What framework is needed to collaborate with industry?

Reflections by the EGRD industry representatives

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Disclosure of interest

- Industry representative on EUCERD and EGRD
- Member of IRDiRC interdisciplinary scientific committee, small clinical trials and patient reported outcomes groups
- Lysogene employee: an SME biotech developing AAV gene therapy in MPS IIIA and GM1 gangliosidosis
- Central role in development and administration of EPNET, EuroWilson, E-IMD and E-HOD

Key messages

- ERN's should contribute to important advances in the diagnosis and treatment of Rare Diseases.
- Should maximise speed and scale of diffusion of research and innovations
  - Reduce differences across EU countries in quality and outcome of healthcare
- Industry and ERN may share common research objectives; but acknowledge different organisation primary aims
- Clinical research guided by open interactions among stakeholders is key to success
- Particularly exciting for SME (few specialist scientists): opportunity to engage with academia and expand scope and quality of research

Parameters determining complexity of clinical research in rare diseases

- Critically ill patients and no standard of care available
- Patient needs intensive with frequent diagnostic and clinical assessments
- Travel to research centres may be impossible
- Hard to identify sufficient number of patients within given time
- Competing trials in an environment of rarity
- Paediatric population: higher regulatory / ethics hurdles
- Advanced therapy medicinal products: first-in-human clinical studies
- RCTs difficult to conduct in small populations
- Limited knowledge of disease natural history

ERN / industry collaboration priorities in clinical research

- Registries, natural history studies and cohorts
  - Unified collection of phenotypic data
  - Facilitate identification of appropriate clinical endpoints or biomarkers
  - Patients pre-concentrated
  - Support drug development discussions with regulators
- Feasibility studies
- Clinical study patient recruitment: aim to meet recruitment targets more quickly
- Training and education
- Standardisation, quality control...
- Remote monitoring of patients?

A successful example of post marketing safety surveillance PPP

Modular IT platform → clustering of disease groups

- **E-IMD** (start 2011)
- **E-HOD** (start 2013)
- **iNTD** (start 2014)
- **New disease groups**

- **UCDC** (USA)
- **Feasibility analysis** (clinical study)
- **Post marketing safety surveillance**

Post marketing safety surveillance (start 2013)

- Principle of controlled access to other research groups
- Development of specific PASS dataset
- Independent patient consent
- Support required (CRO) for set-up, contracts and IRBs
- Additional work paid according to fair market value

