

preclinical study for functional restoration in megalencephalic leukoencephalopathy with subcortical cysts (MLC)

Objectives Evaluate the impact of functional restoration as a therapeutic strategy for MLC.

Budget €188,000

Duration 2 years

Description The functional deficiency of the MLC1 protein leads to MLC. The project aims to evaluate the effect of gene therapy restoring MLC1 functionality in a mouse model.

Project partners: Dr Martine Cohen-Salmon, Interdisciplinary Center for Research in Biology (CIRB), Collège de France, Paris, and Dr Denis Vivien, Caen Normandie University, France.

Impacts Introducing a novel therapeutic approach using gene therapy for patients affected by MLC.

