



Press release

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EU funding in the millions enables international consortium to research rare neurodegenerative disease

Coordinated by Prof. Dr. med. Thomas Klopstock (Friedrich-Baur-Institute at the Neurological Department of the University Hospital at Ludwig-Maximilians University Munich - LMU) and with support from the Bavarian Research Alliance BayFOR (Dr. Florence Gauzy), the international consortium TIRCON succeeded in attracting an EU Research Grant in the amount of 5.2 Million Euros over 4 years to investigate a rare neurological disease. TIRCON ("Treat Iron-Related Childhood-Onset Neurodegeneration") is dedicated to a group of disorders called "Neurodegeneration with Brain Iron Accumulation" (NBIA). NBIA is characterized by high iron levels in the brain. This neurological disease usually has a very severe course, with onset in childhood in the majority of cases. There is still poor knowledge about NBIA primarily because it is so rare, that collecting significant data is a challenge. The grant awarded by the Seventh Research Framework Programme of the European Union now allows new ground to be broken in the treatment of NBIA. All in all, there are 12 project partners from 7 countries involved in TIRCON.

The core project of TIRCON is a multicenter, clinical therapy study with the iron-chelating agent Deferiprone, which will be conducted at 6 clinical centres in Europe and Northern America. The Canadian company ApoPharma, who manufactures Deferiprone, is supporting TIRCON with the realization of this important study. Further objectives of the project include the setup of an international patient registry and a biobank, as well as preclinical work to develop a biomarker for the disease and to investigate new therapeutic options in animal models. The latter takes place in close cooperation with the innovative Biotech enterprise ACIES BIO in Ljubljana (Slovenien). In the overall project, TIRCON will build on the intense preliminary work of the participating centres and rely on the collaboration of many basic researchers, clinicians and patient advocacy representatives, Hoffnungsbaum e.V. in Germany and NBIA Disorders Association in the USA.

"Through this multi-track approach, we hope to improve the situation of the patients in the near future", says Prof. Klopstock. "The upcoming clinical study with Deferiprone shows the immediate capability to achieve a slowdown or even an improvement of disease progression. The other TIRCON modules, like the setup of a clinical network, a patient registry and a biobank as well as the identification of biomarkers and

of further therapeutically relevant substances, shall lead to a distinctive improvement of the infrastructure for NBIA patients within the next years”, said Klopstock.

The project Kick-off Meeting took place in Munich from the 19th to 21st January 2012. With the transfer of the EU funding to the partners, the LMU has now signalled the final go-ahead for TIRCON.