THE EUROPEAN REFERENCE NETWORKS: A VISION FROM INSIDE

MAURIZIO SCARPA
COORDINATOR,
EUROPEAN REFERENCE NETWORK
HEREDITARY METABOLIC DISEASES, MetabERN

CHAIR,
EUROPEAN REFERENCE NETWORK
COORDINATORS GROUP
THE ORIGIN OF THE ERNS

The road to ERNs

2002
Free movement of patients

2005
Commission's High Level Group on Health Services and Medical Care Centres of Reference report

2006
RD Task Force Working Group on centres of reference Updated report CoR Public Health Programme First pilot networks projects

2011
Directive application of patients' rights in cross-border healthcare Article 12 ERN

2013
Eucer Recommendations European Reference Networks (Rd Erns)

2014
Commission Decisions on ERN

Slide kindly provided by Enrique Terol, EU Commission
Grouping Rare Diseases In Thematic Networks

- Rare immunological and auto-inflammatory diseases
- Rare bone diseases
- Rare cancers* and tumours
- Rare cardiac diseases
- Rare connective tissue and musculoskeletal diseases
- Rare malformations and developmental anomalies and rare intellectual disabilities
- Rare endocrine diseases
- Rare eye diseases
- Rare gastrointestinal diseases
- Rare gynaecological and obstetric diseases
- Group on Cancer Control

- Rare haematological diseases
- Rare craniofacial anomalies and ENT (ear, nose and throat) disorders
- Rare hepatic diseases
- Rare hereditary metabolic disorders
- Rare multi-systemic vascular diseases
- Rare neurological diseases
- Rare neuromuscular diseases
- Rare pulmonary diseases
- Rare renal diseases
- Rare skin disorders
- Rare urogenital diseases

*Note: The networking of rare cancers is currently under discussion in EC Expert
ERNs Mimick Nature
<table>
<thead>
<tr>
<th>No.</th>
<th>Name</th>
<th>Description</th>
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<tr>
<td>1.</td>
<td>ERN BOND</td>
<td>European Reference Network on Rare Bone Disorders</td>
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<td>European Reference Network on Rare craniofacial anomalies and ENT disorders</td>
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<td>3.</td>
<td>Endo-ERN</td>
<td>European Reference Network on Rare Endocrine Conditions</td>
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<td>European Reference Network on Genetic Tumour Risk Syndromes</td>
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<td>European Reference Network on Uncommon And Rare Diseases of the HEART</td>
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<td>16.</td>
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<td>European Reference Network on Rare Congenital Malformations and Rare Intellectual Disability</td>
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<td>ERN eUROGEN</td>
<td>European Reference Network on Rare and Complex Urogenital Diseases and Conditions</td>
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European Reference Networks Conference
& ERN Kick-off meeting
Vilnius, Lithuania, 9-10 March 2017
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Involvement of Patients in the ERNs

- care is patient-centred
- transparency in quality of care, safety standards, clinical outcomes and treatment options
- planning, assessment and evaluation of the ERN

- Patients involvement is ongoing
- Patients representatives are equal participant
- Results and act upon them to implement changes and amelioration of the achievements

- All ethical issues & concerns for patients are in place
- the performance of the ERN.
- Provision of patient perspective
- Methods for feedback and evaluation of patient experience,
- Transparency in quality of care, safety standards, CO and treatment options
- Clear planning, assessment and evaluation of the ERN

- Review involvement mechanism regularly
- Incorporate patient feedback to implement improvements
- Involve patients in the addressing of their specific needs, preferences, priorities (Survey, feedback systems, etc)

Advice
Ensure
Monitor
Evaluate
### MetabERN: ERN on HEREDITARY METABOLIC DISEASES

**Coordinator**
Prof. Maurizio Scarpa MD PhD
Helios Dr. Horst Schmidt Kliniken Wiesbaden,

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1) **Aminoacid and organic acids related disorders**
2) **Disorders of pyruvate metabolism, Krebs cycle defects, mitochondrial oxidative phosphorylation disorders, disorders of thiamine transport and metabolism**
3) **Carbohydrate, fatty acid oxidation and ketone bodies disorders**
4) **Lysosomal disorders**
5) **Peroxisomal and lipid related disorders**
6) **Congenital disorders of glycosylation and disorders of intracellular trafficking**
7) **Disorders of Neuromodulators and Small Molecules**

