



## EU cooperation in CBHC, ERNs for RDs: The patient's perspective

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# Ambitious new landscape

# Big Ambition

## Individual ambition:

- No decision about me, without me!

## National ambition (Committee Experts for Rare Diseases):

- All RDs covered by at least one ERN which focus on groups of diseases such as rare hematologic diseases, rare pulmonary diseases, etc.

## European ambition (International Rare Diseases Research Consortium):

- 200 new therapies for rare diseases
- Means to diagnose most rare disease by 2020



# Integrated approach

# Integration and interoperability

## New emerging landscape for Rare Diseases:

- European Patients' Academy on Therapeutic Innovation
- Connecting Big Data revolution for RD
- Integration of Research Infrastructure and transitional research pathway focused on RD
- Centres of Expertise accreditation, connected up
- European Reference Networks to 'level up' of expertise and knowledge
- Market incentives to promote development of orphan drugs – time-limited exclusivity

***“Every person with a rare disease has a home”***

# Challenge our concept of a Network

## Rationale:

- Centralization of expertise or resources for
- Rarity of expertise, low prevalence, complexity, or high cost treatment

## Concept of European Reference Network of Centres of Expertise:

- Demonstrate 'Added Value' at an EU level
- Healthcare, not research
- Effective networking, enhance communication
- Requirement to be responsive
- Flexible in model, inclusive not exclusive,
- Centralised care vs. referral networks: shared care arrangements
- Leveling up of knowledge and expertise through dissemination
- Collaboration and cooperation

# Legislative Framework



# A long road travelled

“Man will occasionally stumble over the Truth, but usually manages to pick himself up, walk over or around it, and carry on”

# Healthcare dynamic

## Healthcare responsibly:

- Common policy and legislation, but healthcare is a 'shared competency'
- ERNs are a European benefit, based on cooperation not investment
- Handing the 'baton' from Commission to Member State
- Engagement in implementation phase is critical for integral partners and leading experts

## European Commission:

- Coordinators of assessment process
- Time is pressure with an ambitious roadmap for delivery
- Lack of articulated common shared vision or coordinated strategic approach

## Member States:

- Experience and expectation variable
- Responses in some MS is slow to stimulate debate and appetite
- RD National Plans key lever at a national level

# Common policy and common legal framework

- National Plans and Centres of Expertise
- Commission Communication, call to address Europe's challenge for Rare Diseases
- Council Recommendation
- CBHC: concept of ERNs was adopted
- **Delegated decision and Implementation decision**
  - The legislative 'What' and the 'How'
  - PO not specifically included in governance, assessment and evaluation, of networks in legislation. Legislation does not excluded networks to do this.
  - Networks are required by law to be, and demonstrate patient centric care and patient empowerment

[http://www.rare-diseases.eu/wp-content/uploads/2014/05/0102\\_Enrique\\_TEROL2.pdf](http://www.rare-diseases.eu/wp-content/uploads/2014/05/0102_Enrique_TEROL2.pdf)

# Our journey from here

# Application & Assessment Process

Networks are required to have:

- Minimum of 10 healthcare providers from 8 MS
- Member State must endorse Centres of Expertise
- Centres can be Associate or Collaborative National Centres
- Technical assessment completed based on a common assessment manual
- Assessment completed by an Independent Body
- Member State Board will approve applications
- Accreditation with EU Logo
- Positive assessments will be made public

# Our journey ahead

- Adoption legal acts
- Awareness campaign launched
- Call for Assessment Manual and toolkit
- Identifying clusters of diseases workshop  
feedback to CERD
- Selection of Independent Assessment body(ies),  
and networks guidelines and technical  
documents
- Call for Networks – Complete individual's  
proposal and self-assessment
- Technical Assessment (criteria and conditions)
- Positive assessment equates to approval of  
network with established membership with ERN  
logo awarded
- Establish of Networks
- March 2014
- 23 June 2014
- July 2014
- November 2014
- Q4 2014
- Q1 2015
- Q4 2015

# Challenges we face

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- Commitment, cooperation and coproduction
- Economic pressure, financial sustainability
- Health systems under pressure
- Practicalities in access care
- Variation across Europe
- Accessing treatment
- Transparent pricing, reimbursement of true cost
- Fragmentation of Rare Diseases
- Dilution of patient voice, lost in translation
- Transparency of decision making
- Communication and language barriers
- Adoption of EU best practice guidelines



# Unlocking Potential

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- Culture of learning, face to face meetings
- Co-production with all partners is integral
- Quality and safety benchmarking
- Timely diagnosis, reduce undiagnosed and misdiagnosed
- Understanding natural history
- Improved clinical outcomes, quality of life
- Reduce burden of care, inappropriate hospital attendances and treatments
- Health competition and collaboration
- Valuing variation vs standardisation of practice

# Charting new territory

## Neurofibromatosis Type 2 – to illustrate:

- Designated 4 Centres of Expertise to provide 1 national service
- Different clinical leadership, multidisciplinary team models in each centre
- Competition between each other moved to cooperative on a shared goal, best and most effective treatment for NF2
- National service specification and cross centre clinical consensus and agreed clinical outcomes (triangulation):
  - Reduction in disease related mortality
  - Timing between presentation of symptoms and surgical intervention
  - Length of time that useful hearing is maintained in at least one ear from date of diagnosis
  - Facial palsy rates post surgery for vestibular schwannoma
  - Proportion of full time users of an Auditory Brain Stem Implant or cochlear implant

## Networking benefits:

- Commission through evaluation – Avastin based on a small clinical study, agreed strict clinical protocol and target population. Prescribing developed from routine treatment to time limited
- Development of surgical philosophy – move from a watch and wait due to risks of surgery, to earlier removal of smaller tumours
- Developed clinical expertise for ABIs and Cochlear Implants. Protocol for MRI ‘head-rapping’

# Thank you