ERN on Rare Bone Diseases

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Istituto Ortopedico Rizzoli
Centres of Expertise and networks.

Member states
Contacted states
Affiliated states
Centres of Expertise for metabolic bone disorders

Outside EU
Istanbul TR
Saint Petersburg RU

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and the name is:
“BOND: ERN on Rare Bone Diseases”
ERN Structure

Hub-and-Spoke Structure

This is the model proposed by the EU.

Multi-Tiered Hub Structure

Country level
Timeline ERN Bone Rare Disease Meetings

- June 30, 2014: 1st EU ERN Conference
- July 1-2, 2015: Meeting Centers ERN Bone RD 12th meeting
- July 31, 2015: ISDN
- Oct 9, 2015: Workshop on ERN Services Brussels
- Jan 21, 2016: EU workshop DG SANTE’ ERN Rare Diseases
- Feb 10, 2016: 2nd EU ERN Conference
- Meeting skeletal dysplasia network & metabolic bone disorders network
- Decision to merge to form one rare bone diseases ERN
Advantages of a Unique ERN on Rare Bone Disease

Skeletal Dysplasia & Metabolic Bone Diseases
- Care
- Guidelines
- Sustainability
- Expertise
- Databases
- Clinical trials

- Common pathways
- Integrated Care
- Holistic Approach on Overlapping RD e.g. OI
- Integrated Research
- Added chances of success by the EC
**TIMELINE**

**PREPARING THE PROPOSAL**
- Define network coordinator:
  - Luca SanGiorgi
- Define Network diseases areas:
  - skeletal dysplasia
  - metabolic bone diseases
- Define governance:
  - Coordination board
  - Advisory board
  - Management Committee

**NETWORK APPLICATION & ASSESSMENT PROGRAMME**
- **8 months**
- **3 months**

**Stage 1**
- Call for interest from EC

**Stage 2**
- Application submitted to EC

**Stage 3**
- Application reviewed by EC then IAB
- Assessments and reports completed by IAB

**Stage 4**
- Assessment results sent to EC

**Stage 5**
- Final approval by BoMS

**HCP candidates must:**
- assign a representative
- fill the self-assessment form for HCP
- request national assessment
- contact patient organisations

**The Network coordination board must:**
- fill the self-assessment form for network
- define Board policies and rules
- define Network inclusion criteria

- Application forms
- Self-assessments
- Supporting documentation
- MS letter of endorsement

- Documentation review
- Virtual interviews
- On-site audits
ERN Architecture & Governance

Coordinator
Luca Sangiorgi - IOR

Steering Committee (max 12)
- experts from skeletal dysplasia network
- experts from metabolic disorders network
  + patients representatives
  + professional societies representatives

Management Committee
- One representative of each HCP
  + patients representative

Scientific Advisory Board

Skeletal Dysplasia & Metabolic Bone Diseases

HCP: Healthcare Providers
What Services should we offer?

• Clinical
  Direct: Clinical evaluation for prenatal, paediatric and adult patients (Genetics, Endocrinology, Fetal medicine, Radiology, Orthopedics, Neurology, Habilitation/Rehabilitation, Neurosurgery, Dentistry, Pathology, Physical therapy, Occupational therapy, and Psychological support), adequate therapies, and teleconsultation.

Support to healthcare providers: e-Health (Exchange, gather and disseminate knowledge)

• Non Clinical
  Clinical guidelines / patient pathways (Implement outcome and performance indicators)
  Epidemiological surveillance, registries
  Training and continuous education programmes
  Dissemination of information

• Trials
  Selection of patients (registries)
  Training of professionals in assessment protocols
Expectations on ERN on Rare Bone Disease

Our expectations ...
→ Improved diagnostics and access to new treatments
→ Increasing visible expert teams being a magnet for attracting complex cases,
→ Improved access to timely advice, diagnosis and treatment (their needs quickly)

Stay the same ...
→ Patients will continue to receive care locally to where they live.
→ Patients will see no change to care, but will have better outcomes.

What is different ...
→ How clinicians connect with each other and the reach of their professional network.
Virtual Consultation System

The web system supports researchers in management of complex clinical cases through online consultations among experts world-wide and integrates tools for Rare Diseases Board review.
**ERN Care Model**

**Complex and rare cases**

- **Multi-system conditions and diseases**

**Ultra-rare and highly specialised intervention or surgery**

**Expertise travels to the patient:**
- Stage 1: Specialist care planning advice to ‘local services’ for complex cases – care delivered by local team
- Stage 2: Specialised case consultation at HC – care delivered by HC

**Expertise travels to HCP in the ERN:**
- Stage 3: ERN virtual case review and specialist advice to HC – care delivered by HC

**Patient travels to the expert:**
- Stage 4: HC to HC internal referral within the ERN to specialist HC:
  - For diagnostic and care planning for ultra rare diseases
  - For highly specialized interventions or surgery
Evolution from concept to reality

Year 1 - 2017:
→ Organisation monitoring / assurance framework and governance of the network
→ Coordination and organisation of care – referral pathways, case review, second opinion and delivery of care and treatment
→ Basic IT platform (communication and first clinical tools)
→ Proof of concept

Year 2 - 2018:
→ Formalising ERN MDT working and establishing external relationships
→ Best practice starting to emerge
→ Extension of disease and geographical coverage
→ Clinical IT platform (common clinical tools)

Year 3 - 2019:
→ Agreed outcome monitoring and benchmarking
→ Greater connection and integration of research and healthcare

Year 4 – 2020:
→ Mature IT solutions increasing interoperability and standards

Year 5 - 2021:
→ Preparation for EC independent evaluation in 2021/2
ERN working groups

- Diagnostic challenges
- Coordination of multidisciplinary care (e-Health tools)
- Databases: natural history of diseases
- Good practices and guidelines
- Clinical Trials
- Education and Training
- Research
- Interactions with other ERNs
- Visibility towards patients and HCP

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Inter-ERNs working groups

- Interactions with other ERNs
- Project managers
- Interoperability between ERNS
- Patients “bridging” ERNs
- Collaborations with ERICs
- Research
- Collaboration with private companies
- Education and Training

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