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advancing diagnosis, care and treatment for people with neuromuscular diseases around the world



Tools and resources for the neuromuscular community

TREAT-NMD

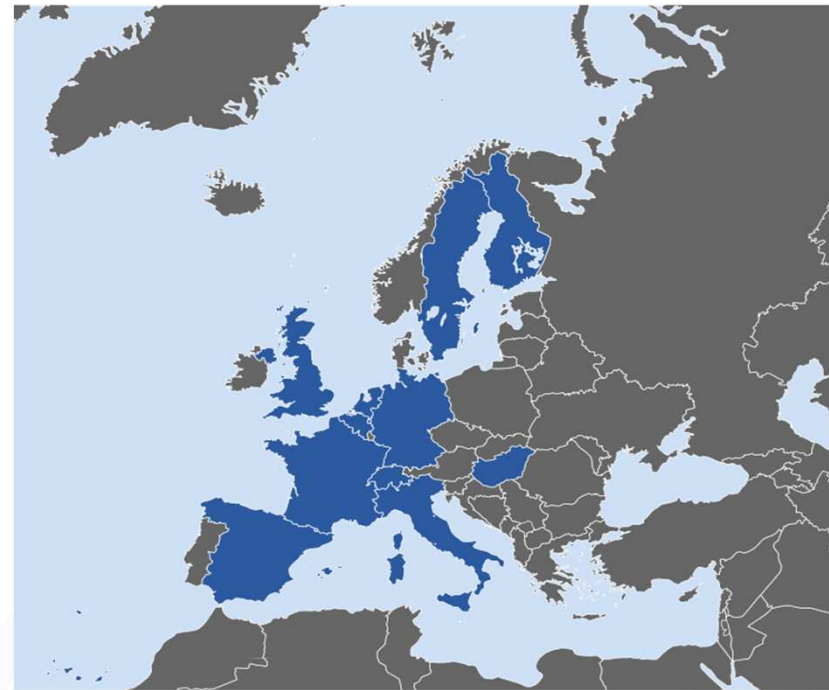
Translational Research in Europe – Assessment and Treatment of Neuromuscular Diseases

What is TREAT-NMD?

1. An EU funded network of excellence

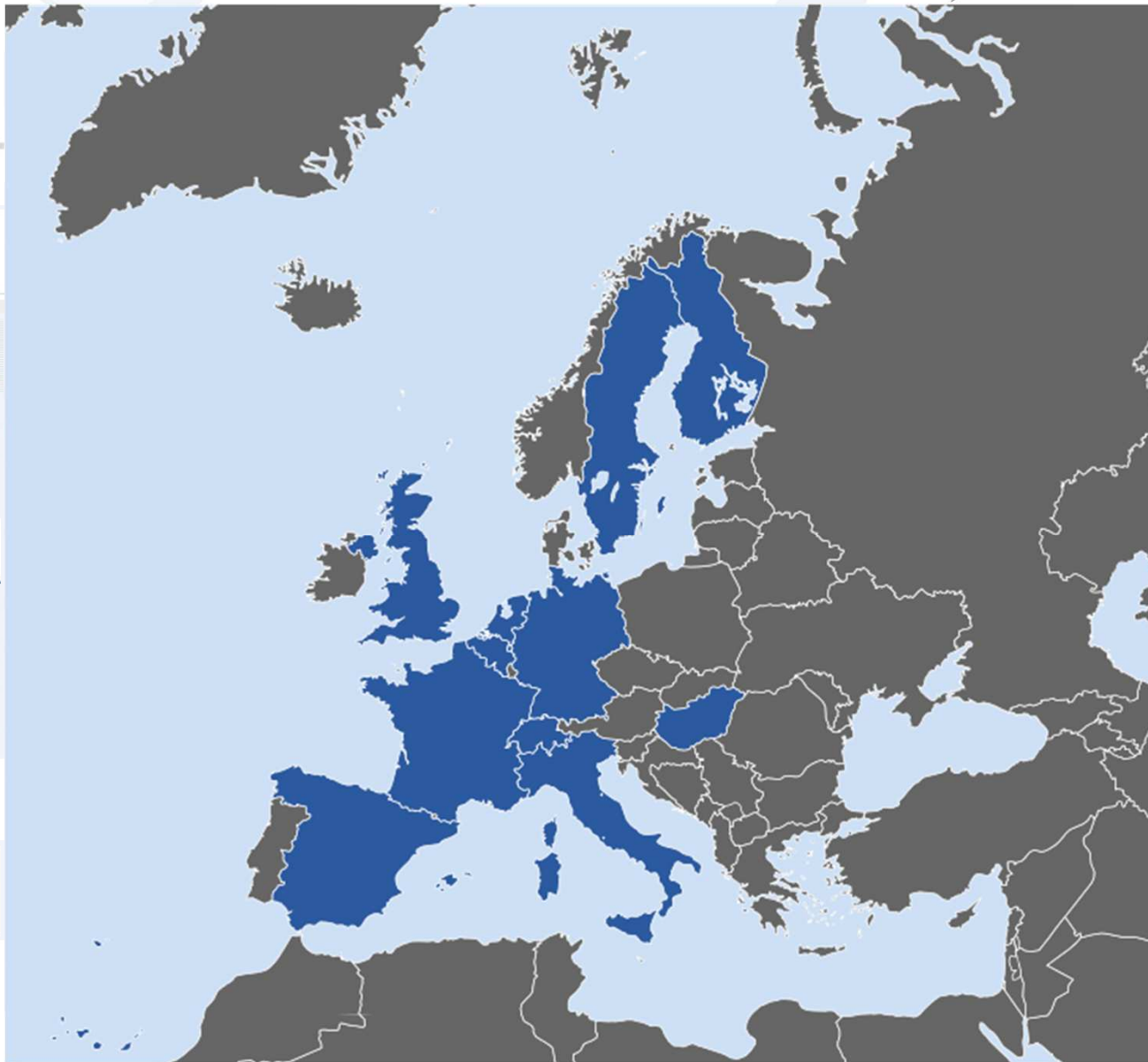
- A 10m Euro project
- Successful lobbying to EU for funding call
- 22 partners
- Specific aims, governance, rules and regulations
- 164 Deliverables, milestones, >30 workshops

