



Press release

Date: June, 17th 2016

Orphan drug designation granted

The European Medicines Agency (EMA) has granted orphan drug designation to a new candidate compound CAB1803 for the treatment of pantothenate kinase-associated neurodegeneration (PKAN), developed by TIRCON partners Acies Bio, a Biotech enterprise in Slovenia and University Medical Centre Groningen (UMCG).

The preclinical development of panthethine derivatives was one of the crucial achievements of the research consortium TIRCON (Treat Iron-Related Childhood-Onset Neurodegeneration). Abnormal Coenzyme A levels are thought to be one of the primary defects in PKAN and they might be restored with the new identified candidate.

After filing of a patent application covering the compound and its synthesis by TIRCON partners UMCG and Acies Bio (EP2868662, WO2015063177), the [recognition of EMA and the European Commission](#) has been the next step towards a clinical trial in order to prove a beneficial effect and normalize primary deficiencies in PKAN.

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