ERNs: From Care to Research/ From Research to Care

EURORDIS Winter School

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This presentation is part of the project / joint action '677024 / RD-ACTION' which has received funding from the European Union's Health Programme (2014-2020).

Learning Objectives

- For all participants to understand ‘ERNs 101’, i.e. to appreciate what a European Reference Network IS
- Understand better the ‘status quo’ for ERNs in 2018
- Better visualise ERN contribution to/responsibilities around ‘research’ by:
  - Drilling down to *some* opportunities to add-value
  - Eliciting your views on what ERNs can ‘solve’ (and perhaps what they cannot)

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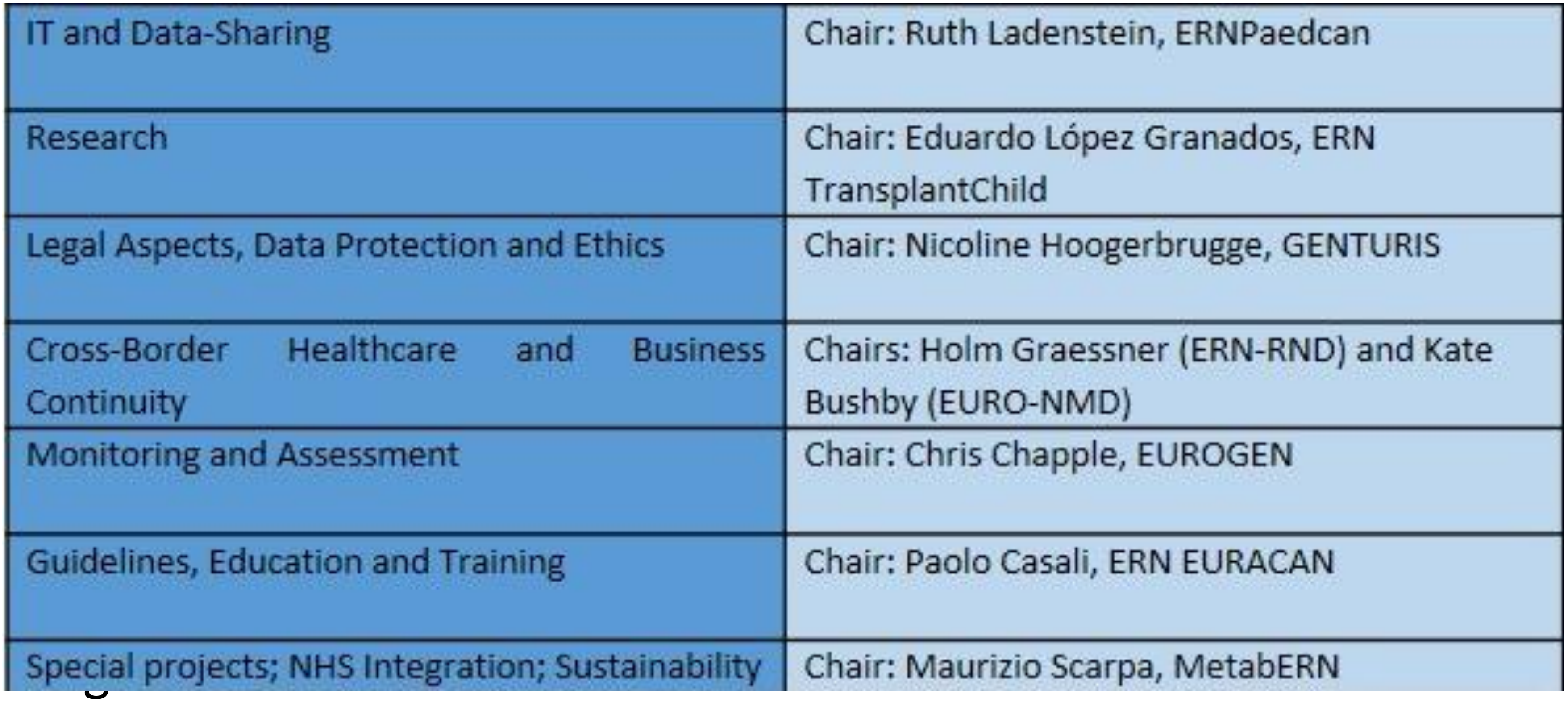
ERNs ‘in a nutshell’