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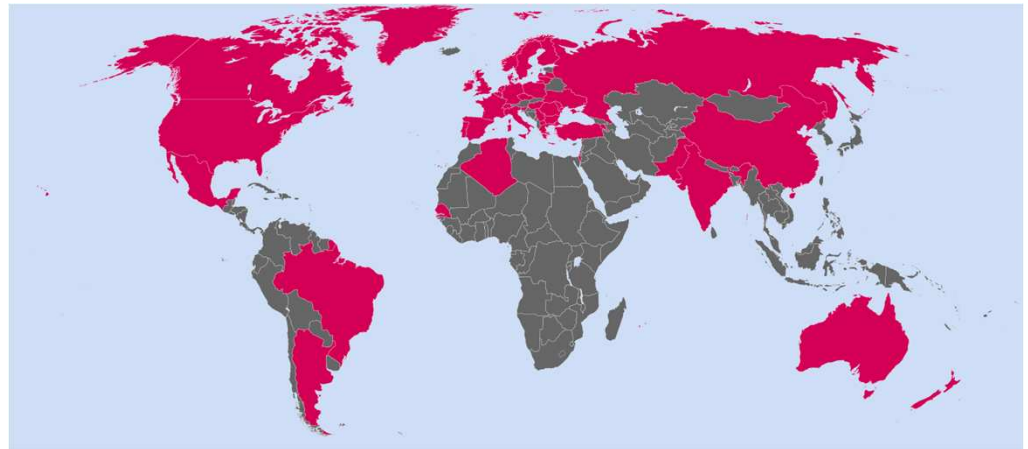
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TREAT-NMD Partners



What is TREAT-NMD?

1. An EU funded network of excellence
2. A huge opportunity!
 - The EU money has been able to initiate a large number of exciting projects
 - These projects involve many interested people from around the world
 - We now have some exciting resources that can be used to help get therapies to patients



.....Parent Project Association, Association Muscular Dystrophy, CINRG, SMA Trust, University Hospital Basel, University of Utah, Washington University in St Louis, UPA! Cura Duchenne, University of Bucharest, Virgen Del Rocio University Hospital, University of Ulm, Jennifer Trust for SMA, Muscular Dystrophy Campaign, Asociace Muskulárních Dystrofií v ČR, Medical University of Warsaw, University Children's Hospital, SMA Trust, Kolpingova rodina Smecno, SMA Deutschland, Myotonic Dystrophy Foundation, Wake Forest University, Australian Neuromuscular Research Institute, Children With SMA, Cure CMD, Centre Hospitalier Régional de la Réunion, Clinic for Child Neurology, Martin House Hospice, DuchenneConnect, University of Glasgow, Hospital Pediatria J.P. Garrahan.....

