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PROGRESS REPORT 2021-2022

"Preclinical studies in the SACS KO mouse model of ARSACS"

Earlier studies with mouse models and cells from ARSACS patient suggested that mitochondrial shape changes (dynamics) may be a driver of ARSACS pathology. We addressed this hypothesis by genetically increasing or decreasing the activity of the mitochondrial fission enzyme Drp1 (dynamin-related protein 1) in a mouse model of ARSACS. Neither manipulation changed the time course of disease progression. Our data indicate that intervention into the mitochondrial fission/fusion equilibrium is unlikely to be of therapeutic benefit and that other targets should be considered in the treatment of ARSACS.

August,2023