- “A network connecting providers of highly specialised healthcare (‘CEs’) in domains where patients and expertise are scarce”
- Concept grounded in ‘Cross-Border Healthcare Directive’
- First and foremost dedicated to Care: great equaliser
- But strong research focus too, which is important in RD

ERNs must possess/must demonstrate

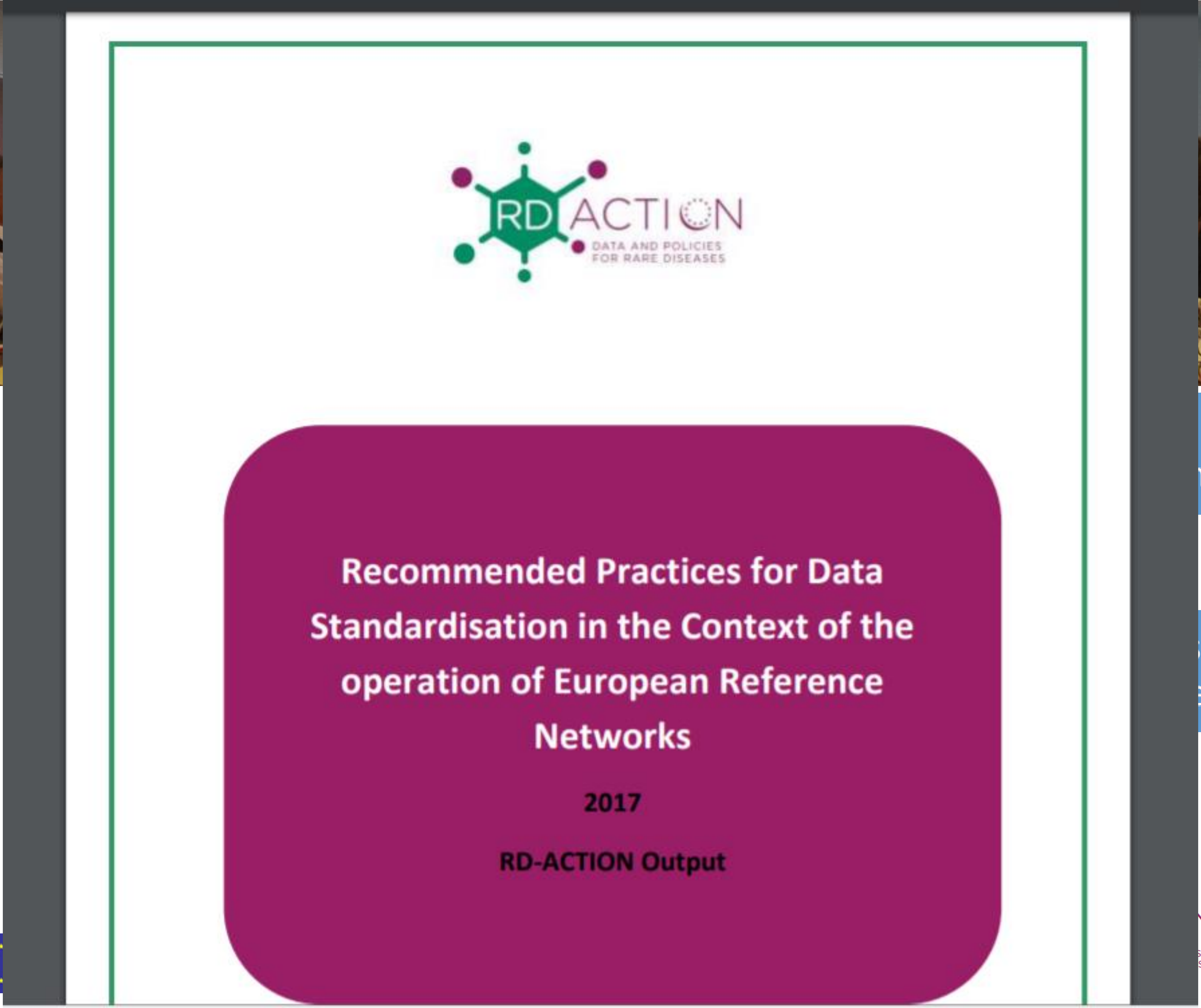
- ✓ knowledge and **expertise to diagnose, follow up and manage patients**
- ✓ Evidence of **good outcomes**
- ✓ **multi-disciplinary** approach
- ✓ capacity to **produce good practice guidelines and to implement outcome measures and quality control**
- ✓ collaborate with **other CEs and networks**
- ✓ **Research, teaching and training**

