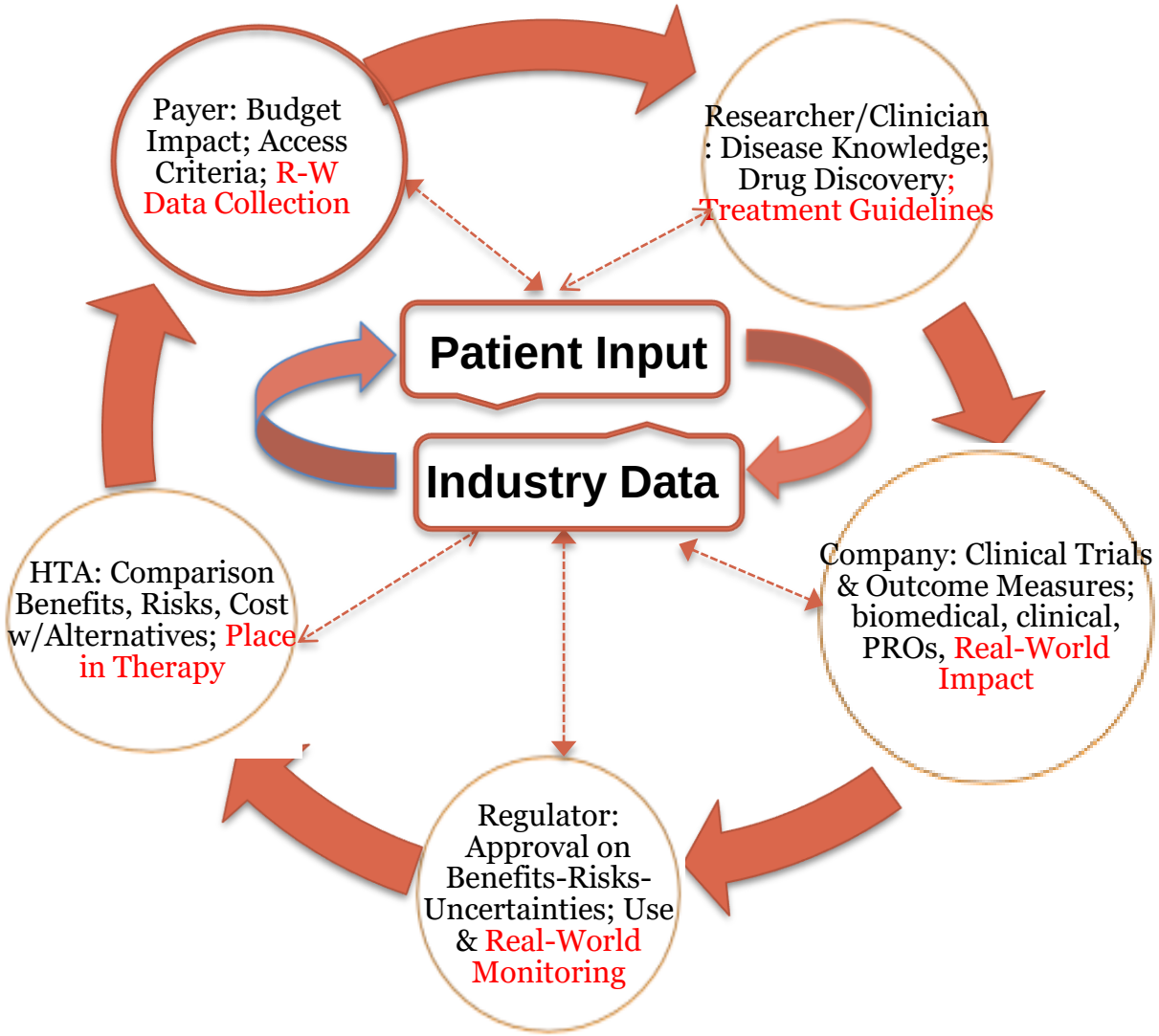
- ✓ ***2011: Legislation promoting treatment and national strategy for RDs***

- ❖ ***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



Rare Disease Drugs: Challenges for Reimbursement

- 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.

HTA: Challenge to Funding of Personalized Medicines?

- 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

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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?

Performance	**Outcomes Guarantee**	▪ Outcomes based / treatment response (idividual) ▪ Value based pricing (sub populations) ▪ Achievement of overall treatment (population)
Financial	**Capping/Rebates**	▪ Price & volume agreements ▪ Capitation agreements (patient or population) ▪ Portfolio agreements (patient or population) ▪ Portfolio trade-offs
	Portfolio	
Other	**Services**	▪ Value added services
Affordability	**Consumer Oriented**	▪ Differential pricing

Canadian Organization for Rare Disorders

Thank You!

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