Collaborative Research for a very rare disease as a global approach under FP7

Experiences of the German NBIA patient organization "Hoffnungsbau e.V." as TIRCON-Partner

Contact: tircon@bayfor.org
The disease: What is NBIA?
Neurodegeneration with Brain Iron Accumulation
The German NBIA patient group

- Launched in 2002
- Our mission: Improvement of diagnostics, treatment options, medical and psychosocial care and research
- Our tasks:
  - providing emotional support and advice to families
  - providing information to all stakeholders in the field of NBIA
  - lobbying for adequate infrastructures in medical care
  - networking and cooperation
  - supporting research
From start till today: Our research support strategies

- Common main goal: to support research in NBIA
- Research grants:
  - NBIADA totaling about $ 720,000
  - HoBa totaling about € 50,000
- Identification of researchers, purposes and approaches
- Networking between all stakeholders
- Building a bridge between scientists and patients
- Dissemination of research related information
- Lobbying
The origins of TIRCON
TIRCON Partners

12 partners from 7 countries:

• 2 clinical and basic research centers
• 4 clinical centers
• 2 basic research centers
• 1 Biotech-Enterprise
• 2 patient Advocacies
• 1 Research support company
**Work package 7-Dissemination**

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7.1 Networking and communication plan
7.2: NBIA Network in Europe and USA

- Participants
7.4: Dissemination of research results

Letter to the Editor

An international registry for neurodegeneration with brain iron accumulation

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Abstract (provisional)

We report the development of an international registry for Neurodegeneration with Brain Iron Accumulation (NBIA), in the context of TIRCON (Treat Iron-Related Childhood-Onset Neurodegeneration), an EU- FP7 -funded project. This registry aims to combine scattered resources, integrate clinical and scientific knowledge, and generate a rich source for future research studies. This paper describes the content, architecture and future utility of the registry with the intent to capture as many NBIA patients as possible and to offer comprehensive information to the international scientific community.

The complete article is available as a provisional PDF. The fully formatted PDF and HTML versions are in production.
7.5: Teaching PKAN/NBIA

- Specific educational training of the early-career scientists and clinicians
- Creation of an international research prize for early-career scientists
Positive experiences as partner in an EU-project:

Integration of patient advocacy as partner works:
- Contributing specific competences or knowledge
- building the bridge between scientists and patients
- Bringing the patients’ perspective into science
- Learning and Professionalizing

Rules
=> Grant /Consortium Agreement
- Responsibility Confidentiality, Reliability
- Restraint, if lack of knowledge
Patient organisations in EU-projects: Challenges

- Special skills => urgently needed
- Home office => space, equipment
- „World of science“ vs. „World of patients“
- Nursing vs. Advocacy
Our partners:

NBIA and Rare Diseases Patient Advocacies
Thanks for your attention!