Where are we today?



IT and Data-Sharing	Chair: Ruth Ladenstein, ERNPaedcan
Research	Chair: Eduardo López Granados, ERN TransplantChild
Legal Aspects, Data Protection and Ethics	Chair: Nicoline Hoogerbrugge, GENTURIS
Cross-Border Healthcare and Business Continuity	Chairs: Holm Graessner (ERN-RND) and Kate Bushby (EURO-NMD)
Monitoring and Assessment	Chair: Chris Chapple, EUROGEN
Guidelines, Education and Training	Chair: Paolo Casali, ERN EURACAN
Special projects; NHS Integration; Sustainability	Chair: Maurizio Scarpa, MetabERN

- Funding opportunities emerging, but v. much ‘incremental’
- Collaborative partnering with RD-ACTION (workshops)



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Recommended Practices for Data Standardisation in the Context of the operation of European Reference Networks

2017

RD-ACTION Output

ERNs & Research – the Legal Acts

(5) To fulfil the requirement set out in point (iv) of Article 12(4)(a) of Directive 2011/24/EU ('make a contribution to research'), the Networks must:

(a) identify and fill research gaps;

(b) promote collaborative research within the Network;

(c) reinforce research and epidemiological surveillance, through setting up of shared registries.

(c) with regard to research and training capacity, applicant providers must:

(i) have the capacity to provide academic, university or specialised level training;

(ii) have human, technical and structural capacity, skill mix and resources;

(iii) have research capacity, and demonstrated research experience or production in the area of expertise of the Network, at national and international level;

Research Priorities of ERNs

- 1 year ago, RD-ACTION surveyed the Networks….
- All recognised the opportunity to straddle that ‘boundary’ of care and research and support ‘bench to bedside & back again’
- Differences between the ERNs! Need to remember that some Groups are very new, not to research, but to research at a pan-European or global level, and/or to research at the level of the current ERN scope.
- In some cases, goal is to devote the first year or two years entirely to *mapping* research. Others plan to accelerate and further develop research activities within the ERN
- **Are ePAGs aware of/influencing ERN research goals?**

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What *kind* of Research?

- How ERNs can –at least within themselves- conduct basic research is still a little unclear:
  - Some good basic research plans but…Cannot be done within ERNs as a closed ecosystem, surely…
- Most obvious areas for research activity at present seem:
  - In diagnostic sphere (Jill ☺)
  - Registries (span care & research, depending on type)
  - Clinical Trials/Studies

What makes ERNs an exciting ‘ecosystem’ for research?