Outreach: newsletter and website



29 January 20

TREAT-NMD newsletter no. 1

[view this newsletter online](#) | [full newsletter](#)

welcome

Welcome to the latest newsletter from TREAT-NMD, which includes details of a great opportunity to study and work in neuromuscular translational medicine in Newcastle. A new article addressing the realities and myths surrounding stem cell 'therapies' is now available on the TREAT-NMD website, and this week we also highlight the Institut de Myologie's 13th annual summer school in Paris and two interesting opportunities to develop skills in trial design for small patient cohorts.

As always, if you have anything you wish to include in our next newsletter, please contact the coordination office and we will be happy to help.

Best wishes from Katie, Volker, Hanns, Steve, Emma, Rachel, Brigitta, Samantha, Karen and Michael, the Newcastle TREAT-NMD team.



"Stem Cell Tourism" - Hope versus Hype: an online guide

TREAT-NMD is aware that new scientific discoveries and innovations can lead to raised expectations and enthusiasm and this may in turn lead to hype, which can be seen as increased publicity and unfounded claims. In recent years, "stem cell tourism" has become more and more popular with patients who have NMDs. Unregulated clinics all over the world are charging substantial amounts of money for stem cell "therapies", which have not been shown to be effective, or more importantly safe. To help inform patients and the public,

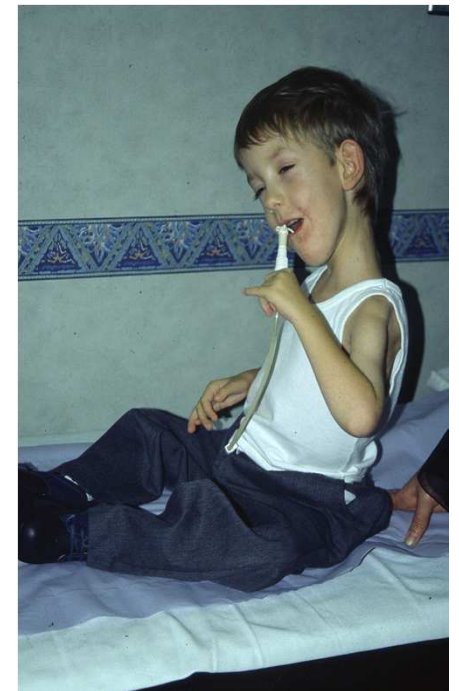
Clinical training opportunity in neuromuscular translational medicine at Newcastle University

A new opportunity has arisen for a doctor wishing to develop a specialisation in neuromuscular medicine. Based in a UK Centre of Excellence at Newcastle University this programme has been developed around the partnership between academic and industrial partners, namely Newcastle University and PTC Therapeutics. Together they offer a unique opportunity for translational medicine training in the UK.

>4980 visits in July, >1300 pdf downloads

What is TREAT-NMD?

