Challenges in Medicines Funding for Rare Diseases

ISPOR Barcelona

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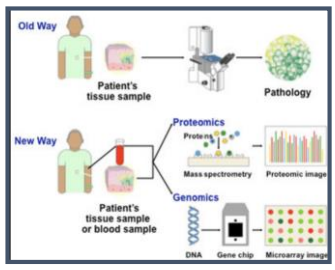
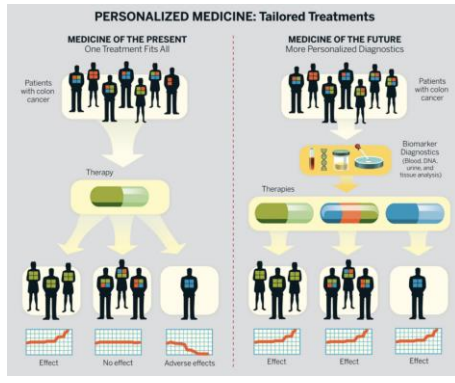
Proteomics and genomics are transforming Medicine



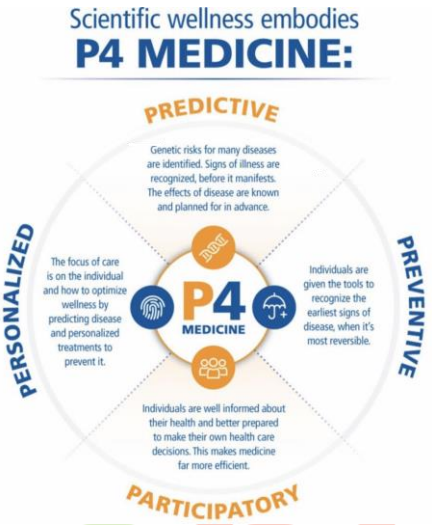


Moving from:
- N to n
- $ to $$$

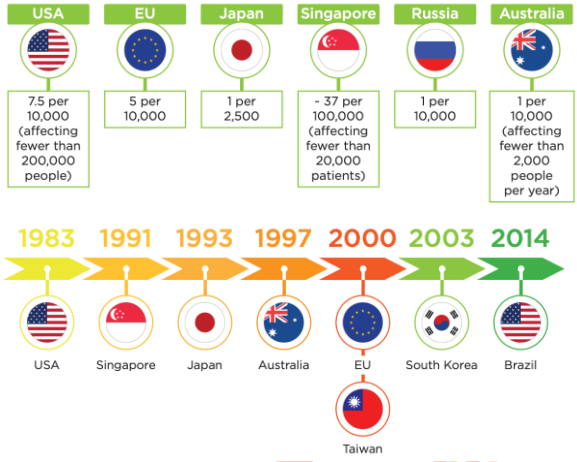
Population Medicine

Personalized Medicine

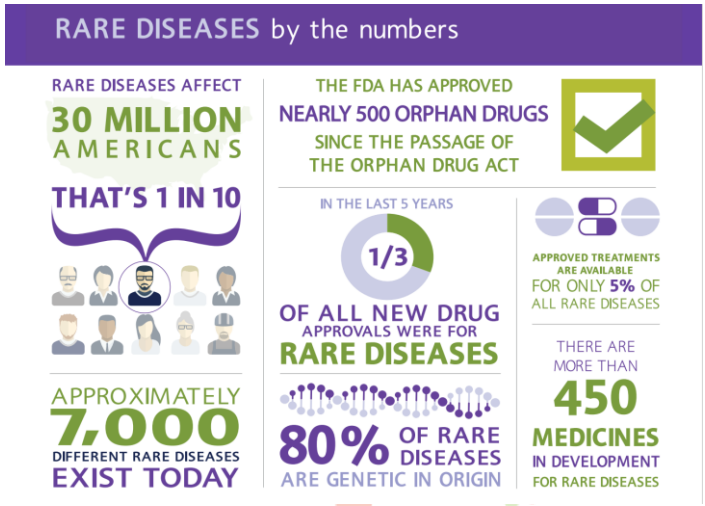
A new paradigm shift based on P4 Medicine is ongoing



Rare Diseases approach vary by country estimating that around 350 million people (5%) will be impacted worldwide



Rare Diseases are gaining momentum



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Challenges of orphan drug development

COMPLEXITY

- Complexity of the diseases
- Reduced patient population
- Heterogeneity of the disease
- Little or null knowledge on the history of the disease
- Limited medical and scientific knowledge

COSTS

- Up to 15 years before commercialization
- Development costs
- Clinical trials in multiple countries to assure patient recruitment
- Production to guarantee supply

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Policy Principles to succeed I

❑Ensuring rare diseases are a *public health priority*

❑Gain *patient centricity* throughout stronger *empowerment*

- ✓ *Access to information at all levels*
- ✓ *Greater involvement in clinical research and evidence-based decision making*
- ✓ *Emphasis on patient-reported-outcomes registries*
- ✓ *Partnering regulatory decisión making*

