

We are committed to developing treatments for rare diseases with a life-threatening unmet medical need

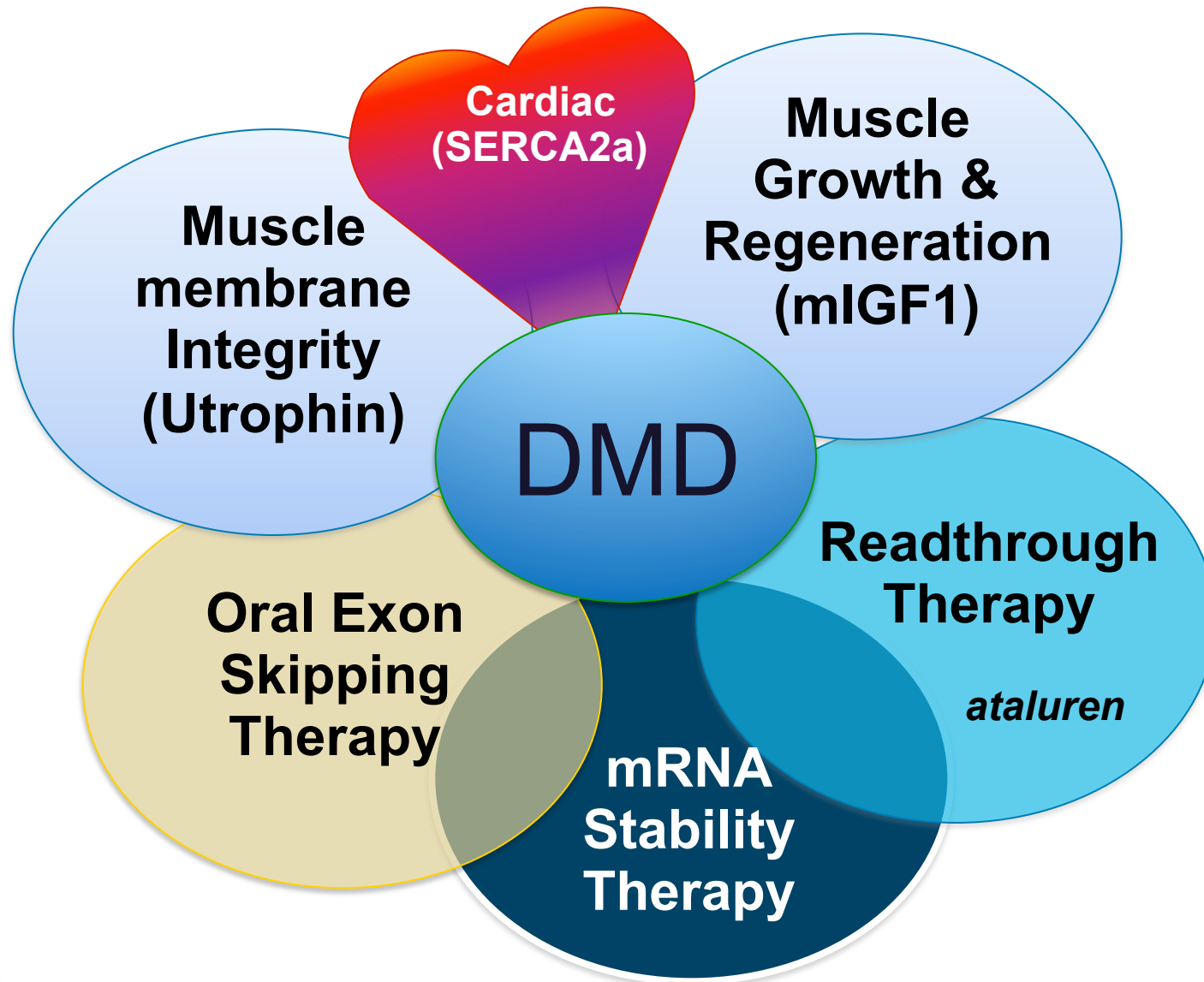
Our mission is to leverage our knowledge of RNA biology to bring novel therapeutics to patients affected by rare and neglected disorders



Established 1998; Global HQ in South Plainfield, NJ, US

We are passionate about making a difference for the DMD community.