1. An EU funded network of excellence
2. A huge opportunity!
3. All about patients with inherited NMD

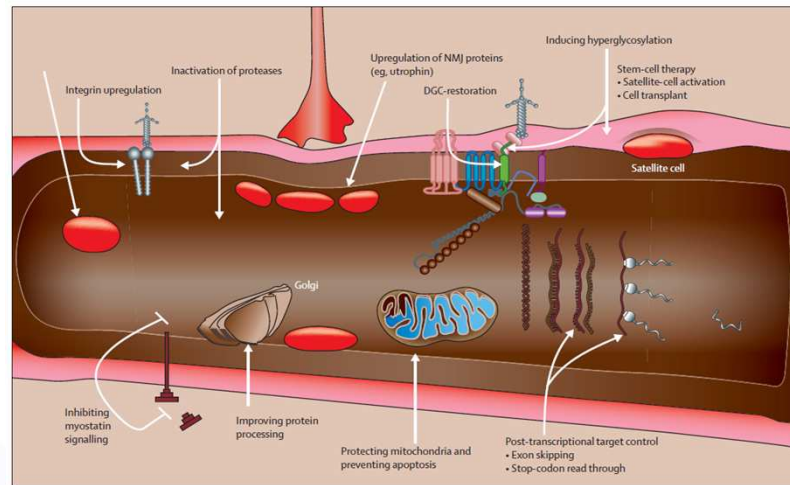


Therapy delivery to patients is the goal

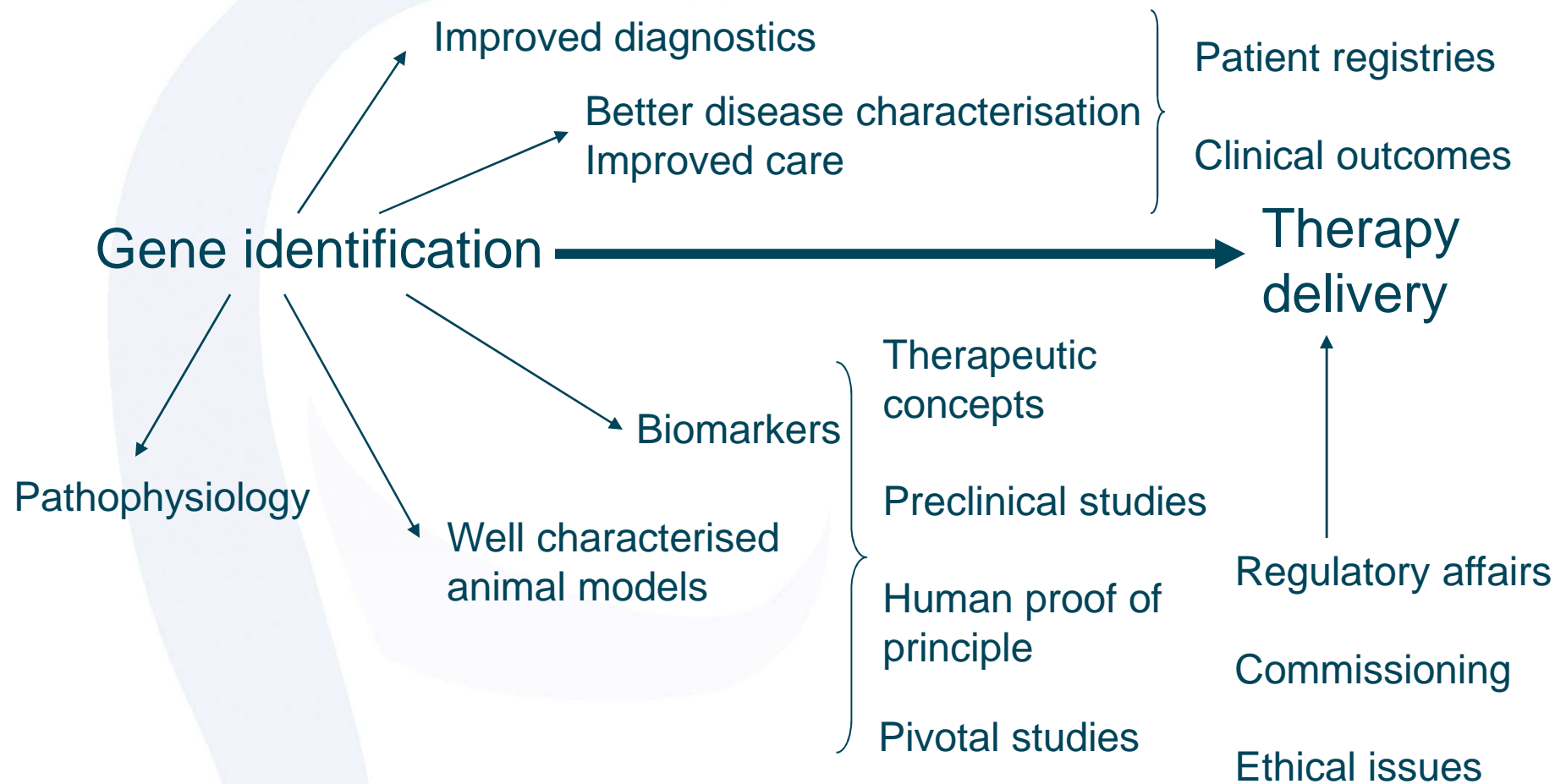
Gene identification

Dystrophin
Sarcoglycans
Dysferlin
Calpain 3
Lamin A/C
FKRP
Laminin alpha 2
Collagen VI
TRIM32
POMT1
SEPN1
ANO5
LARGE
Myotilin
Titin..... Etc.....