Source: IFPMA

Policy Principles to succeed II

❑ *Incentivating continued Research and Development*

- ✓*Radical collaboration*
- ✓*Basic Research funding*
- ✓*Regulatory frameworks estimulating innovation*
- ✓*Data generation through disease registries*
- ✓*Proactive approach with regulators bodies & payers*

Policy Principles to succeed III

❑Ensuring *sustainable patient access* along their patient journey

- ✓ *Maximize RD knowledge by healthcare actors*
- ✓ *Screening and better diagnostic testing (prevent when posible)*
- ✓ *Complement treatment with specialiazed support services*
- ✓ *Moving beyond Price & Short term impact*
- ✓ *More holistic, joined-up approach to find a multistakeholder partnership solution*

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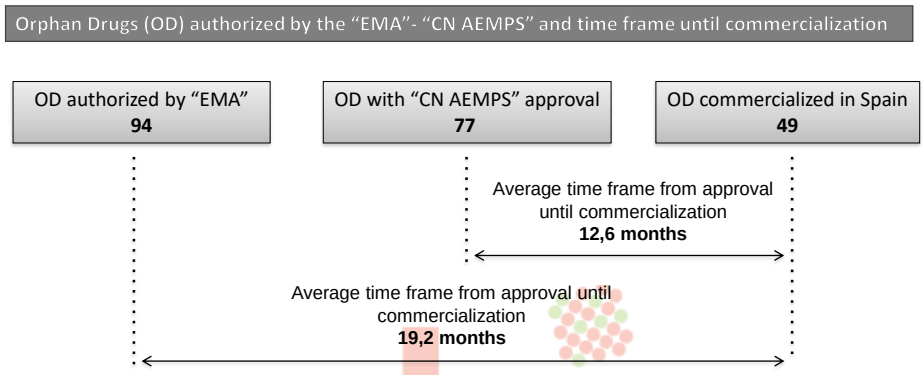
Spain: Study report on orphan drugs

ORPHAN DRUGS AUTHORIZED BY THE "EMA" AND COMERCIALIZED IN 2002-2006



The total time frame from "EMA" authorization until CN attainment must be attributed to the company's application date instead of the AEMPS.

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Spain: the # of OD pending of reimbursement is worriedly growing

ANALYSIS OF ORPHAN DRUGS (OD) AUTHORIZED IN EUROPE-SPAIN DURING 2002-2011 AND 2012-2016

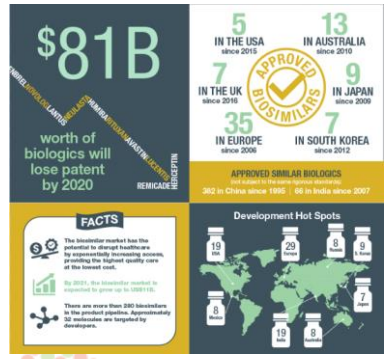
	2002-2011	2012-2016
OD authorized by "EMA" with active orphan designation	42	58
OD authorized with "P&R" in Spain	38 (90,5%)	18 (31%)
OD that haven't applied for "CN" in Spain	0	17 (29,3%)
OD in process of "P&R"	4 (9,5%)	23 (39,7%)

Increase in the number of OD pending for "P&R" in Spain:
9,5% (2002-2011) to 39,7% (2012-2016)

Spain: 29% of OD are currently not commercialized

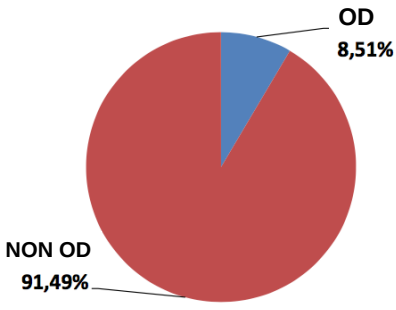
Orphan drugs (OD) status in Spain on August 31st 2018		
OD with "OD" designation-approved by "EMA"	**108**	
OD with national code of the "AEMPS"	**91**	*84%*
OD commercialized in Spain	**60**	*56% with respect to the authorized by the EMA*
OD not commercialized in Spain	**31**	*29% with respect to the the authorized by the EMA*

It is possible to fund the innovation?

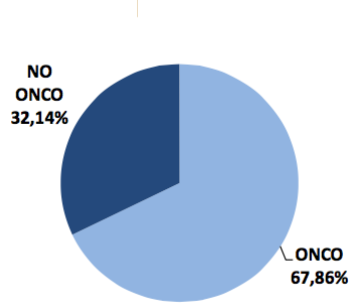


Bugdetary impact

Hospital Sales



Orphan Drugs (OD)



- Hospital sales data from 2017: 9,517 million euros
- **Hospital sales of OD to "PVL": 809,66 million euros (8,5% of total hospital pharmaceutical expense**

Concluding remarks

- Orphan medicinal products have been proven to have a significant impact in patients' lives and well-being
- Industry interest and focus in Rare Diseases is increasing
- Implement a National plan for Rare Diseases with sufficient funding
- Need to develop policies to ensure that patients with RD have access to high-quality care
- An earlier collaboration between all actors in the value chain, with industry together with regulators, HTA, and payers has the potential to lead to earlier and more sustainable patient access

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