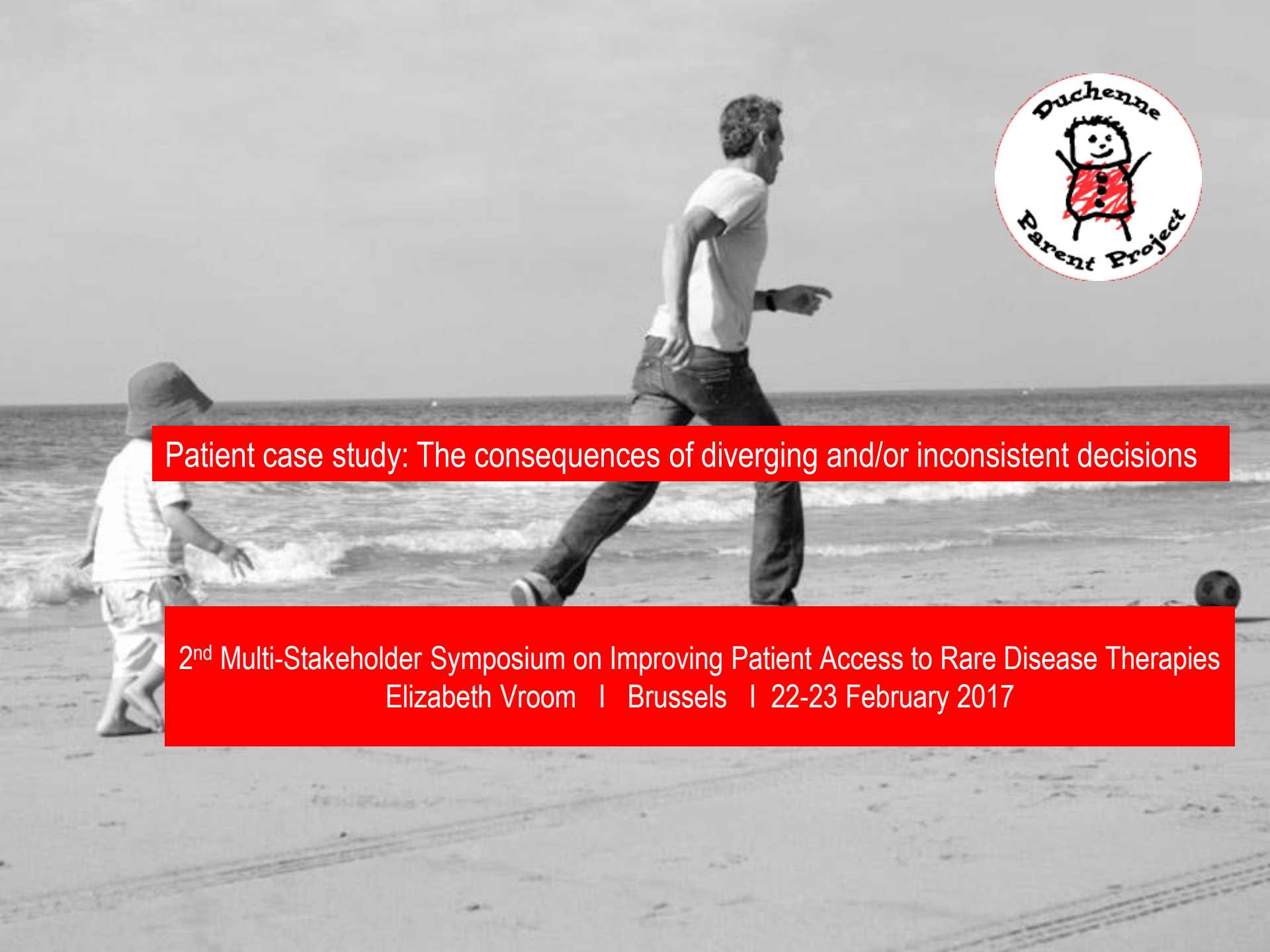
