TIRCON

Treat-**I**ron-**R**elated-**C**hildhood-**O**nset **N**eurodegeneration

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

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

277984-2

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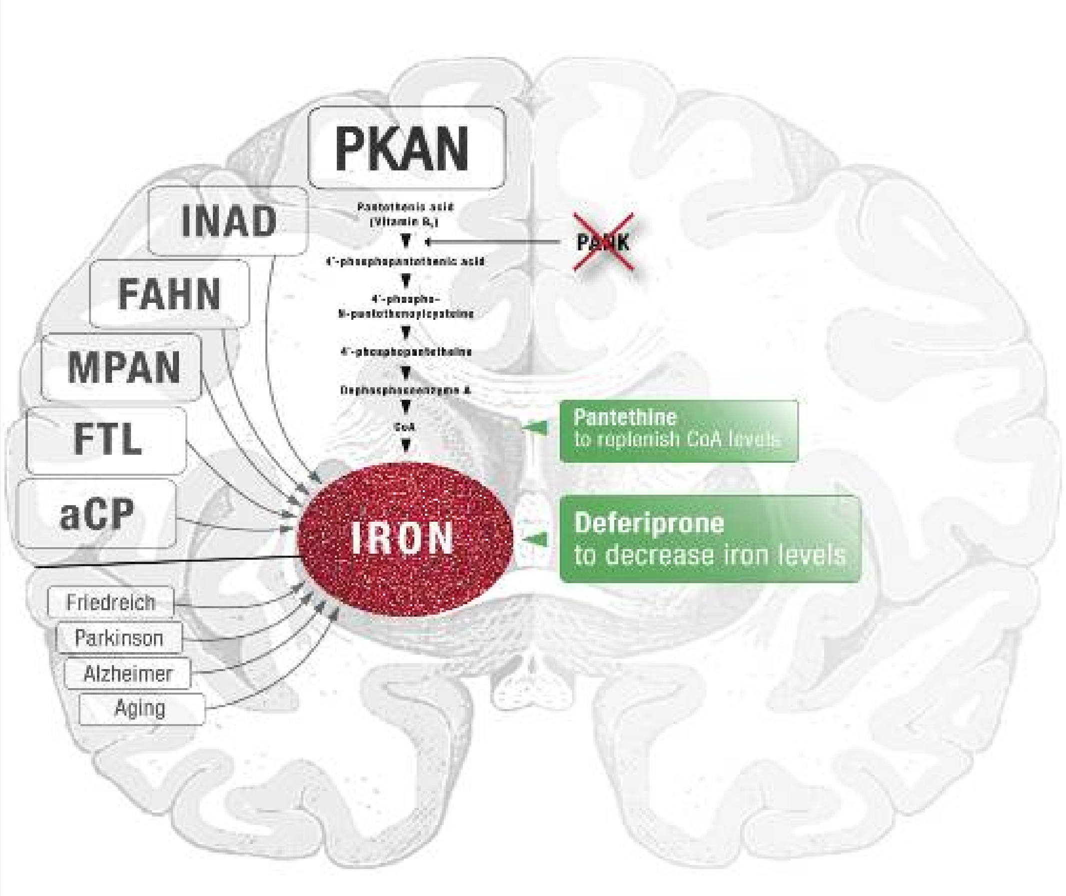
SEVENTH FRAMEWORK PROGRAMME