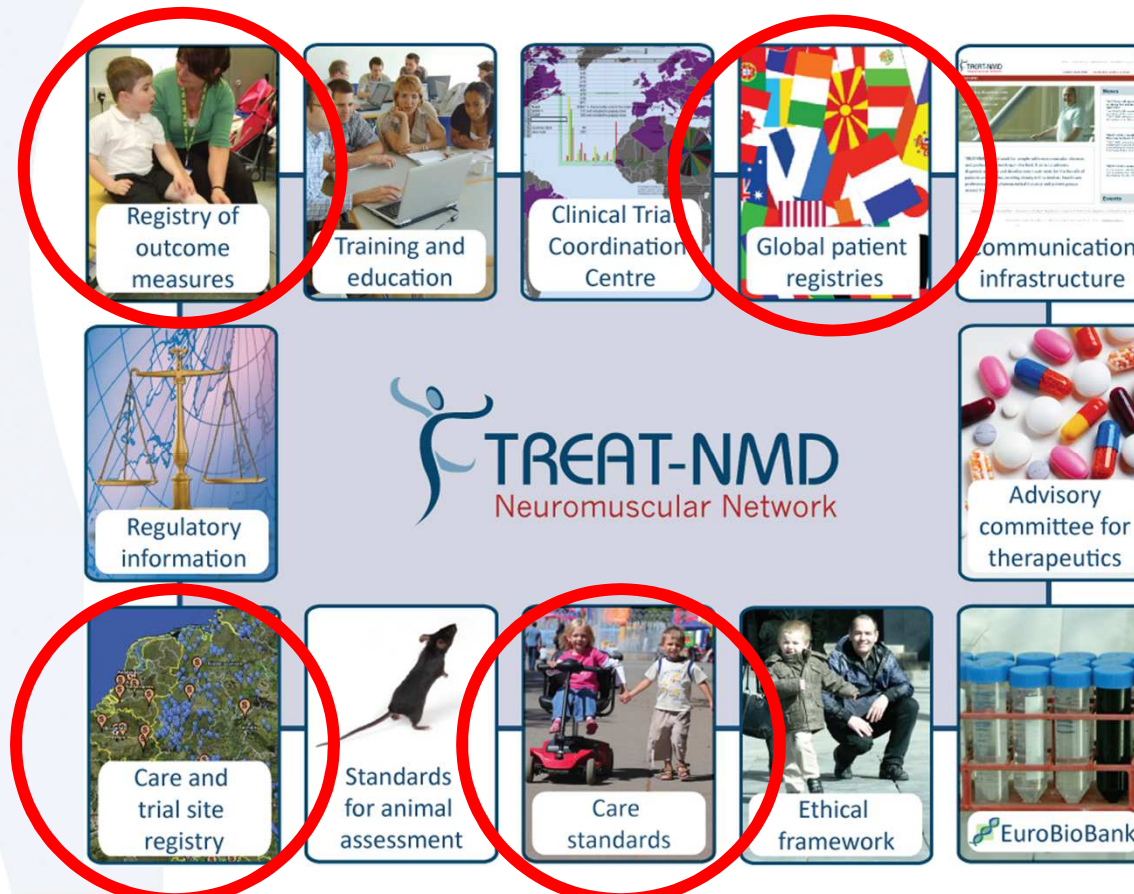
Therapy
delivery



9 | The route to therapy



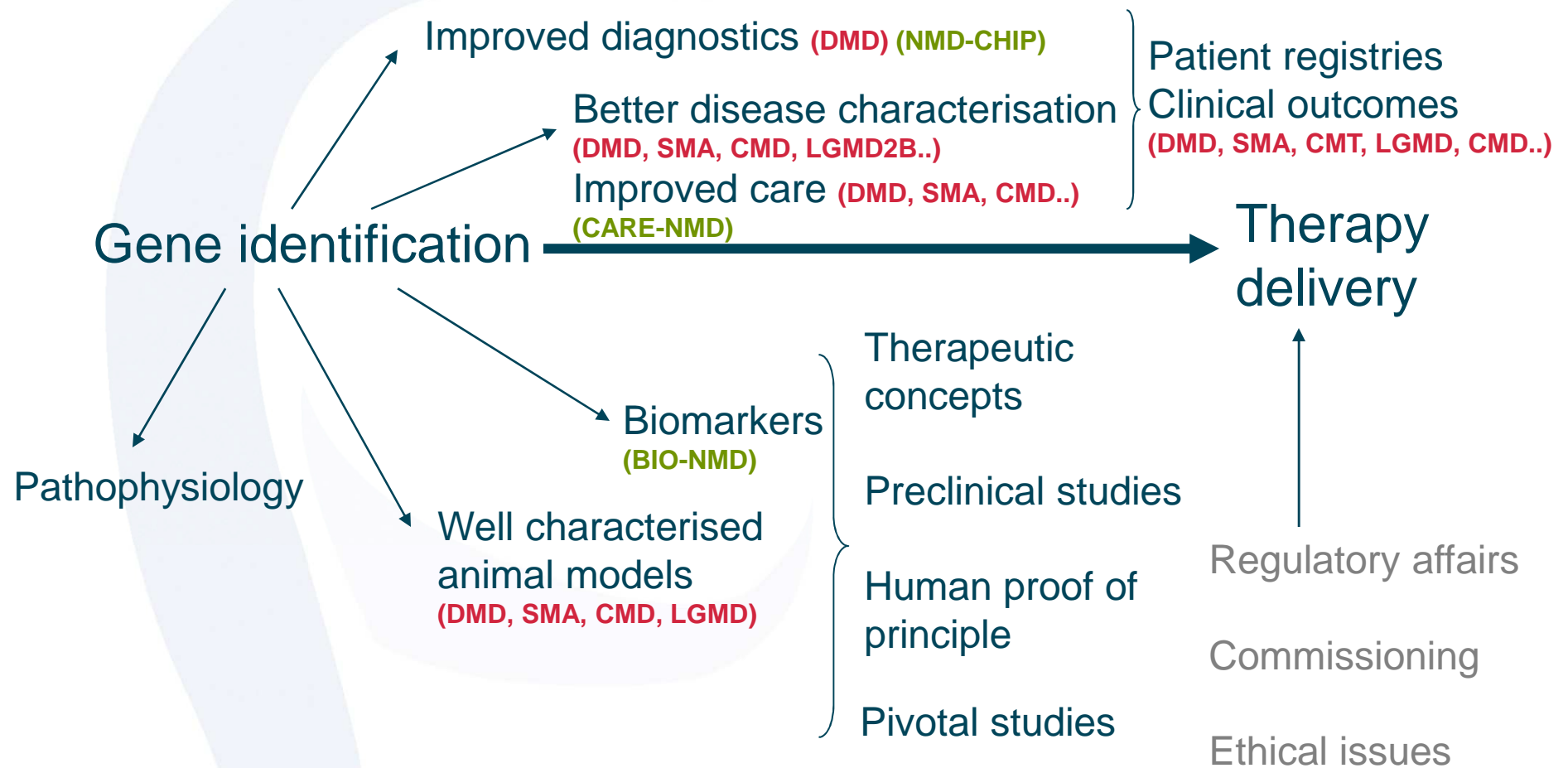
Network tools and resources have been set up aiming to accelerate therapy delivery for NMD