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### MetabERN List of the Italian HCPs

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<th>Vice</th>
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<td>Serena</td>
<td>Alberto Piperno</td>
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<tr>
<td>Azienda Ospedaliera Universitaria &quot;Federico II&quot;, Napoli</td>
<td>Giancarlo</td>
<td>Pietro Strisciuglio</td>
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<td>Azienda Ospedaliera Universitaria Integrata di Verona</td>
<td>Alessandro</td>
<td>Andrea Bordugo</td>
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<td>Azienda Ospedaliera Universitaria Senese</td>
<td>Antonio</td>
<td>Maria Teresa Dotti</td>
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<tr>
<td>Azienda Ospedaliero-Universitaria Pisana</td>
<td>Ferruccio</td>
<td>Giovanni Ceccarini</td>
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<td>Azienda Sanitaria Universitaria Integrata di Udine</td>
<td>Bruno</td>
<td>Andrea Dardis</td>
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<tr>
<td>Giannina Gaslini Institute (IRCCS Giannina Gaslini)</td>
<td>Maja</td>
<td>Roberto cerone</td>
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<tr>
<td>Meyer Children's Hospital</td>
<td>Maria Alice</td>
<td>Elisabetta Pasquini</td>
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<tr>
<td>Ospedale Pediatrico Bambino Gesù</td>
<td>Carlo</td>
<td>Enrico Bertini</td>
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<tr>
<td>San Paolo Hospital, ASST Santi Paolo e Carlo</td>
<td>Graziella</td>
<td>Giuseppe Banderali</td>
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<tr>
<td>University Hospital of Padova</td>
<td>Alberto</td>
<td>Giulia Polo</td>
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</table>
**MetabERN Structure**

- **MetabERN Board**
  - General assembly of representatives from all HCPs, patient groups and other third party stakeholders involved in the activities of the MetabERN

- **Medical Executive Board (MEB)**
  - (Including coordinator, vice coordinator and leadership of sub-networks)

- **MetabERN Patient Board**
  - (Including ePAG representatives as well as all other patient representatives involved in the advisory boards of individual sub-networks)

- **MetabERN Advisory Board (MAB)**
  - (Including ePAG patient and family associations representatives, policy makers, foundations, ethics specialists, collaborating networks and partnering ERS where an overlap of diseases requires coordination)

- **External Experts Committee**
  - (Including external experts, lead and supervised by the SEEM)

### Sub-Groups

- **Amino- and organic acids related disorders (AOD)**
  - MetabERN Advisory Board (AOD Advisory Board)
  - Phenylketonuria
  - Urea cycle disorders
  - Maple syrup urine disease

- **Carbohydrate, fatty acid oxidation, ketone bodies disorders (C-FAO)**
  - MetabERN Advisory Board (C-FAO Advisory Board)
  - Glycogen storage disorders
  - Disorders of galactose metabolism
  - Gluconeogenesis

- **Lysosomal storage disorders (LSD)**
  - MetabERN Advisory Board (LSD Advisory Board)
  - Mucopolysaccharidosis
  - Niemann-Pick disease
  - Refsum disease

- **Peroxisomal disorders (PD)**
  - MetabERN Advisory Board (PD Advisory Board)
  - Zellweger spectrum
  - Disorders of peroxisomal proteins

- **Disorders of pyruvate metabolism, mitochondrial oxidative disorders, thiamine transport and metabolism (PM-MID)**
  - MetabERN Advisory Board (PM-MID Advisory Board)
  - Disorders of pyruvate metabolism
  - Disorders of mitochondrial oxidative disorders
  - Disorders of thiamine transport and metabolism

- **Congenital disorders of glycosylation and intracellular trafficking (CDG)**
  - MetabERN Advisory Board (CDG Advisory Board)

- ** Disorders of neurotransmitters and other small molecules (tSOMS)**
  - MetabERN Advisory Board (tSOMS Advisory Board)