Collaboration with scientific consortia and industry

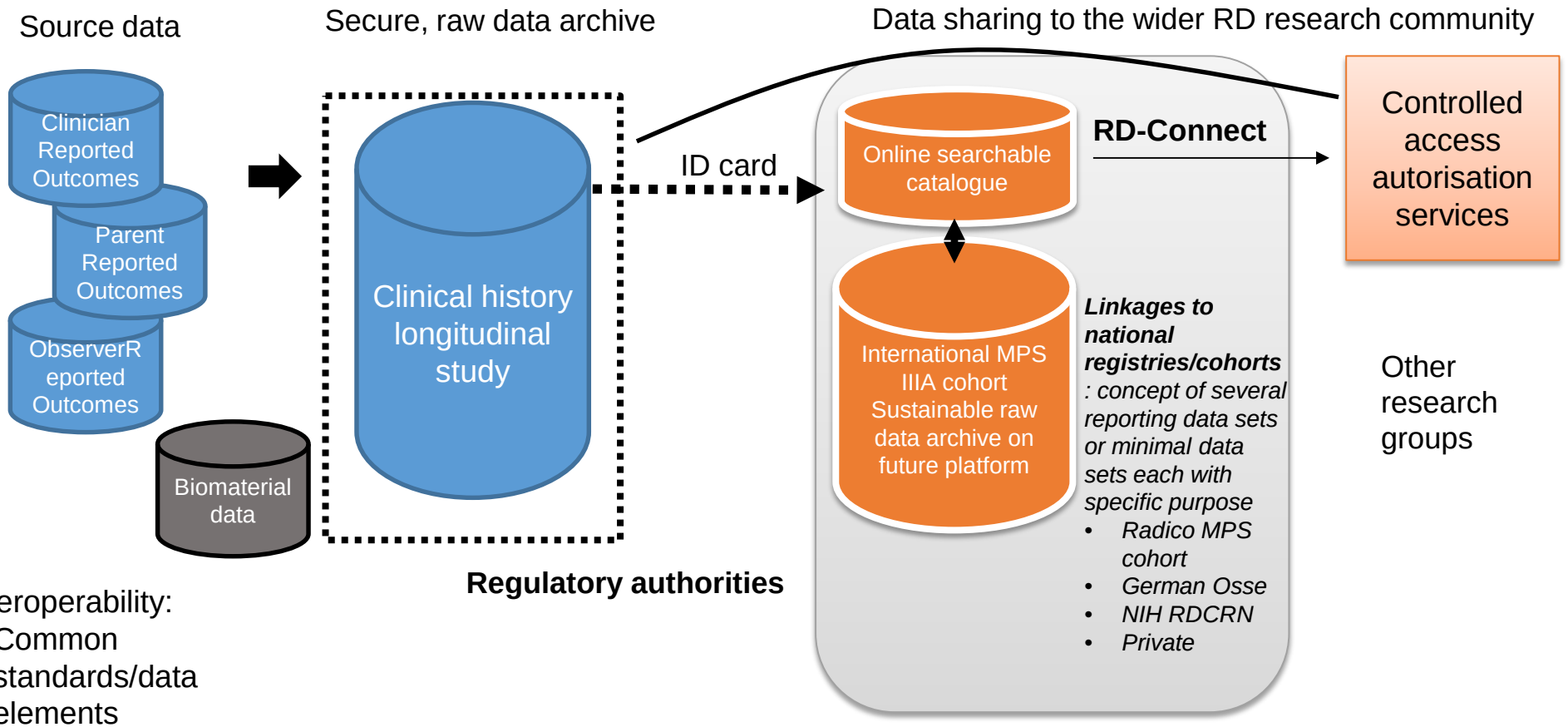
Adapted from presentation Professor Stefan Kölker, University Hospital Heidelberg

A collaborative approach to natural history studies

- To support drug development programmes
- « In rare cases, patients in natural history studies can serve as historical controls » (Anne Pariser, FDA): data from the natural history study and therapeutic trial will need to be comparable
- High quality: accurate, timely, balanced
- Should suit requirements of EMA, FDA and HTAB
- Adequate geographical representation of patients
- ERN's may be the home for collaborative natural history studies or at a minimum ensure harmonisation of data agreed between the experts.

