INCENTIVES FOR DEVELOPING ORPHAN DRUGS AROUND THE WORLD

ICORD, Mexico City - 16 October, 2015

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Introduction – Problem Statement

- 6-8% of the worldwide population suffer from a rare disease
- Less than 10% of pharmaceutical spending is invested in rare diseases
- Why so few orphan drugs?
  - Stringent requirements for authorization of medicinal products that make R&D programs a challenge
  - Too small populations to secure return on investment
- Yet, more and more rare diseases are identified so the problem intensifies

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Introduction – Problem Solution

- Encourage both research in rare diseases and development of orphan drugs
- How?

➢ **Specific legislation for Orphan Drugs that include incentives for their development**

  - A few countries have adopted a specific orphan legislation with incentives
  - Some countries actively promote the treatment of rare diseases but lack adequate orphan legislation
  - Most countries have no specific orphan legislation or rare disease program

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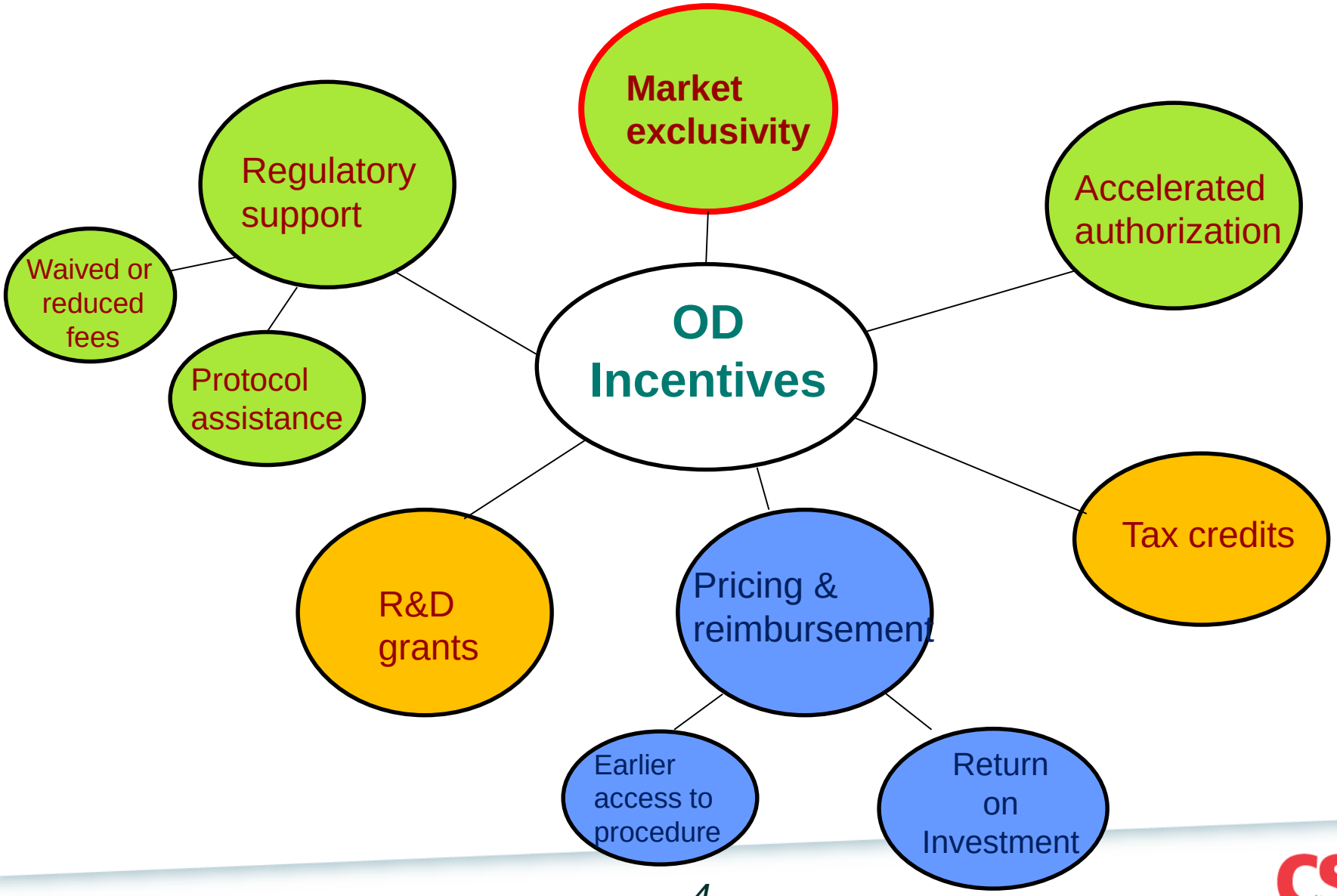
What are the most common incentives?



What is Market Exclusivity?