I will discuss:

1. Multistakeholder Involvement
2. Disease scope of the ERNs
3. Clinical Trials & added-value

Multistakeholder Engagement

- Clinicians/Researchers (need to have both)
- Patients:
  - *"Patients and patient representatives should play an integral role in the decision and opinion making process in RD ERNs and be involved in structural and clinical network activities"* (https://ec.europa.eu/health//sites/health/files/rare_diseases/docs/20150610_erns_eucerdaddendum_en.pdf)
- European Commission (several DGs)
- Policy-Makers in each MS/EEA country
- Professional/Learned Societies
- EMA, HTA bodies, Payers
- Industry…

Pilot ERNs

CURENet

TREAT-NMD
Neuromuscular Network

EN-RBD

EuroCareCF
European Coordination Action for Research in Cystic Fibrosis

eb Clinet
Linking clinical expertise in EB.

ExPO-r-Net

e-pilepsy

EUROGLYCANET
CDG

EuroCleftNet

European Skeletal Dysplasia Network

CARE-NMD

Epnet

EuroCYST
bringing knowledge - improving outcome

ecorn-cf.eu
Expert Advice on Cystic Fibrosis

EUHANET
Improving the care of European citizens with inherited bleeding disorders

EUROFEVER PROJECT

A network of centres of expertise
dyscerne
for dysmorphology

NEUROPED

Euro-Histio-Net
EHN
A reference network for Langerhans Cell Histiocytosis and associated syndromes

HD
EUROPEAN HUNTINGTON'S DISEASE NETWORK

EUNEFRON

PRINTO

EIMD
European registry and network for Intoxication type Metabolic Diseases

PEDDCReN
Paediatric European Digestive Diseases Clinical Research Network

enerca
European Network for Rare and Congenital Anaemias

EuroWilson

EURenOmics

SWEET
PEDIATRIC DIABETES

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RARECARENet
Information Network on Rare Cancers

Pre-ERN era	What is different now?	What needs to happen?
Networks were disease-specific meaning sometimes arbitrary prioritisation of who 'got' a network and had funding	Comprehensive = only way for true equality	Need *thorough* Mapping to asses how comprehensive ERNs really are (e.g. based on Orphanet) Having realised the 'gaps' need support to realise the step-wise implementation… end goal is, someone somewhere takes 'responsibility' for your disease
Limited visibility of expertise in particular RD across Europe	Logical taxonomy Definition of what constitutes an expert in each field	Need time and energy to do that mapping (goes beyond ERNs) and then illustrate where those centres are Again, gaps cannot magically be filled
…		

Pre-ERN era	What is different now/CAN be different	What needs to happen?
Experts sometimes worked in silos, little cross-talk	Colleagues who never would have liaised, now beginning to do so: within Networks but also Between. Major potential in those 'experience-sharing' case debates	Networks need Time to harness the research potential here, in terms of disease-causing pathways, endpoints etc (and also suitable F2F opportunities)
Experts often worked competitively	Systematically identifying expertise and unifying it in non-competitive Networks offers hope of attitudes 'shifting'	Collaboration is easier when there is equal funding to go around… Need to avoid alienating those experts NOT in ERNs

Opportunities to add value to Clinical Trials (1)

Huge potential to 'build the village' and make disease areas more attractive: build trial-readiness:

- Mapping sites and experts: important to Industry but important for Regulators too! Potential to make Early Dialogue much more strategic
- Diagnostic potential means more patients, better described, for potential trials and studies
- Big opportunity to support PMS and gather real-world data…

Opportunities to add value to Clinical Trials (2)

- **Data Opportunities are vast, e.g.**
  - Need a better understanding of the disease and its epidemiology, its prevalence
  - Need more -and 'better'- natural history data, to design trials, agree endpoints, etc.
  - stratifying cohorts in registries means it is much easier to assess feasibility of a study
- Registries and CPMS offer opportunity to get data, on more diseases, more shareable
  - Logical approach is to invest in tools to enable patients to contribute data directly
    - Starting anew in some cases, need to integrate legacy collections in others. But need more clarity and funding for registries here…

- How can ERNs add value to research?
- What can they do better than the pre-ERN era?
- What needs to happen to realise these opportunities?

http://www.rd-action.eu/european-reference-networks-erns/

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