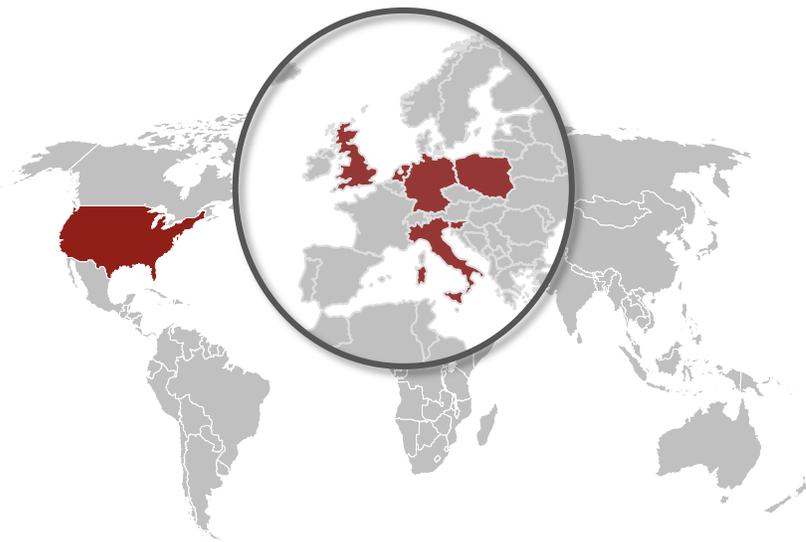


TIRON

Treat Iron-Related Childhood-Onset Neurodegeneration



Information leaflet

FP7 (277984-2)



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

Treat Iron-Related Childhood-Onset Neurodegeneration (TIRCON) 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.

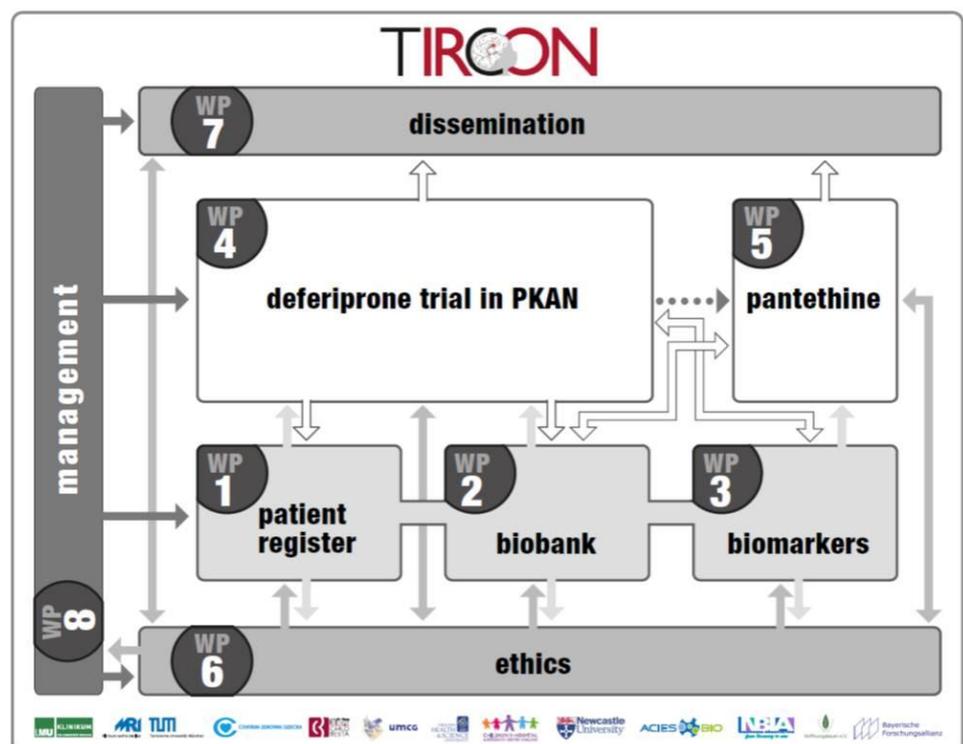
TIRCON's Focus

Neurodegeneration with Brain Iron Accumulation (NBIA) is a clinically and genetically heterogeneous group of rare hereditary neurodegenerative disorders characterized by high levels of brain iron. Many NBIA cases are characterized by early childhood onset and rapid progression to disability and death. The most frequent form of NBIA is Pantothenate Kinase-associated Neurodegeneration (PKAN). Currently, there is no proven therapy to halt or reverse PKAN or any other form of NBIA. This is especially unfortunate as both the iron accumulation in NBIA and the biochemical defect in PKAN are predicted to be amenable to drug-based treatment. Thus, the absence of adequately powered randomized clinical trials is not due to a lack of therapeutic options but to the rarity of the disease, the lack of patient registries and the fragmentation of therapeutic research worldwide.

In TIRCON, for the first time, an international group of scientists and clinicians have elaborated a collaborative project with patient representatives and innovative companies committed to orphan products.

TIRCON's Structure

Each partner institute has specific duties in the one or more of the 8 work packages of TIRCON.



TIRCON's goals are:

1. Setup of an international NBIA registry
2. Implementation of a biobank
3. Development of biomarkers for the disease
4. Conduction of a randomized clinical trial of the iron-chelating drug Deferiprone in PKAN
5. Preclinical development of pantethine derivatives for the treatment of PKAN

TIRCON's Impact

A coordinated international initiative, TIRCON will set up a framework for improvements in clinical practice and research with the following main impacts to be expected:

- *Structural impact:* Through the cooperative effort of the major medical NBIA centers in Europe and the United States, NBIA patient advocacy groups and the Biotech industry, TIRCON will bring together patients and patient data, scientific and clinical expertise, and participation of Biotech industry for the first time in a structured, well networked form.
- *Scientific impact:* Structuring research in the field of NBIA at an international level will ensure sufficient patient cohorts for statistical evidence and for the generation of solid scientific results in order to create new knowledge on NBIA, transferable into more appropriate drug therapy strategies through a strong cooperation with industry.
- *Economic impact:* SME participation in TIRCON will increase innovation and competitiveness of European health-related industries.
- *Health care impact:* Engaging in a dialogue with public and private health bodies in health care system will translate the scientific results into clinical therapy and health care strategies.
- *Social impact:* TIRCON shall contribute to an improvement of the therapeutic options for a progressive, disabling and often life threatening disease and lead to a distinctive improvement of the infrastructure for NBIA patients.

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TIRCON Partners:



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**Children's Hospital Oakland,
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umcg

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Klinikum rechts der Isar



Technische Universität München

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**Newcastle University,
England**



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University,
USA**



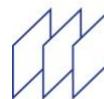
ApoPharma, Canada



**Children's Memorial Health
Institute, Poland**



**ACIES BIO,
Slovenia**



**Bavarian
Research Alliance**

**Bayerische Forschungsallianz
GmbH, Germany**



**Foundation Neurological
Institute Besta, Italy**



**NBIA Disorders Association,
USA**



Hoffnungsbaum e.V.

**Hoffnungsbaum e.V.,
Germany**