- Competent authority may not authorize a same/similar medicinal product for the same therapeutic indication

  → **Protection against competition**

- Market exclusivity duration, which is valid from Marketing Authorization (MA), varies country by country, e.g.:

- **USA**: 7 years

- **EU**: 10 years
  (+ 2 years if paediatric development)

- **Japan**: 10 years

Countries with Well Established Orphan Drug Regulations

	USA	EU	JAPAN	AUSTRALIA	SWITZER-LAND	ARGENTINA
Regulation	ODA 1983	EC 141/CE 2000	ODR 1993	ODP 1998	OASMéd (2006)	4622/2012
Competent Authorities	FDA/OOPD	EMA/COMP	MHLW/PMDA	TGA	SwissMedic	ANMAT
Prevalence (10,000)	200,000 inhabitants (≤7.5/10,000)	≤5/10,000	50,000 inhabitants ≤4/10,000	2000 inhabitants ≤1.1/10,000	≤5/10,000	≤1/2,000
Market Exclusivity	7 years	10 years (+2 for peadiatrics)	10 years	No	No	No
Taxes Reduction	Up to 50% for Clinical Trial	managed by Member States	12% calculation as tax credit deduction	No	No	No
R&D Grant	< NIH program	CE/ MS	< Government	No	No	Yes
Exemption of Regulatory Fee	Yes	Reduced (Advice, Inspection and MAA)	Reduced	Yes	No	No
Protocol Assistance	Yes	Yes	Yes	No	Yes	Yes

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Overview of Current & Emerging Orphan Drug Regulations/Laws

Country	Specific Regulation/Law with provisions for Orphan Drugs	Prevalence Definition	Priority/ Accelerated Review	Reduced Data Requirement	Data Protection Incentive
Canada	No Initial *draft* discussion document	≤5/10,000	✓		
India	No Some specific provisions for ODs		✓	✓	
Kazakhstan	**Yes**	Not defined			
Malaysia	**Yes** Submission pathway not described	Not available	✓	✓	
Philippines	Senate bill available, actual regulation not listed on official site				
Russia	**Yes** Federal Law FZ-61	≤10/100,000	✓	✓	
Saudi Arabia	Yes		✓		
S. Korea	**Yes**	<20,000		✓	
Singapore	**Yes**	<20,000	✓	✓	
Taiwan	**Yes**	≤1/10,000	✓	✓	
Turkey	No, *draft* available	≤5/100,000	✓	✓	✓
Ukraine	**Yes**	≤5/10,000	✓	✓	

Overview of Current Orphan Drug Regulations in Latin America

Country	Official Regulation (Law) for Orphan Drugs	Prevalence Definition	Priority/ Accelerated Review	Reduced Data Requirement	Data Protection Incentive
Mexico	No Decree in law to recgonize ODs	≤5/10,000	✓	Same as for a new molecule	
Argentina	Yes	≤1/2000	✓	✓	✓
Chile	No, MOH Recomendations for OD registration	≤5/10,000	✓	✓	
Columbia	No, Decree for vital medicines Applicable to ODs		✓		
Costa Rica	No Some specific provisions for ODs				
El Salvador	No Some specific provisions for ODs				
Guatemala	No Some specific provisions for ODs				
Honduras	No Some specific provisions for ODs				
Nicaragua	No Some specific provisions for ODs				
Panama	Yes		✓	✓	✓

Medicine intended to treat a rare, severe or patient disabling illness and where the commercial interest or stimulus are unlikely.

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Latin American Countries without any Provision for Orphan Drugs

Bahamas

Barbados

Bolivia

Brazil

Dominican R.