One familys' journey to 2 NH studies

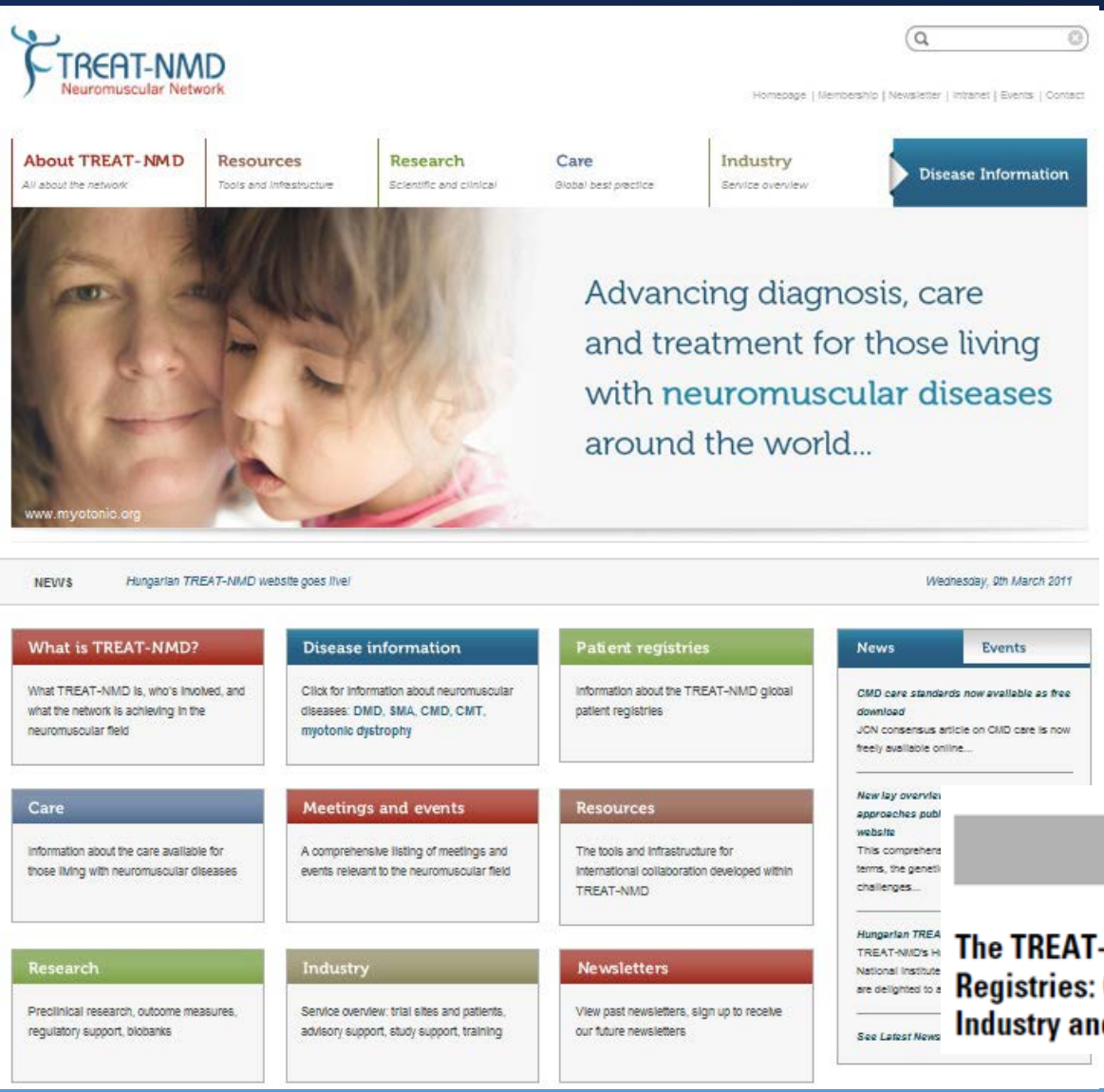
A collaborative approach to natural history studies



Ineroperability:

- Common standards/data elements
- Minimum information for GUI
- Human phenotype ontology

Experience from the Neuromuscular Network



- Advisory committee for therapeutics
- Trial sites and patients
- Regulatory affairs (EMA workshops)
- Advisory boards
- Training

DATABASES

The TREAT-NMD Duchenne Muscular Dystrophy Registries: Conception, Design, and Utilization by Industry and Academia