### Thematic Committees

- **Thematic committees**
  - (Including aim, mission, areas of focus, thematic guidelines and SOPs, quality of life, ethical issues, transparency, replies and outcome measures, patient empowerment, education and training)
The MetabERN Multidisciplinary Team (MDT)

MDT composed by:
- 871 Specialized Medical Doctors
- 188 Biochemists/Biologists
- 184 Nurses
- 121 Dietician/Nutritionist
- 76 Physical therapists/Rehab
- 73 Psychologist
- 49 Social worker
- 34 Pharmacists
- 34 Coordinator/Medical Secretary
- 13 Expert in Palliative care/Pain management
- 10 PHD/student
- 28 Other

a Total of 1681 Experts

+ 47 Patients Organizations

The European Team

MDT composed by:
- 112 Specialized Medical Doctors
- 11 Psychologist
- 10 Dietician/Nutritionist
- 8 Nurse/specialized and study nurse/genetic counsellor
- 2 Pharmacist
- 27 Biochemist/Biologist/lab specialist
- 3 PHD/student
- 4 Social worker
- 4 Coordinator/Medical Secretary
- 5 Physical therapist/Rehab
- 2 Other

a Total of 188 Experts

The Italian Team
Composition of the MetabERN MDT

- Specilized Medical Doctors: 52%
- Biochemists/Biologists: 11%
- Nurses: 11%
- Dieticians/Nutritionists: 7%
- Physical therapists/Rehab: 4%
- Psycologist: 4%
- Social worker: 3%
- Pharmacists: 2%
- Medical Secretaries: 11%
- Experts in Palliative care/Pain management: 11%
- PHD students: 11%
- Other: 52%
Composition of the MetabERN Specialists

- Pediatrician/Neuropediatrician: 17%
- Internist/Geneticist: 15%
- Neurologist/neuromuscular: 11%
- Metabolic physician: 10%
- Neurophysiologist/Neurophatologist/Radiologist: 8%
- Cardiologist: 5%
- Hepatologist: 4%
- Pediatric intensivists/neonatology: 3%
- Nephrologist: 2%
- Orthopedic: 2%
- Ophthalmologist: 2%
- Gastroenterologist: 2%
- Endocrinologist: 2%
- Surgeon: 2%
- Hemato/onco/infectio/Immunologist: 2%
- Psychiatric: 2%
- Pneumologist: 2%
MetabERN: Number of Patients per Country
MetabERN: Patients Coverage

Number of Patients

<table>
<thead>
<tr>
<th>Patients Coverage</th>
<th>Pediatric patients</th>
<th>Adults</th>
<th>Total</th>
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<td>MetabERN _Italian</td>
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<td>2060</td>
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32% Pediatric Patients
68% Adults

35% Pediatric Patients
65% Adults
MetabERN: Main Diseases Categories

Main categories coverage

AOA: 11%
PM-MD: 21%
C-FAO: 8%
LSDs: 12%
PD: 11%
CDG: 21%
NOMS: 16%
Patients Managed by MetabERN in the last year
| 1. ERN BOND                        | 2. ERN CRANIO                       |
| 3. Endo-ERN                        | 4. ERN EpiCARE                      |
| 5. ERKNet                           | 6. ERN RND                           |
| 7. ERNICA                           | 8. ERN LUNG                           |
| 9. ERN Skin                         | 10. ERN EURACAN                      |
| 11. ERN EuroBloodNet               | 12. ERN EURO-NMD                     |
| 13. ERN EYE                         | 14. ERN GENTURIS                     |
| 15. ERN GUARD-HEART                | 16. ERN ITHACA                       |
| 17. MetabERN                       | 18. ERN PaedCan                      |
| 19. ERN RARE-LIVER                  | 20. ERN ReCONNET                     |
| 21. ERN RITA                        | 22. ERN TRANSPLANT-CHILD             |
| 23. VASCERN                         |                                |
| 24. ERN eUROGEN                    |                                |