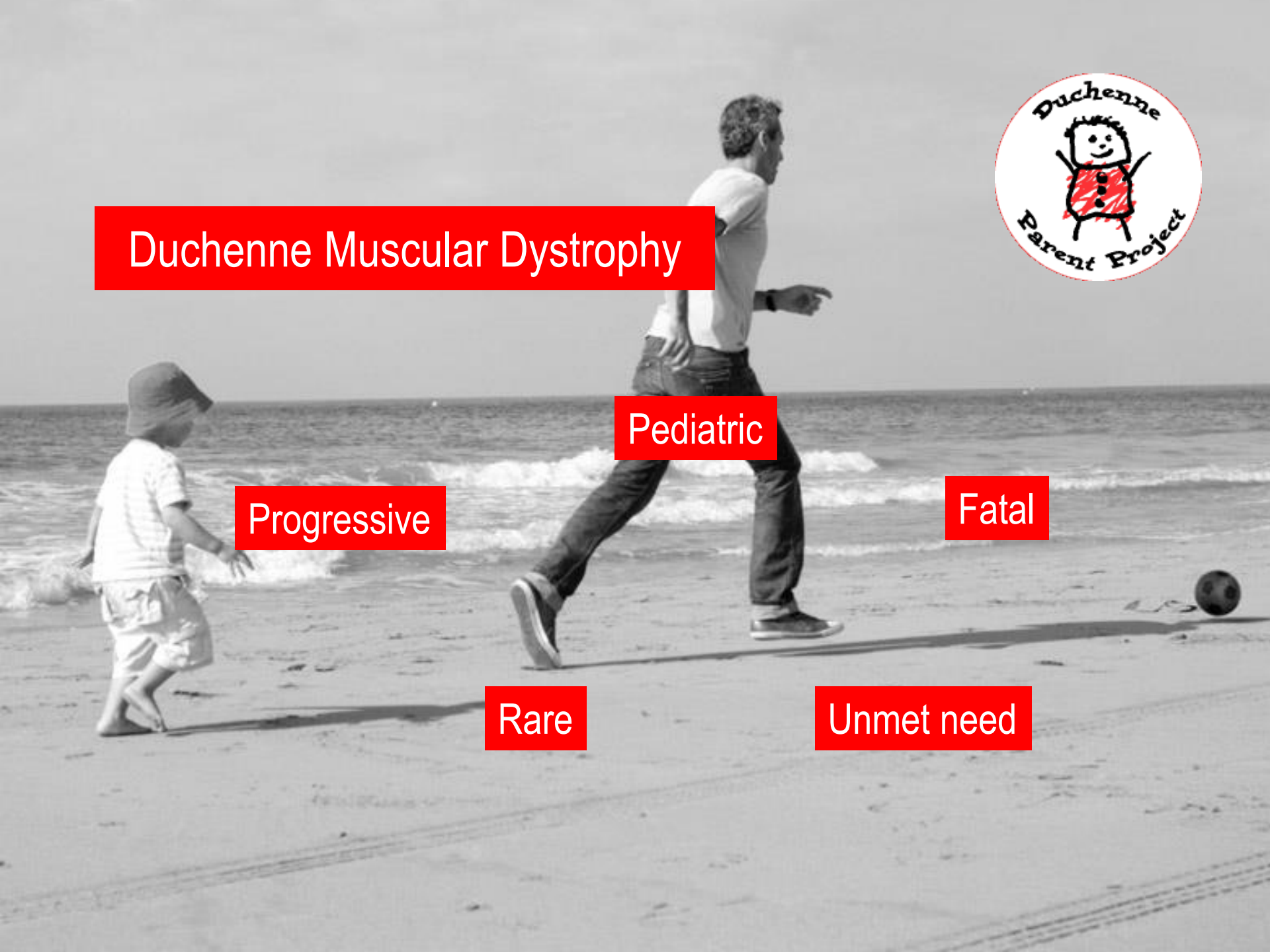