Human Mutation
OFFICIAL JOURNAL
HGVS
HUMAN GENOME VARIATION SOCIETY
www.hgvs.org

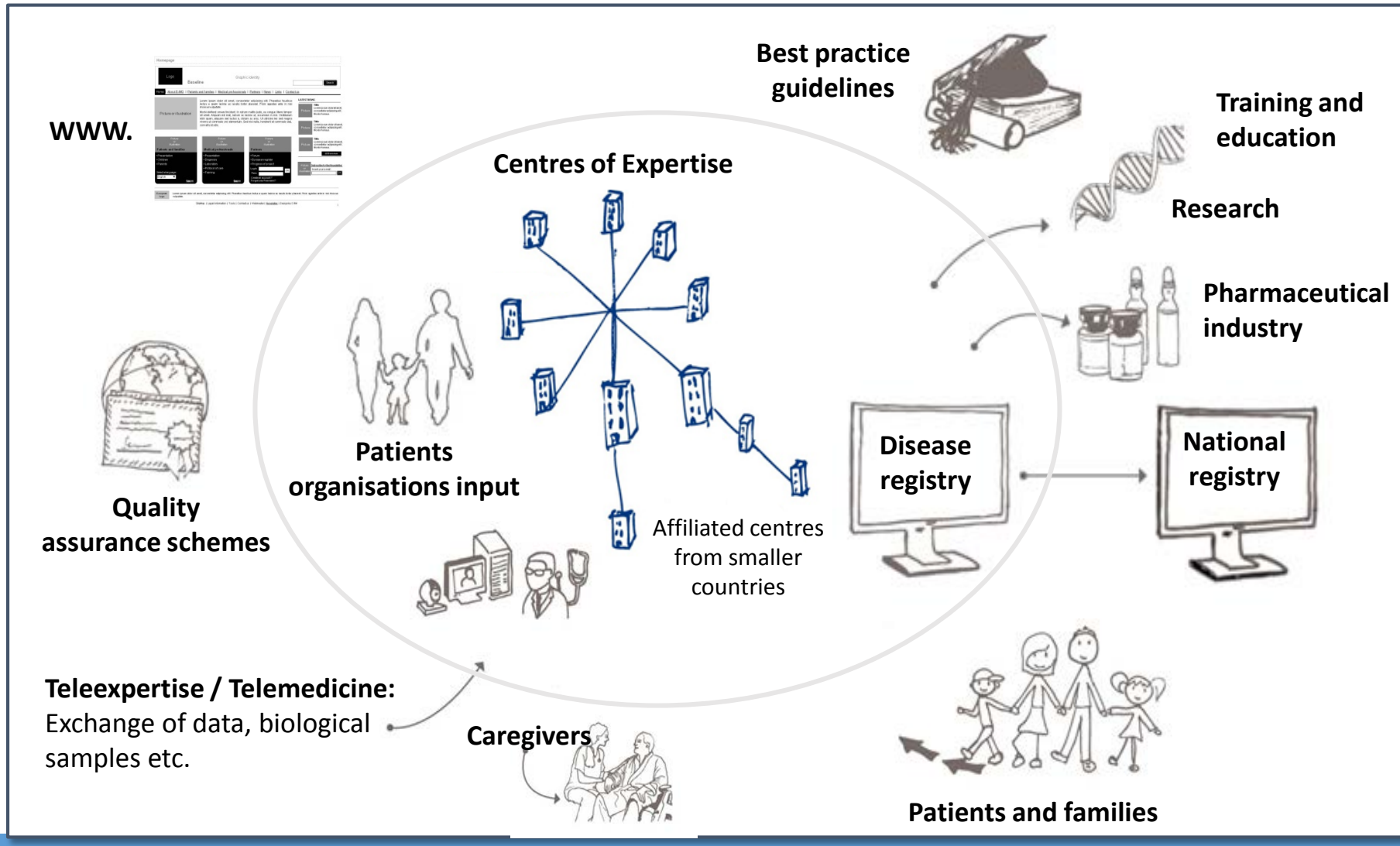
A framework to collaborate with industry - aim

- Advance and improve current approaches to RD clinical research
  - Key leaders organised
  - Work closely with family organisations
  - Reduce redundancy and additional burden to patients
  - Cost-effective and rigorous approach
- Adherence to good practice guidelines
- Sustainability for the timespan of the industry collaboration

A framework to collaborate - operational

- Build on achievements by sharing tools/models between networks
- Regular meetings that involve network clinical researchers and leaders from biotechnology/pharmaceutical industry
  - Foster communication about areas of need
- Transparency: EFPIA guidelines…
- Interest in sharing information to avoid duplication
  - Data standards
  - Natural history
  - Data access
- Cost-sharing arrangements to reflect different contributions to clinical research
  - Public private partnerships
  - Agreement with ERN – legal entity?
- Publication rules

ERN framework and industry



Thank you

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