ERN ACTIVITIES

- Prevention and Screening
- Diagnosis/New Diseases Diagnosis
- Epidemiology/Registries and Outcome
- Management/Quality of Life and Transition Guidelines and Pathways
- Virtual Counselling
- Education and Training
- Patient Empowerment
- Clinical Trials
- Research
- Dissemination/Stakeholders Relations
One Of The Secrets For Success Of The ERN Crosslinking : The IT Platform
**Major Opportunities Offered By The ERNs**

**Health System**

- Close contact with regulatory authorities, key opinion leaders, patient advocacy organizations, and non-traditional players, like technology companies, to create a new coordinated way to manage patients affected by rare diseases and optimize access to diagnosis and therapy.

- Impact on the National Health System to spare resources, optimize services and avoid waste of expertise

- Impact on the awareness for rare diseases. Creation of an European epidemiology on rare diseases, common registries, databases, research projects.

- Impact on the awareness for cost of rare diseases. Creation of EU HTAs for the calculation of the cost of the management of patients with rare diseases and prediction of sustainability.

- Impact on the discussion on the sustainability of orphan drugs.

- Interaction with National Health Institute and Drug agency for postmarketing analysis.
EUROPEAN REFERENCE NETWORKS

Major Opportunities Offered By The ERNs

Patients

• Optimization of care pathways for patients with rare disease: diagnosis, management access to therapy

• More effective clinical trial designs that consider the very special and unique circumstances of rare disease research

• Creation of databases/registries/natural histories of patients in a coordinated way

• Close interaction with family associations which may help logistical challenges associated with conducting trials for small patient population

• Close interaction among ERNs to integrate expertise needed for clinical trials

• Possibility of helping Companies to generate new clinical trial designs based on the right choice of the primary and secondary endpoints based on the well characterised patient population followed by the ERN.

• Possibility of generating studies also for ultrarare patients due to the availability of numbers of patients in a coordinated way

• Protection of patients from not well designed clinical trials

• Counselling the patients in the understanding the value of the clinical trials
# The ERN Coordinators Group

Chair: Maurizio Scarpa, MetabERN  
Vice-Chair: Franz Schäfer, ERKNET  
Board: WG Coordinators

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<th>WG</th>
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<tr>
<td>IT &amp; DATA-SHARING</td>
<td>Ruth Ladenstein, ERNPaedcan</td>
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<td>RESEARCH</td>
<td>Eduardo López Granados, ERN TransplantChild</td>
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<td>LEGAL ASPECTS, DATA PROTECTION &amp; ETHICS</td>
<td>Nicoline Hoogerbrugge, GENTURIS</td>
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<td>CROSS BORDER HEALTHCARE AND BUSINESS CONTINUITY</td>
<td>Holm Graessner, ERN-RND; Kate Bushby, EURO NMD</td>
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<td>MONITORING &amp; ASSESSMENT</td>
<td>Christopher Chapple, EUROGEN</td>
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<td>GUIDELINES, EDUCATION, TRAINING</td>
<td>Paolo Casali, ERN EURACAN</td>
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<td>SPECIAL PROJECTS: NHS INTEGRATION SUSTAINABILITY</td>
<td>Maurizio Scarpa, MetabERN</td>
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Achievements
The ERN Interactome: Coordination of the Activities

1. ERNs COORDINATION
2. PATIENT COMMITMENT
3. DISEASE IDENTIFICATION
4. REGISTRY
5. DATA SHARING
6. COMUNICATION/AWARENESS
7. EDUCATION
8. SHARED VIRTUAL COUNSELLING
9. NETWORK OF SPECIALISTS
10. INSTITUTIONAL STAKEHOLDERS
11. PAYERS
12. NATIONAL HEALTH SYSTEMS/NPRD
13. RESEARCH COORDINATION
14. GUIDELINES/PATIENT PATHWAYS
15. STANDARD OPERATING PROCEDURES
16. QUALITY INDICATORS
25th Workshop of the EURORDIS Round Table of Companies (ERTC)

Healthcare Companies & European Reference Networks:
Expectations & Potential for Collaboration