Patient case study: The consequences of diverging and/or inconsistent decisions

2nd Multi-Stakeholder Symposium on Improving Patient Access to Rare Disease Therapies

Elizabeth Vroom I Brussels I 22-23 February 2017


Duchenne Muscular Dystrophy
Pediatric
Progressive
Fatal
Rare
Unmet need
Duchenne
Parent Project


Only 2 drugs approved
2014 EU - EMA Conditional Market Authorisation for Translarna
2016 US - FDA Accelerated Approval for Exondys 51
Duchenne
Parent Project

Translarna

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
Duchenne Parent Project

Countries

Early patient access

France

Italy

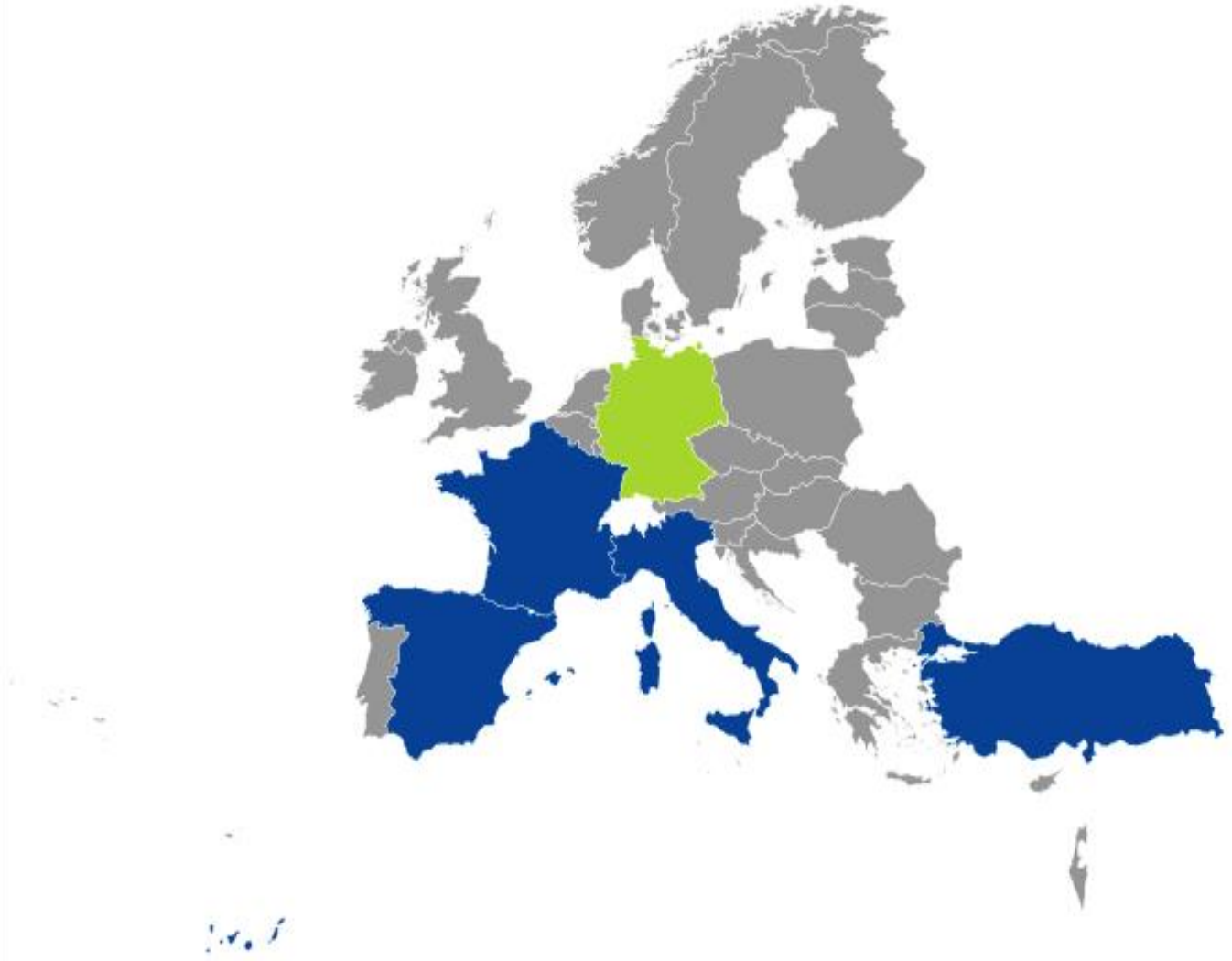
Spain

Turkey

