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INTRINSIC ACTIVITY

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MEETING ABSTRACT

A1.8

Developing new therapies for rare Batten disease

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The neuronal ceroid lipofuscinoses (NCLs), also referred to as Batten disease, denote fatal genetic lysosomal storage disorders that are the most common of the rare neurodegenerative childhood diseases, affecting approximately 14,000 world-wide. It is a devastating and severely debilitating group of genetic diseases. There are no curative treatments yet offered in the clinic for any type of NCL anywhere in the world. The EU-funded Horizon 2020 consortium BATCure aims to develop the first effective treatments for three genetically distinct NCL subtypes caused by mutations in intracellular transmembrane proteins. The goal of BATCure is to provide and test novel therapeutic leads, and to increase our understanding of the underlying biochemical and molecular basis of Batten disease, to use this knowledge to design new therapeutic options and develop new tools for diagnostics and the monitoring of treatments. BATCure partners are driving concerted activities to create new models, tools and technologies for developing and testing therapies, further delineate disease biology and gene function to identify new therapeutic target pathways, facilitate effective evaluation of preclinical therapies and improve diagnostics, extend a comprehensive natural history beyond the brain to include metabolic changes, identify new and repurpose existing small-molecule therapy, triage new compound treatments in zebrafish and mouse models. The Latvian Institute of Organic Synthesis will be involved in the medicinal chemistry and innovative drug discovery BATCure activities aiming to improve ADME/tox profile and brain bioavailability of active compounds, as well as participate in the preclinical drug development. In addition to novel compounds, also hits from FDA collection of 1,500 compounds, after validation in secondary assays and *in vivo*, could be immediately tested in patients to allow repositioning of known drugs as correctors of Batten disease. BATCure provides a mechanism to involve patients and their families to inform and fully contribute to therapy development and prepare for clinical trials. By the end of the project, BATCure expects to have a lead therapy ready for a clinical trial and developed faster diagnostics suitable for pre-symptomatic testing and monitoring efficacy of new treatments.

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Keywords: neuronal ceroid lipofuscinoses – rare disease – Batten disease – drug discovery and development

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