Inventory of Access and Prices of Orphan Drugs across Europe:
A Collaborative Work between National Alliances on Rare Diseases & Eurordis

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Access to care in the current context

• Each European country faces economic difficulties
• There is a temptation to stop and even reduce the improvements obtained for patients with rare diseases

Orphan Drugs are the ideal scape goat:
- Some Orphan Drugs are individually expensive - true
- Some Orphan Drugs are actually profitable - good
  but also
- “Pharma Industries are bad boys”
- “RD Patient Organisations are communitarian lobbyists”

• Some unethical positions expressed by few policy makers and academic leaders
Protecting Orphan Drugs

- We must defend this innovative process of drug development
  - From a ‘Justice’ point of view
  - As a pilot for innovation & access to innovation
- We must struggle against misconceptions about ODs
  - “The most profitable sector in pharma”
  - “The tsunami of ODs” – refer to EURORDIS’s counter analysis
  - “The “Glivec” precedent as the “orphan blockbuster”
  - “OD are not accurately evaluated”...........

Inventory of the real-life situation of European Patients with respect to ODs

Discuss strategies to improve overall access for European Patients to ODs
‘Orphan Drug’ life: the stakeholders

Development
- Pharma Industry
- Academic Researchers
- Patient Organisations
- European disease networks
- EMA
- EURORDIS

Access
- National Competent Authorities
- National Insurance Systems
- Pharma Industry
- Patient Organisations
- National Alliances

A European frame, because ODs are generated through a European policy

A multi-National frame, because Patients are primarily supported by national policies
In July 2010, the EURORDIS Board of Directors decided to draw up an inventory of access and prices of ODs across Europe.

**National Alliances**
- Collected the data
  - Through own POs network
  - By contacting
    - Pharmacies
    - Insurances
    - Professionals.....

**Eurordis**
- Provided a common data collection template
- Registration and analyses
- Feedback of the overall results to participants
10 National Alliances collaborated

- Belgium
- Denmark
- France
- Greece
- Hungary
- Italy
- The Netherlands
- Romania
- Spain
- Sweden

representing

2.2 Millions patients* among 256 Millions EU citizens from 10 countries

returns

480 cards describing the real life situation of 60 ODs

* patients concerned by an authorised OD according to the prevalence of the condition and the national population
Availabilty of 60 ODs with EU MA, by country

Number of available ODs in each country

- **FRA**: 93%
- **NTL**: 88%
- **DNK**: 87%
- **ITA**: 67%
- **HUN**: 67%
- **BEL**: 65%
- **ESP**: 33%
- **ROM**: 28%
- **GRE**: 25%
- **SWE**: 7%

Legend:
- Black: unavailable
- Gray: ?
- Green: available
Patients with potential access to ODs, by country

% of patients with potential access

- **FRA**: 98%
- **NTL**: 89%
- **DNK**: 87%
- **ITA**: 77%
- **HUN**: 78%
- **BEL**: 70%
- **ESP**: 49%
- **ROM**: 36%
- **GRE**: 34%
- **SWE**: 2%

Legend:
- **unavailable**
- **available**

Note: For one country, the data is marked as unavailable (?).
Rarest ODs, less available?

% of «access»

- Number of ODs available
- Patients accessing to ODs

Countries: FRA, NTL, DNK, ITA, HUN, BEL, ESP, ROM, GRE, SWE

Access percentages vary across countries.
Availability by therapeutic field: the products

- **Metabolic Diseases (n=14)**: 64% available, 36% unavailable
- **Oncology (n=23)**: 59% available, 41% unavailable
- **Others* (n=23)**: 47% available, 53% unavailable

*others: cardiology, neurology, pneumology, hematology, immunology, inflammation,*
Access by therapeutic field: the population served

* others: cardiology, neurology, pneumology, hematology, immunology, inflammation,

- Number of ODs available
- Patients accessing to ODs

<table>
<thead>
<tr>
<th>Condition</th>
<th>ODs Available</th>
<th>Patients Accessing</th>
</tr>
</thead>
<tbody>
<tr>
<td>Metabolic Diseases</td>
<td>n=14</td>
<td></td>
</tr>
<tr>
<td>Oncology</td>
<td>n=23</td>
<td></td>
</tr>
<tr>
<td>Others*</td>
<td>n=23</td>
<td></td>
</tr>
</tbody>
</table>
## Patients with access or not to Orphan Drugs

<table>
<thead>
<tr>
<th>Target population*</th>
<th>metabolism</th>
<th>oncology</th>
<th>others</th>
<th>total</th>
</tr>
</thead>
<tbody>
<tr>
<td>With access</td>
<td>237 000</td>
<td>1 268 000</td>
<td>739 000</td>
<td>2 244 000</td>
</tr>
<tr>
<td>Without access</td>
<td>73 000</td>
<td>212 000</td>
<td>206 000</td>
<td>491 000</td>
</tr>
</tbody>
</table>