Commercially available

Germany

2014



Countries

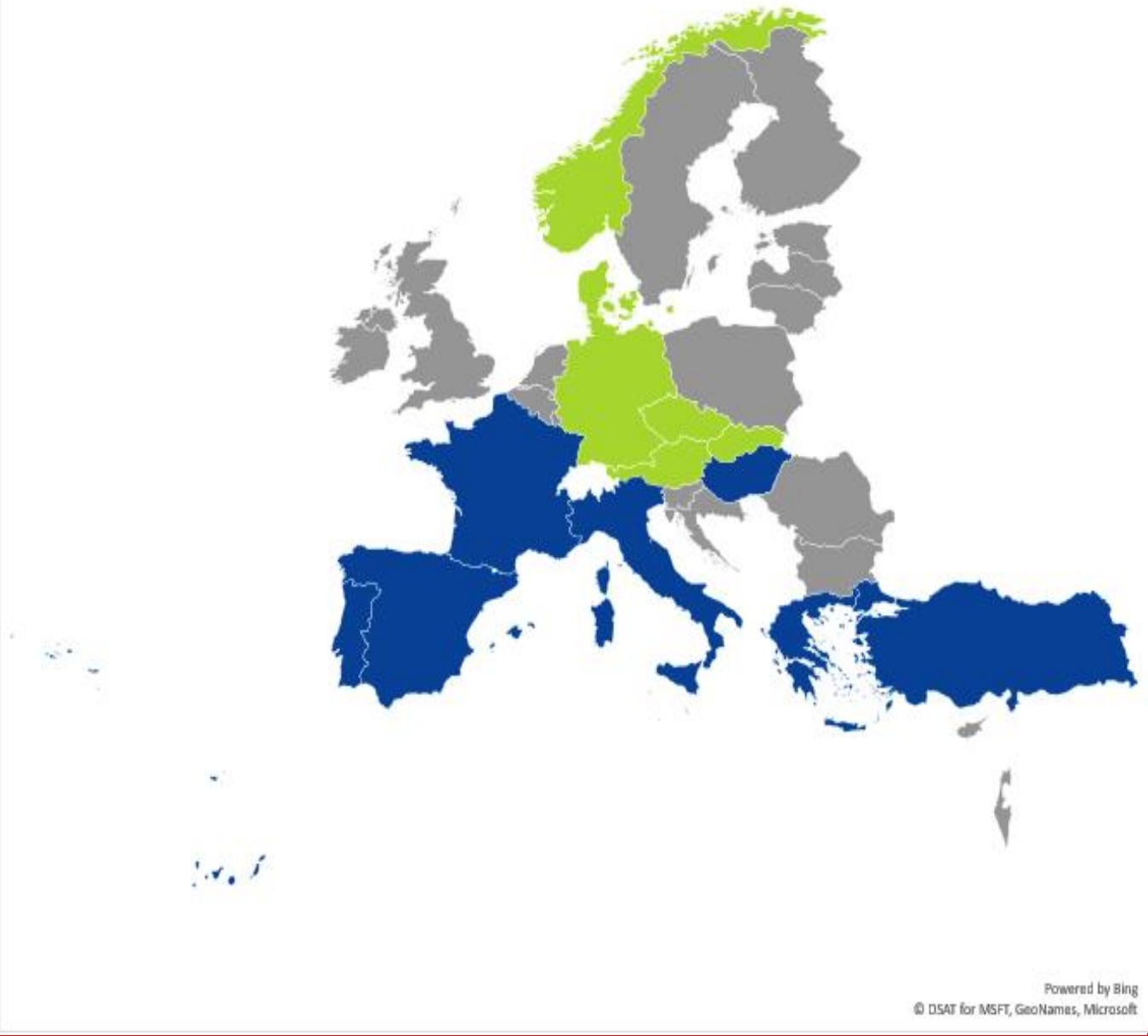
Early patient access

- France
- Italy
- Spain
- Turkey
- Greece
- Portugal

Commercially available

- Austria
- Germany
- Denmark
- Norway
- Czech Republic
- Hungary

2015



Countries

2016

Early patient access

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

Commercially available

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

Countries

2017

Early patient access

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

Commercially available

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

Translarna I Germany

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.