We are committed to the DMD community



Our journey together

Discovery

Clinical
Development

Preclinical

Regulatory
approval




Patients
Treated




Our
focus
now

EMA approval expands the possibilities for all DMD drugs

An agency of the European Union

 EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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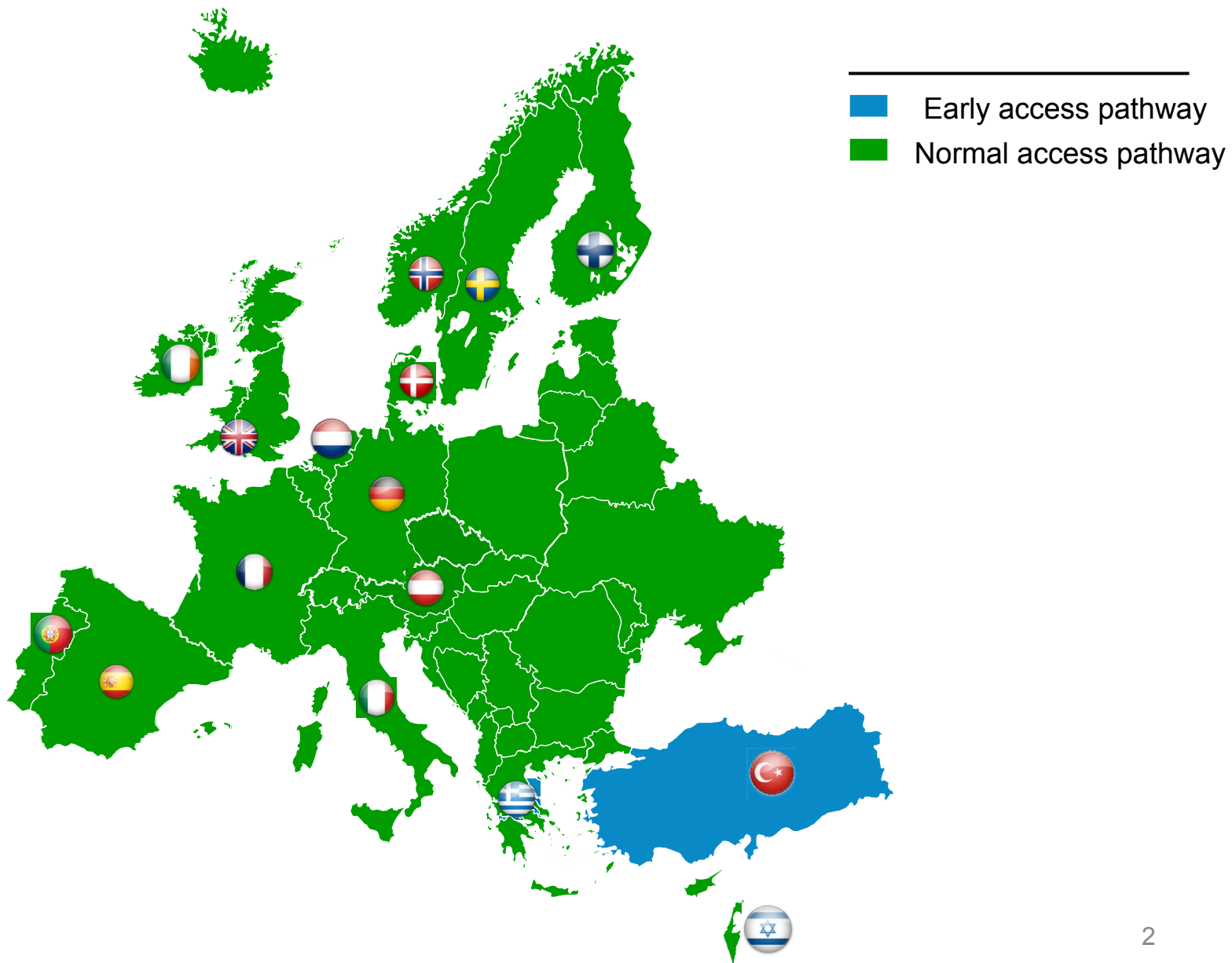
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 **AUTHORISED**
This medicine is approved for use in the European Union

News

- Small and medium-sized enterprises: driving innovation in medicines (29/09/2014)
- European Medicines Agency recommends 39 medicines for human use for marketing authorisation in first half of 2014 (18/07/2014)

