

Title (en)

METHODS OF TREATING MITOCHONDRIAL DISORDERS

Title (de)

VERFAHREN ZUR BEHANDLUNG VON MITOCHONDRIALEN ERKRANKUNGEN

Title (fr)

MÉTHODES DE TRAITEMENT DE TROUBLES MITOCHONDRIAUX

Publication

EP 4121120 A2 20230125 (EN)

Application

EP 21771515 A 20210311

Priority

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Abstract (en)

[origin: WO2021188349A2] Provided herein are methods for treating a disease or disorder associated with mitochondrial dysfunction through ex vivo introduction of a nucleic acid molecule into hematopoietic stem and progenitor cells (HSPCs) followed by transplantation of the HSPCs into a subject in need of treatment. The nucleic acid molecule may include a functional human frataxin (hFXN) or may include a gene editing system that when transfected into the cells removes a trinucleotide extension mutation of endogenous hFXN.

IPC 8 full level

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