Project Title: Quantitative description of the clinical course of Canavan disease

Canavan leukodystrophy is a rare degenerative brain disease of childhood. Caused by a metabolic defect of the brain the white matter (myelin) deteriorates and causes severe disabilities and early death of affected children. Diagnosis can be made e.g. by proof of elevated NAA (N-Acetyl-Aspartate) in blood, urine, MRS or by molecular genetic testing. Until today no treatment exists to cure Canavan disease. Various study approaches are focused on gene therapy or usage of disease modulating substrates. To link the knowledge out of the laboratories toward the patient's improvement of their health status detailed knowledge of the natural course of the disease is necessary.

The funding of 2014 MDBR together with funding by the Myelin Project Germany enabled collection and analysis of 22 Canavan patients in the Hamburg Leukodystrophy database. As Canavan is a rare disease cooperation of researchers and physicians who care for Canavan patients is important. Cooperation of researchers in Hamburg, Boston and New York was initiated. Based on the Canavan database that was developed in Hamburg during the European LeukoTreat project the team of European and American researchers develop a standardized and harmonized database structure. This will enable the team to analyse data of the Hamburg Leukodystrophy database and the NeuroBANK™ database together in the future.

Hamburg, 14th of February 2016 Annette Bley, MD