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

Ludwig-Maximilians-Universität München

Children's Hospital Oakland, USA

Technische Universität München

Newcastle University, England

Children's Memorial Health Institute, Polen

ACIES BIO, Slowenien

Foundation Neurological Institute Besta, Italien

NBIA Disorders Association, USA

University Medical Centre Groningen, Niederlande

Hoffnungsbaum e.V., Deutschland

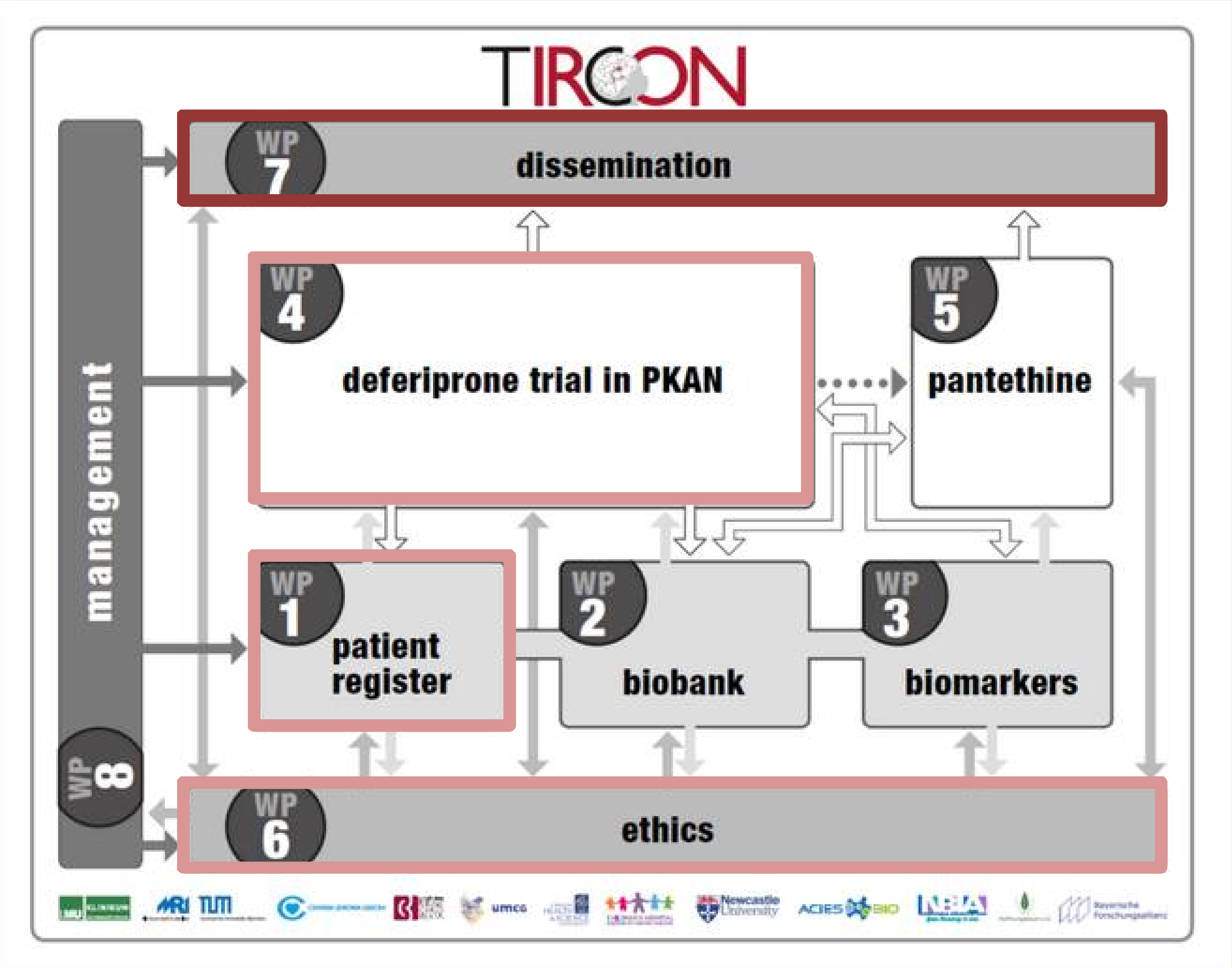
Oregon Health & Science University, USA

Bayerische Forschungsallianz GmbH

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

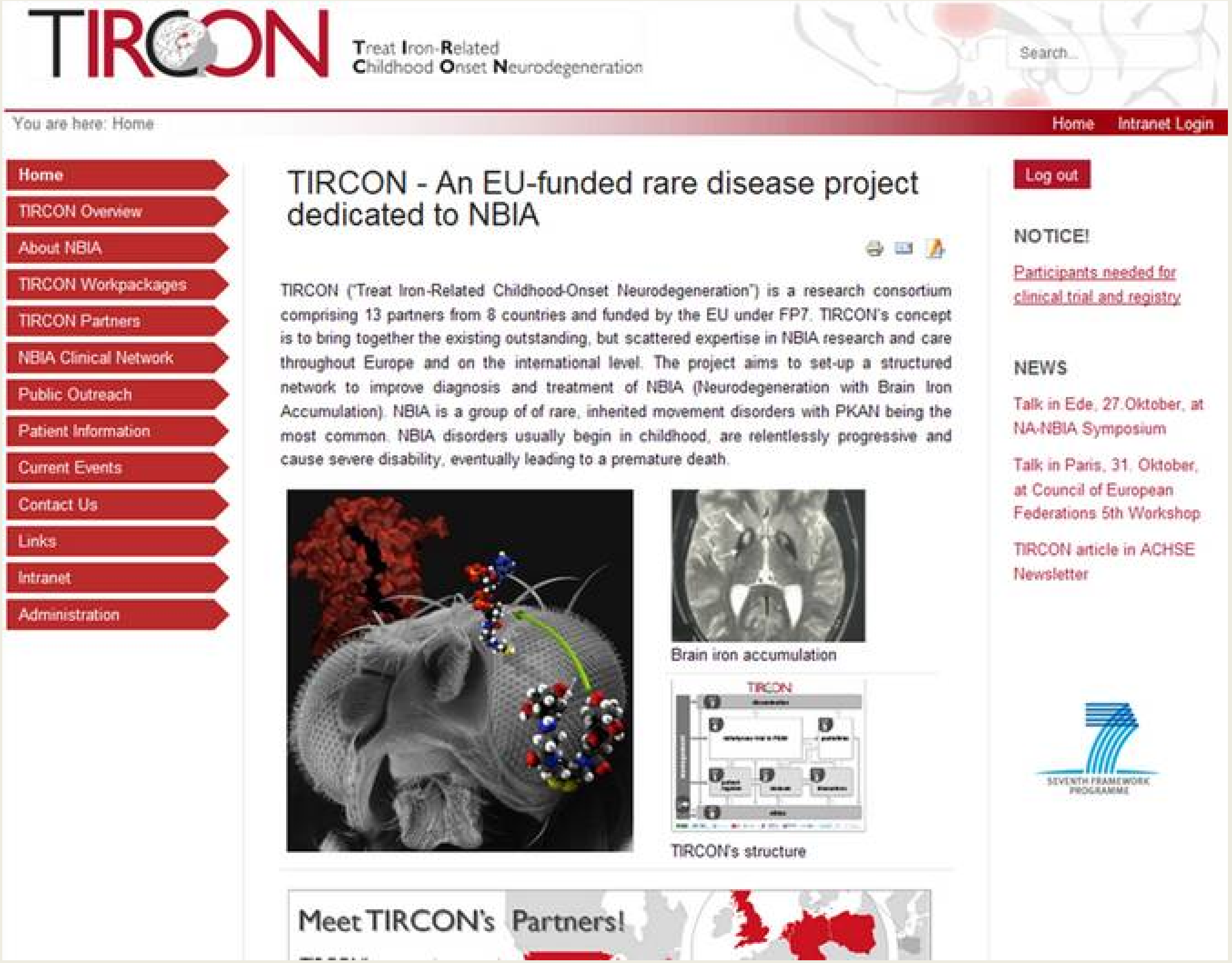
TIRCON‘s structure



Work package 7-Dissemination

Tasks
7.1: Networking and communication plan **7.2:** NBIA Network in Europe and USA **7.3:** Exploitation plan **7.4:** Dissemination of research results **7.5:** Teaching PKAN/NBIA **7.6:** Steps towards further clinical trial

7.1 Networking and communication plan



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TIRCON - An EU-funded rare disease project dedicated to NBIA

TIRCON ("Treat Iron-Related Childhood-Onset Neurodegeneration") is a research consortium comprising 13 partners from 8 countries and funded by the EU under FP7. TIRCON's concept is to bring together the existing outstanding, but scattered expertise in NBIA research and care throughout Europe and on the international level. The project aims to set-up a structured network to improve diagnosis and treatment of NBIA (Neurodegeneration