



CARE-NMD: overview of the field and where next?

Kate Bushby
Newcastle University

CARE NMD International Meeting
Budapest April 18th 2013 #CARENMD

Changing dimensions and optimism

- Use of current management strategies have changed natural history
 - Clinical care guidelines developed and widely disseminated
- Multiple ongoing clinical trials
- Disease modifying therapies showing promise, many others in development
- In the best centres, patients with DMD have real opportunities for holistic support and access to trials

The real world situation with Duchenne muscular dystrophy?

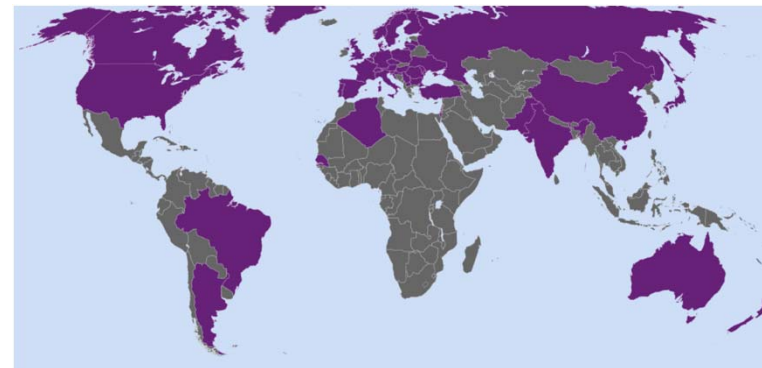


The rationale for CARE NMD

- Cystic fibrosis model for monitoring care via patient registries has had great utility in raising standards
- International collaboration in generation of care standards offered a platform for comparison of care
- Dissemination activities including multiple translations allowed maximum outreach
- What is the experience of care and barriers to its delivery?

Building on tools and resources to study care

- TREAT-NMD (FP6 Network of Excellence)
- CARE NMD (Health Programme funding)
- Patient and care site registries
- Snapshot of the current situation with care
- Opportunity to collaborate worldwide for greater comparative data



Over the course of the meeting:

- Comparison of care across different EU countries and extension outside Europe
- The largest collection of QOL data in DMD
- Address areas where the care considerations require extension and updating
- Take stock of where the upcoming therapies may contribute to the process of care
- Look at prospects for building DMD care and therapy delivery into policy

DMD: a microcosm of the rare disease challenge

- EU definition of RD: conditions affecting less than 5 in 10,000 people
- There are estimated to be 6,000- 8,000 different RD
- This includes rare cancers, rare acquired diseases as well as inherited diseases
- Frequently strong and active patient organisations
- Speaking of them as a whole presents a challenge, but adoption of a common RD strategy has been a strong and effective measure at the EU

How do these challenges look on the ground?

- Delayed diagnosis
- Lack of visibility and priority in health care planning
- Lack of access to specialised health and social care
- Few definitive treatments available and access to new drugs highly variable



EU RD policies addressing challenges

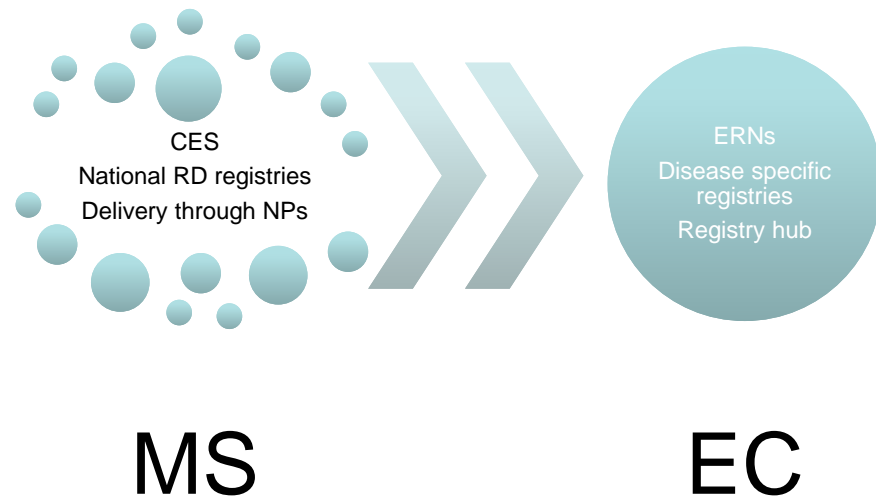
- Focus on rarity of patients
 - Few health and social care providers have knowledge of the conditions
 - Relevant experts are also rare!
 - Lack of appropriate coding leads to even greater invisibility
 - Evidence based guidelines for care are often lacking
 - Less incentive for companies to develop drugs as market may be small (or invisible)
- Grant funding to date has placed DMD strongly amongst other RD: so we should be well placed to benefit from further developments in policy

Policy areas of interest

- Requirement for MS to have a National Plan for RD in place by end 2013
 - NP should address centres of expertise, registries, coding issues, research funding....
- Formation of EU Committee of Experts on RD (EUCERD) www.eucerd.eu
- Support for European Reference Networks (including for RD) within the Cross Border Healthcare Directive

Ideal model of delivery of RD care

- National plans for RD implemented and funded
- Centres of expertise deliver holistic MD care within MS
 - Models will vary, but offer access to all RD patients
- ERNs oversee healthcare pathways and collection of condition specific data
- Centralised registry hub allows data comparisons



Currently: Steady progress towards RD national plans

- BUT
 - Economic climate is not favourable for major investment: many MS struggling to fund RD services as envisaged
 - Cross talk not always optimal with individual disease efforts
 - Attention needs to be given to the “invisible” costs of getting it wrong.....
 - Invisible to the authorities, very highly visible to the patient
- CARE NMD findings are crucial to inform the overall debate and ensure that NMD are central in the new systems for RD care

Thank you!

- Project partners
- Patients and families
- Further information: info@care-nmd.eu
- Twitter: @bushbykate #carenmd