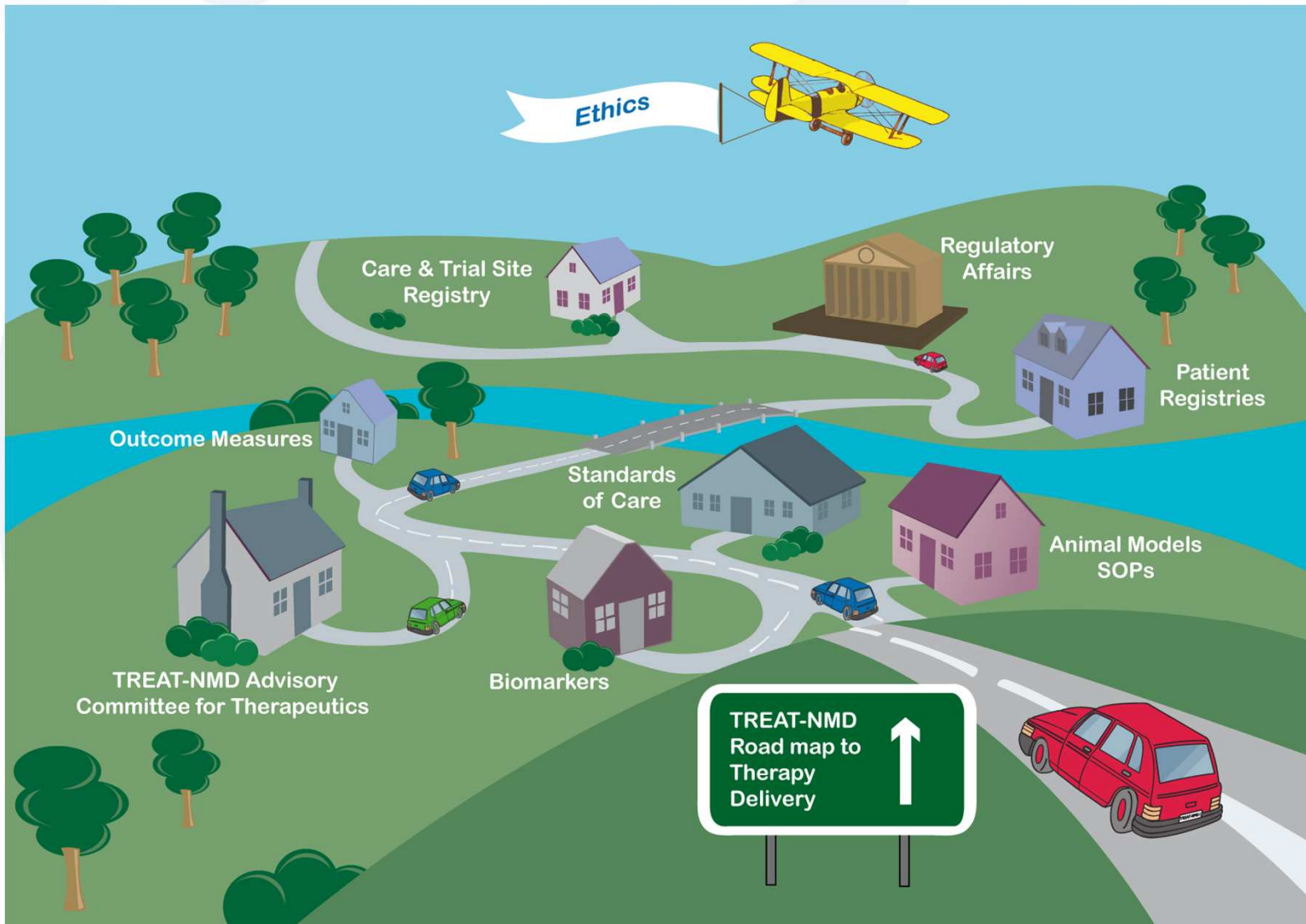


Network tools and resources

- Established via expert consensus
 - Involving TREAT-NMD partners and many other collaborators
- For use by the community
 - And other interested parties
- For the benefit of patients
- A platform to support care, research and therapy delivery

12 | The route to therapy





Industry use of TREAT-NMD tools

Current collaborations include:

Accelaron
AVI Biopharma
Biomarin
Debiopharm
Genzyme
Prosensa/ GSK
PTC Therapeutics
Santhera
Trophos.....

Current activities include:

Preclinical and biochemical OM
consensus
Patient and trial site enquiries
Patient recruitment
SAB meetings
OM selection and training
CRO support
Preparing funding applications
TACT appraisal

Examples of international academic collaborations

- NMD-Chip (Diagnostics)
- BIO-NMD (Biomarker identification)
- CARE-NMD (Implementation of standards of care)
- FOR-DMD (NIH steroid trial in DMD)
- Jain Foundation Dysferlinopathy natural history project
- CMT global TREAT-NMD registry (NIH)
- ICC SMA Rasch study of outcomes in SMA



Regulatory interactions

- EMEA meeting (SMA outcome measures) 2008
- EMA meeting (DMD AON therapies) 2009
- Support for NIH funded FDA meeting (AON in NMD) 2010

