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 $$$

PERSONALIZED MEDICINE: Tailored Treatments

Effect

Medicine of the Present

Medicine of the Future

New Horizons in Medicine

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

RARE DISEASES by the numbers

- RARE DISEASES AFFECT 30 MILLION AMERICANS THAT'S 1 IN 10
- APPROXIMATELY 7,000 DIFFERENT RARE DISEASES EXIST TODAY
- THE FDA HAS APPROVED NEARLY 500 ORPHAN DRUGS SINCE THE PASSAGE OF THE ORPHAN DRUG ACT
- IN THE LAST 5 YEARS 1/3 OF ALL NEW DRUG APPROVALS WERE FOR RARE DISEASES
- 80% OF RARE DISEASES ARE GENETIC IN ORIGIN

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
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 decision making

Policy Principles to succeed II

- Incentivizing continued **Research and Development**
  - Radical collaboration
  - Basic Research funding
  - Regulatory frameworks stimulating 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 possible)
  - Complement treatment with specialized 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 COMMERCIALIZED IN 2002-2006**

<table>
<thead>
<tr>
<th>OD authorized by “EMA”</th>
<th>OD with “CN AEMPS” approval</th>
<th>OD commercialized in Spain</th>
</tr>
</thead>
<tbody>
<tr>
<td>94</td>
<td>77</td>
<td>49</td>
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Average time frame from approval until commercialization:
- 12.6 months
- 19.2 months

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

<table>
<thead>
<tr>
<th></th>
<th>2002-2011</th>
<th>2012-2016</th>
</tr>
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<tbody>
<tr>
<td>OD authorized by “EMA” with active orphan designation</td>
<td>42</td>
<td>58</td>
</tr>
<tr>
<td>OD authorized with “P&amp;R” in Spain</td>
<td>38 (90,5%)</td>
<td>18 (31%)</td>
</tr>
<tr>
<td>OD that haven’t applied for “CN” in Spain</td>
<td>0</td>
<td>17 (29,3%)</td>
</tr>
<tr>
<td>OD in process of “P&amp;R”</td>
<td>4 (9,5%)</td>
<td>23 (39,7%)</td>
</tr>
</tbody>
</table>

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

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<tr>
<td>OD with “OD” designation- approved by “EMA”</td>
<td>108</td>
<td></td>
</tr>
<tr>
<td>OD with national code of the “AEMPS”</td>
<td>91</td>
<td>84%</td>
</tr>
<tr>
<td>OD commercialized in Spain</td>
<td>60</td>
<td>56% with respect to the authorized by the EMA</td>
</tr>
<tr>
<td>OD not commercialized in Spain</td>
<td>31</td>
<td>29% with respect to the the authorized by the EMA</td>
</tr>
</tbody>
</table>
It is possible to fund the innovation?

Bugdetary impact

- 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