Ecuador

Jamaica

Paraguay

Peru

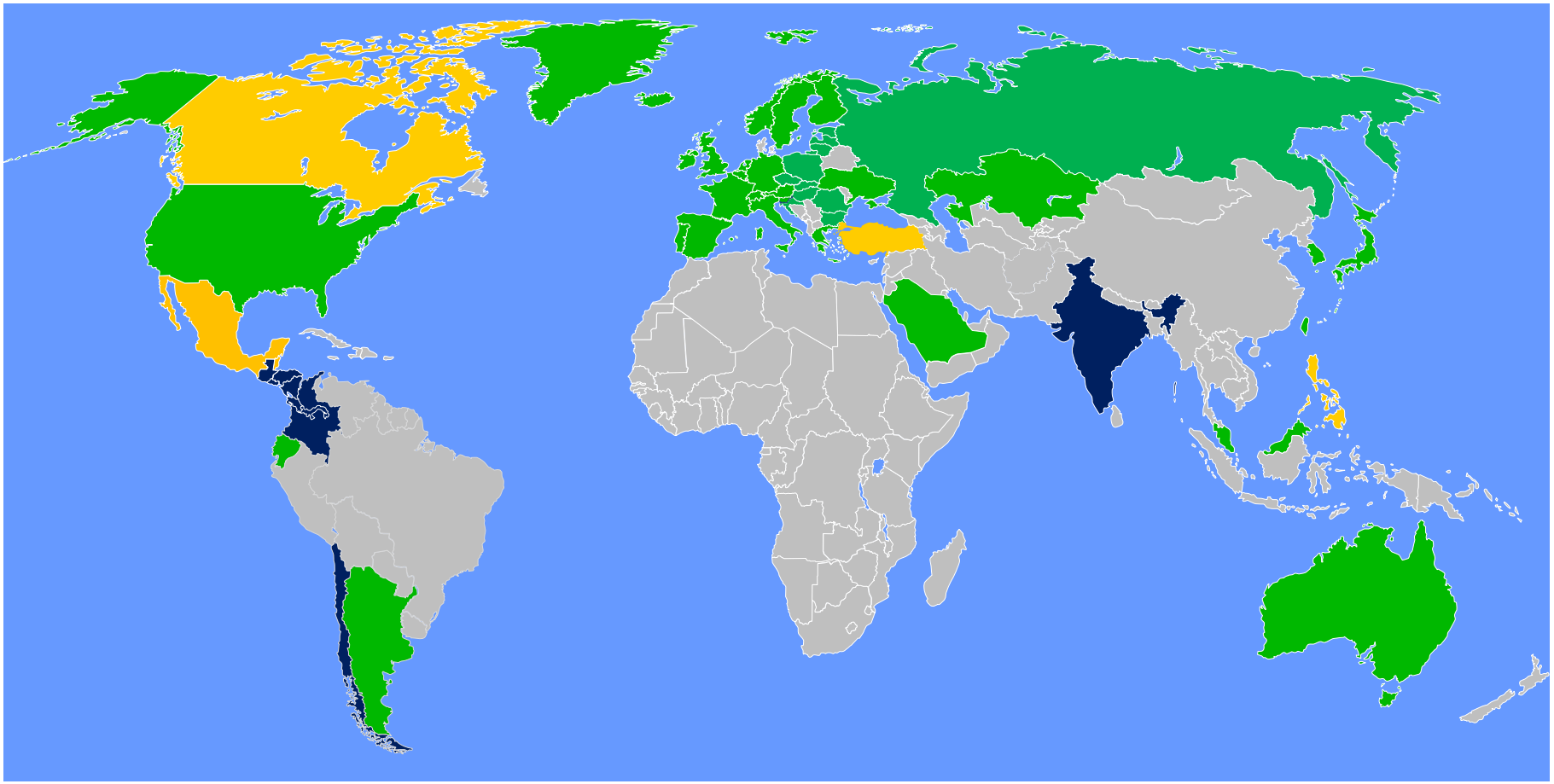
Puerto Rico

Trinidad & Tobago

Uruguay

Venezuela

Orphan Drug Regulations/Laws or provisions (2015)



OD Regulation /Law available

Draft Orphan Drug Regulation available

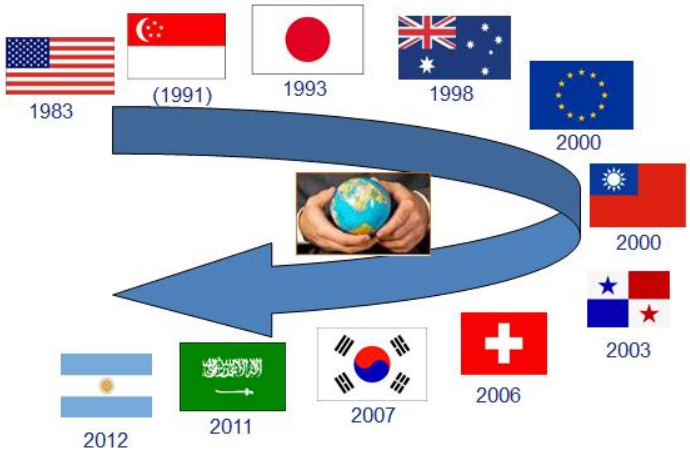
Official provisions from which Orphan Drugs can benefit (but which are not specific to Orphan Drugs)

Summary

- Incentives enacted in US & EU laws have given birth to an Orphan Drug industry and increased focus on Rare Diseases.

However:

- Only a small number of countries have adopted specific orphan drug laws
- A few countries have implemented incentives for orphan drug development.



➢ **More regulations/laws providing incentives for Rare Disease research and Orphan Drug development are lacking in the majority of countries around the globe.**

Conclusions

Given the characteristics of rare diseases (serious, debilitating life threatening) and their increasing number:

- Governments should consider them as a **priority** and **adopt specific regulations/laws** that **incentivize**
  - **Research in Rare Diseases**
  - **Development of Orphan Drugs**
  - **Patient Access to such treatments**

It is in our hands to improve the lives of patients with Rare Diseases globally!