New national initiatives linked to TREAT-NMD

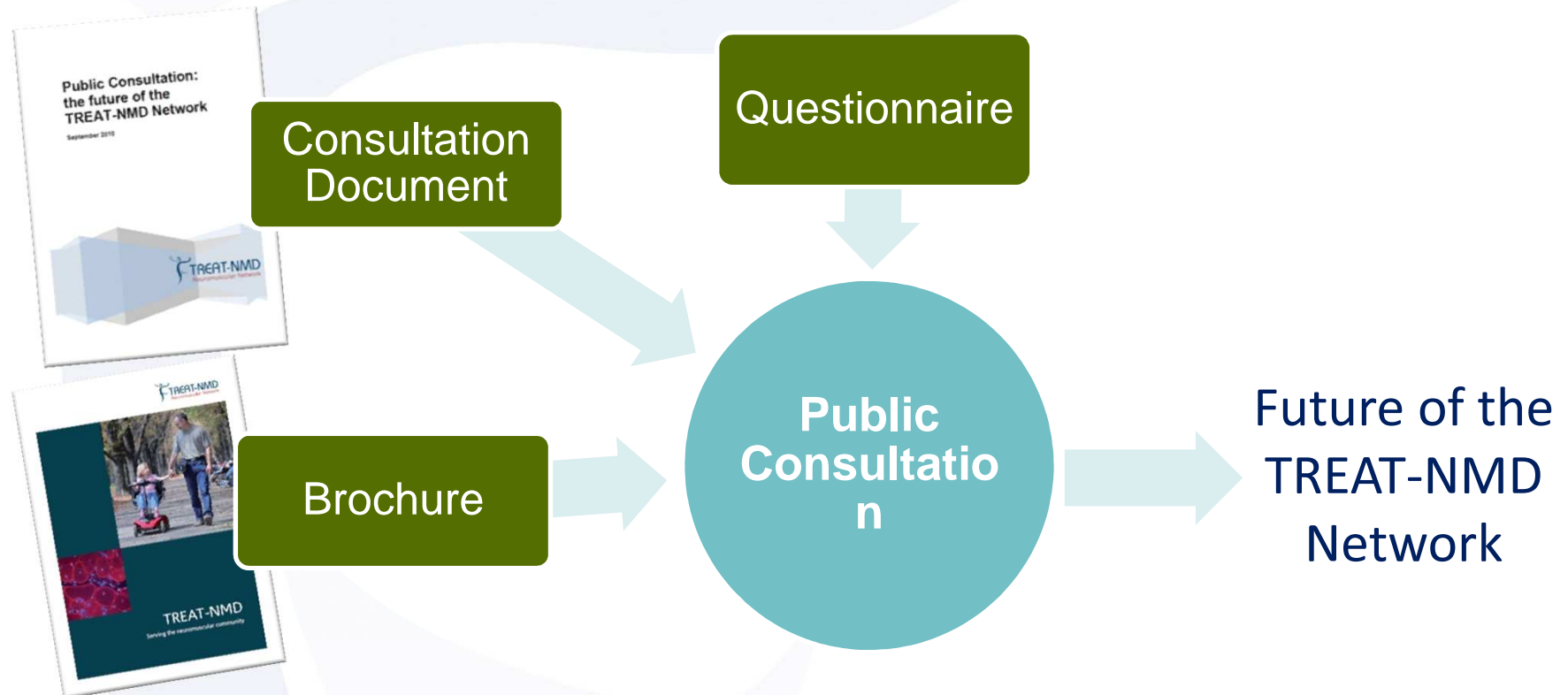
- Registries (40+ countries involved to date)
- Australasian neuromuscular network
- Brazilian neuromuscular network
- Numerous existing national networks “implementing” resources locally and contributing to overall project
 - A two way street



The future

- EU NoE funding ends at the end of 2011
- Sustainability discussions have taken place throughout the lifetime of the network
- Partners and others wish to continue to develop and maintain tools and resources for inherited NMD via a continued and extended consortium
- Funding streams will include academia, government, patient organisations, industry
 - Shared ownership, shared responsibilities
- Stakeholder consultation launched September 2010

Public Consultation



www.treat-nmd.eu/consultation-document

Closing date –1st October

What are the determinants of success in rare diseases?

- Strong patient organisations driving (and funding) research
- Patient registries
- Strong partnerships
 - Academic
 - Advocacy
 - Industry
 - Regulatory
- TREAT-NMD offers the platform for these partnerships
- To facilitate shared goals



Global partners

