Patient case study: The consequences of diverging and/or inconsistent decisions

2nd Multi-Stakeholder Symposium on Improving Patient Access to Rare Disease Therapies
Elizabeth Vroom  I  Brussels  I  22-23 February 2017
Duchenne Muscular Dystrophy

Progressive

Pediatric

Fatal

Rare

Unmet need
Only 2 drugs approved

2014 EU - EMA Conditional Market Authorisation for Translarna

2016 US - FDA Accelerated Approval for Exondys 51
EMA concluded that Translarna offered therapeutic innovation and relevant benefits for a rare disease with high unmet need.

Granted Conditional MA for ambulant patients 5 years and older.
Translarna

Significant delays in securing patient access to this treatment at a national level
Early patient access

- France
- Italy
- Spain
- Turkey

Commercially available

- Germany

Countries

2014
2015

Countries

<table>
<thead>
<tr>
<th>Early patient access</th>
<th>Commercially available</th>
</tr>
</thead>
<tbody>
<tr>
<td>France</td>
<td>Austria</td>
</tr>
<tr>
<td>Italy</td>
<td>Germany</td>
</tr>
<tr>
<td>Spain</td>
<td>Denmark</td>
</tr>
<tr>
<td>Turkey</td>
<td>Norway</td>
</tr>
<tr>
<td>Greece</td>
<td>Czech Republic</td>
</tr>
<tr>
<td>Portugal</td>
<td>Hungary</td>
</tr>
</tbody>
</table>
Countries

Early patient access

- France
- Hungary
- Spain
- Turkey
- Greece
- Portugal

Commercially available

- Austria
- Germany
- Denmark
- Norway
- Czech Republic
- Slovakia
- England
- Scotland
- Wales
- Northern Ireland
- Sweden
- Cyprus
- Italy
- Cyprus
- Romania
- England

2017
May 2015, Germany's Federal Joint Committee (G-BA), the highest decision-making body in German healthcare, granted Translarna an Early Benefit Assessment rating of 3, which signifies Translarna provides a quantifiable added benefit to patients.

Reimbursement negotiations between PTC and GKV-SV (Germany’s statutory health insurance provider) failed. February 2016 PTC announced it would consider delisting Translarna from the German pharmacy ordering system.
Translarna I UK

NICE (2016): Managed Access Agreement (MAA) with NHS England. The MAA for Translarna allows further efficacy data to be collected over a five-year period, after which time NICE guidance will be reviewed again.

Wales, Northern Ireland and Scotland followed
Translarna I NL

Zorg Instituut Nederland (ZIN) has not yet started to review the dossier. ZIN wants more data before even allowing the company to submit the file.
‘Translarna has changed the course of the disease completely. He started at the age of 9yrs. During that period my son was able to skate and skeeler. After a 2 yr break he started again in 2010. He is turning 18 this october and is still able to walk and his muscle strength has been keeping up as well.’
Patients and clinicians positive
Safe drug, easy to take
Small number of patients, low budget impact
Conclusion

Inequality of speed of access across the EU post EMA approval

Drugs unavailable for patients who need it the most

Inequality of procedures across the EU

Loss of information
Thank you!