Press release

Date: March, 13th 2012

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

Coordinated by Prof. Dr. 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 (Treat Iron-Related Childhood-Onset Neurodegeneration) was awarded an EU Research Grant in the amount of 5.2 Million Euros over 4 years to investigate NBIA (Neurodegeneration with Brain Iron Accumulation), a rare neurological disease. NBIA is characterized by high iron levels in the brain with onset in childhood in the majority of cases. A rare, inherited, neurological movement disorder, NBIA is characterized by the progressive degeneration of the nervous system. Because NBIA is so rare, there is still little known about it, making collection of significant data a challenge. The grant, awarded by the Seventh Research Framework Programme of the European Union, allows new ground to be broken in the treatment of NBIA. Twelve project partners from seven countries are involved in TIRCON.

The core project of TIRCON is a multicenter, clinical therapy study with the iron-chelating agent Deferiprone that will be conducted at six clinical centers in Europe and Northern America. ApoPharma, the Canadian company that manufactures Deferiprone, is supporting TIRCON in 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, Slovenia. TIRCON will build on the intense preliminary work of the participating centers and rely on the collaboration of many basic researchers, clinicians and patient advocacy groups 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 of January 2012. With the transfer of the EU funding to the partners, the LMU has now signalled the final go-ahead for TIRCON.

Rare disease researchers at Oregon Health & Science University are part of the consortium. OHSU's Susan Hayflick, M.D., is one of leading researchers in the world on NBIA, having studied the group of diseases for almost two decades. Her OHSU research laboratory, in concert with researchers at the University of California at San Francisco and at the University of Birmingham and University College London, discovered the three genes that cause NBIA, in 2001, 2006 and 2010.

Beyond Hayflick's work, OHSU is one of the top institutions in the United States doing research on rare diseases. It has more than 60 scientists and doctors studying more than 90 rare disorders.

Contact

- **OHSU Principal Investigator, Susan Hayflick, M.D., ph. 503-494-7703**