* patients concerned by an authorised OD according to the prevalence of the condition
Diffusion of ODs across Europe by therapeutic field

Number of ODs according to their level of diffusion

- **metabolism**
- **oncology**
- **others**

0-4 countries
5-6 countries
7-9 countries

0 2 4 6 8 10 12

0 2 4 6 8 10 12
21 Orphan Drugs widely available

- Tracleer™
- Vidaza™
- Volibris™
- Exjade™
- Fabrazyme™
- Somavert™
- Aldurazyme™
- Myozyme™
- Naglazyme™
- Glivec™
- Tasigna™
- Afinitor™
- Lysodren™
- Nexavar™
- Sprycel™
- Torisel™
- Yondelis™
- Evoltra™
- Litak™
- Revlimid™

**7-9 countries**

- **13% of others (23)**
  - Tracleer™
  - Vidaza™
  - Volibris™

- **52% of oncology (23)**

- **35% of metabolism (14)**

- **35% of metabolism (14)**
25 Orphan Drugs with limited availability

- Elaprase™
- Kuvan™
- Orfadin™
- Replagal™
- Wilzin™
- Carbaglu™
- Cystadane™
- Increlex™

- Atriance™
- Savene™
- Arzerra™
- Busilvex™
- Trisenox™

- Pedea™
- Revatio™
- Ventavis™
- Firazyrtm
- Nplate™
- Xagrid™
- Thelin™
- Mozobil™
- Revolade™
- Inovelon™
- Prialt™
- Onsenal™

- 52% of others (23)
- 22% of oncology (23)
- 57% of metabolism (14)
- 25-6 countries

- # Orphan Drugs

- oncology
- metabolism

- EURORDIS
  Rare Diseases Europe
14 Orphan Drugs scarcely available

- Thalidomide
- Gliolan™
- Tepadina™
- Photobarr™
- Ceplene™
- Mepact™
- Soliris™
- Diacomit™
- Ilaris™
- Firdapse™
- Arcalyst™
- Cayston™
- Siklos™
- Nymusa™

None of metabolism

- 35% of others (23)
- 26% of oncology (23°)
Availability of ODs by date of Marketing Authorisation

- Date of MA

- Countries

- Metabolism
- Oncology
- Others
Mean national price* vs minimum EU price

* for each OD, the national price was compared to the lower in EU, these ratios were thereafter averaged by country
* for each OD, the national price was compared to the mean EU value, these ratios were thereafter averaged by country
Individual Price vs Collective Cost

Pharma Perspective
To assure return on investment (previous development) and further profits (including for R&D)

NCA Perspective
To make financially possible the availability of ODs. To foresee possible significant cost increase (number of patients)

2 parameters for mastering costs
- the actually paid price
- the number of patients treated
Individual Price vs Collective Cost:

at least 4 actual strategies known

- **# of patients**
  - Individual price
  - Individual price
  - Individual price

- **# of patients**
  - reimbursement
  - untreated patients
  - saving

- **Price negotiation**
  - Basic saving
  - Possible over expense
  - Possible re-export

- **Possible over expense**

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EURORDIS
Rare Diseases Europe
The concepts of “price” and “cost” seem to differ in some countries. This makes it difficult to perform comparisons between countries. There is probably more heterogeneity in costs than in prices.

A European Reference Price may avoid re-export, but a large discrepancy between official prices and actual costs expose the smaller (weaker) countries to be the only ones to pay the “full price”
Fact Findings

• For nearly 1 out of 10 patients in EU, a developed OD is officially unavailable.

• By adding the ODs in the “grey area” – where information on availability is difficult to find –, the lack of availability goes up over 30%.

• Introduction in the different EU markets is not just a question of time: after the first 2 years, the availability of OMPs desperately does not increase anymore across EU.

• Rare Cancers represent 38% of authorized ODs and 56% of patients potentially treated with an OD in Europe today.
Conclusions

• The rarer the diseases, the least the access

• The smaller the countries, the more neglected the patients are

• The better the national healthcare organisation for rare diseases in the country, the better the access

• The more expertise available and organised across Europe, the better the access
• Special thanks to the 10 National Rare Disease Alliances who participated in and dedicated about 40 to 60 hours each to this collaborative work

• Special thanks to Eurordis – François Faurisson & Fabrizia Bignami & Simone Keita & Anja Helm – for coordinating and dedicating 500 hours overall, to send quality feedback on due time to participating National Alliances