PROGRAMME
Morning session co-chaired by:
Chairpersons: Ms. Olivia Cindy, Managing Director, HNT Europe, Italy & Mr. Josep Torrent-Farrell, Head of Medicines Division, CatSalut (Catalan Health Service), Generalitat de Catalunya, Spain

<table>
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<th>Time</th>
<th>Activity</th>
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<td>09:00 - 09:10</td>
<td>Welcome introduction, setting the scene &amp; goals for the day: A reminder of why we are all here. Our shared ambition for ERN.</td>
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<td></td>
<td>Mr. Yann Le Cam, Chief Executive Officer, EURORDIS, France/Belgium</td>
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<td>09:10 - 09:30</td>
<td>State of the art of ERNs: What they are (core responsibilities), how they are structured and organized and their overall vision</td>
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<tr>
<td></td>
<td>Mr. Jeroen Van Gijn, Policy Officer, European Commission</td>
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<tr>
<td>09:30 - 09:45</td>
<td>Board of Member States Reflections</td>
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<td>Prof. Nicole Haegener-Brunner, Chair, EC ERN Committee on Data Protection, Ethics &amp; Informed Consent, The Netherlands</td>
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<tr>
<td>09:45 - 09:55</td>
<td>From concept to reality: European Reference Network for Hereditary Metabolic Diseases, MetaDiMN</td>
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<td>Dr. Mauritza Scarp, Clinical lead for Rare Metabolic Diseases, Director of the Centre for Rare Diseases Helios Dr. Horst Schmidt Clinic, Germany</td>
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<td>09:55 - 10:05</td>
<td>Round table Q&amp;A: Current state of play, needs and expectations, looking ahead</td>
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<td>Moderator: Mr. Josep Torrent-Farrell, Head of Medicines Division, CatSalut (Catalan Health Service), Generalitat de Catalunya, Spain</td>
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<td>Panelists: Mr. Jeroen Van Gijn, Policy Officer, European Commission</td>
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<td>Dr. Mauritza Scarp, EURORDIS Chairperson, Clinical lead for Rare Metabolic Diseases, Director of the Centre for Rare Diseases Helios Dr. Horst Schmidt Clinic, Germany</td>
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<td>Prof. Nicole Haegener-Brunner, Professor in Hereditary Cancer, Radboud University Medical Center, The Netherlands &amp; Chair, EC ERN Committee on Data Protection, Ethics &amp; Informed Consent</td>
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20TH European Health Forum
Gastein
Health in All Politics – a better future for Europe

1) WORKSHOP ON PERSONALISED MEDICINE AND RD
2) WORKSHOP ON SUSTAINABILITY OF ORPHAN DRUGS
3) WORKSHOP ON BIG DATA
4) MEETING WITH THE COMMISSIONER:
   • LEGAL ENTITY
   • SUSTAINIBILITY
Work in Progress for the Coordinators

- Continuous collaboration with the BoMS representatives
- Sustainability of the ERNs
- Integration into National Health Systems
- Legal entity of the ERNs
- Reimbursement of the ERN activities
- Liability of Coordinators/ recognition of the Coordinator role
Conclusions

✓ ERNs are NOT a project or a program, they are a CONCEPT* and represent the major achievement of the European spirit of collaboration, sharing and health investment in the field of rare diseases.

✓ ERNs group the most acknowledged centers of excellence in Europe, all sharing the same level of commitment and interest for rare diseases.

✓ ERNs are NOT single networks, but rather a critical mass of about 300 hospitals and over 1000 specialised units, cross feeding each other to meet the needs of our patients.

✓ Crosslinking is in the nature of the ERNs due to multidisciplinarity implications of the cared diseases.

✓ The ERNs´ integration inside the MS National Health Systems/National Plans for Rare Diseases will represent a major achievement for the Cross Border Care.

✓ The ERNs´/MS National Health Systems/National Plans for Rare Diseases integration is instrumental to define strategies to optimize the cost of management of patients of rare diseases.

✓ The ERN Coordinators Group is an unvaluable instrument for the EC/ National Health Systems/National Plans for Rare Diseases to define a uniform pan-European plan for rare diseases.

* Quoted after Victoria Hedley, Newcastle, UK