with Brain Iron Accumulation). NBIA is a group of of rare, inherited movement disorders with PKAN being the most common. NBIA disorders usually begin in childhood, are relentlessly progressive and cause severe disability, eventually leading to a premature death.

Brain iron accumulation

TIRCON's structure

Meet TIRCON's Partners!

NOTICE!

Participants needed for clinical trial and registry

NEWS

Talk in Ede, 27.Oktober, at NA-NBIA Symposium

Talk in Paris, 31. Oktober, at Council of European Federations 5th Workshop

TIRCON article in ACHSE Newsletter

7.2: NBIA Network in Europe and USA

7.4: Dissemination of research results

Letter to the Editor

An international registry for neurodegeneration with brain iron accumulation

Bernadette Kalman, **Ronald Lautenschlaeger**, **Florian Kohlmayer**, **Boriana Büchner**, **Tomasz Kmiec**, **Thomas Klopstock** and **Klaus Kuhn**

For all author emails, please log on.

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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 vor 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

AISNAF ONLUS
ASSOCIAZIONE ITALIANA
SINDROMI NEURODEGENERATIVE
DA ACCUMULO DI FERRO

NBIA
DISORDERS ASSOCIATION
from discovery to cure

Hoffnungsbaum e.V.

ENACH
Asociación

NBIA
ALLIANCE
Worldwide Partners
for a Cure

EURORDIS
Rare Diseases Europe

achse
Allianz Chronischer Seltener Erkrankungen

KINDERNETZWERK
WIR HELFEN WEITER

Thanks for your attention!

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