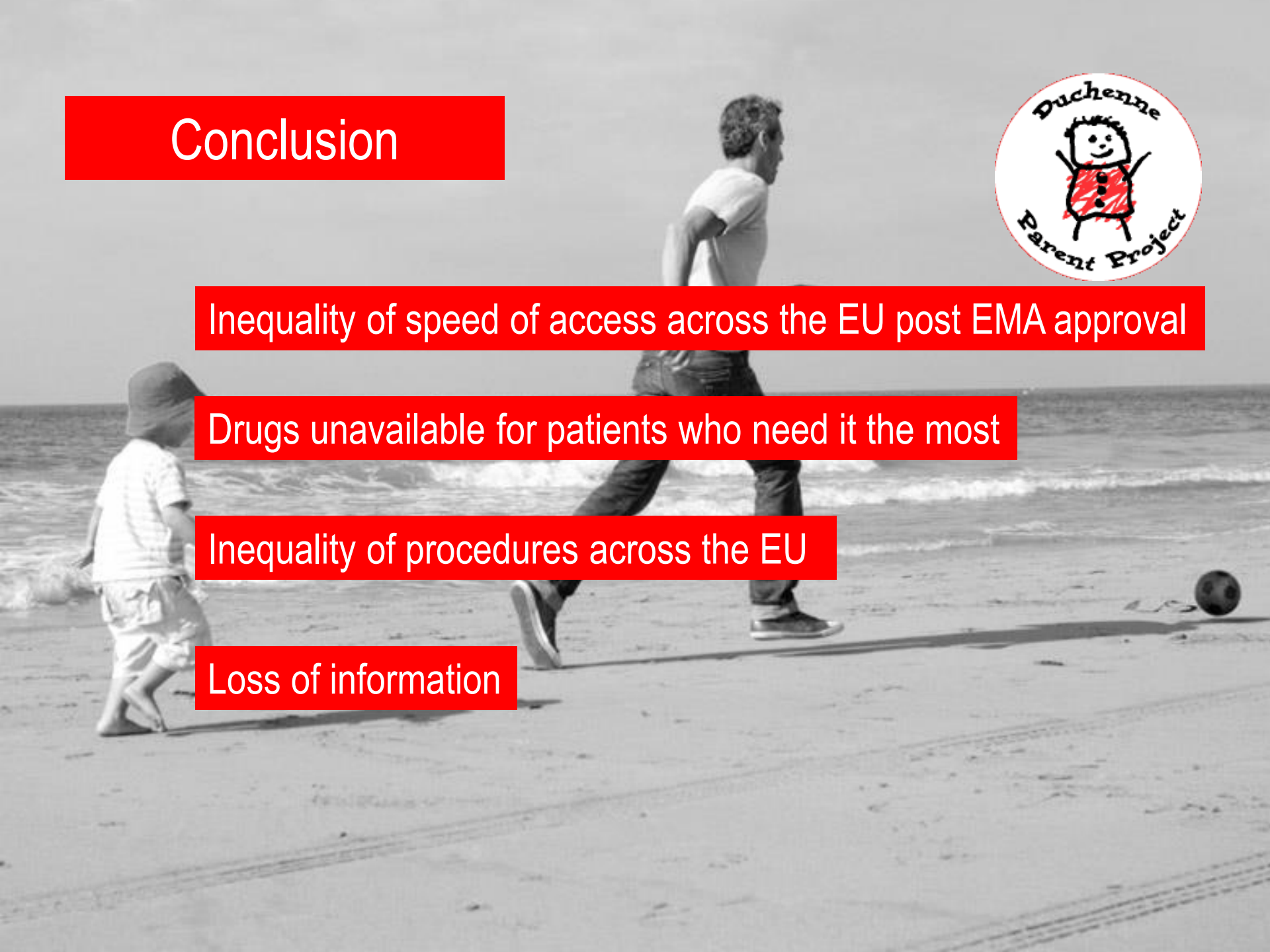
Wales, Northern Ireland and Scotland followed

Translarna I NL

Zorg Instituut Nederland (ZIN) has not yest 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 en clinicians positive
Safe drug, easy to take
Small number of patients, low budget impact
Duchenne
Parent Project



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!