Ataluren availability is expanding rapidly for a small company like PTC



Global patient access programs underway



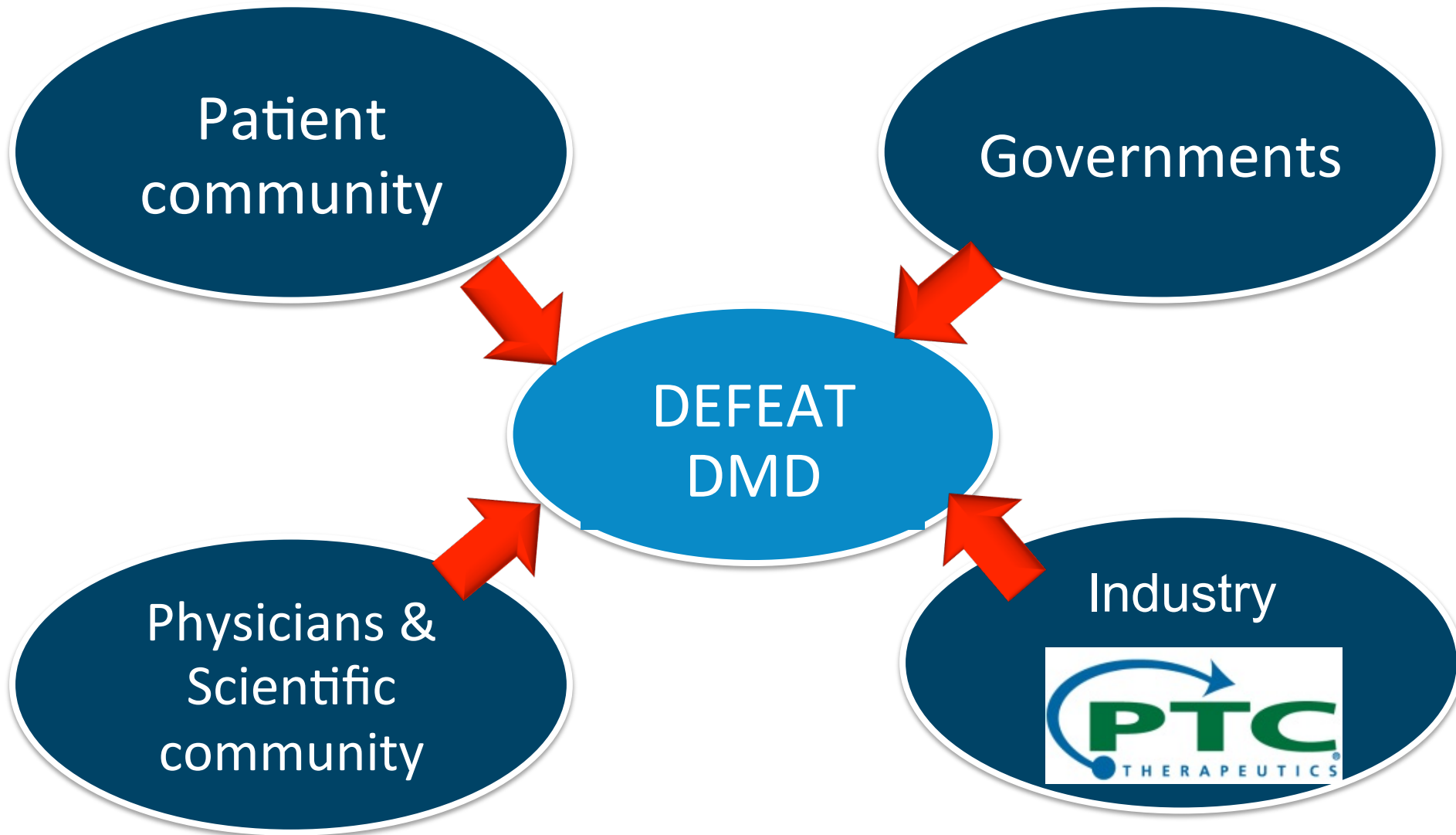
Our goal is to bring ataluren to nmDMD patients around the world as fast as possible

The journey has just begun...

- ✓ Additional trials (e.g. Pediatric Investigation)
- ✓ Establishing registry to learn more about the disease
- ✓ Safety monitoring
- ✓ Support improving disease awareness and diagnosis
- ✓ Learn from DMD community how to continue to improve

There is no time to waste!

Collaborations is the key of success



Medical Information

24 h/7day – Service for The Netherlands:

- Email: medinfo@ptcbio.com
- Phone: (+31) 0207 095138



Thank you