GENE THERAPY FOR GLUTARIC ACIDURIA

A research group from CIBER, IDIBAPS and Hospital Clinic de Barcelona have identified a novel gene therapy approach for the treatment of Glutaric Aciduria type I (GA-I).

The Need

Glutaric Aciduria type I (GA-I), classified as an **orphan disease**, is a genetic **metabolic disorder** caused by a deficiency in glutaryl-CoA dehydrogenase (GCDH), a key enzyme for the **metabolism of lysine, hydroxylysine and tryptophan**. The altered GCDH activity causes the development of a **complex movement disorder and premature death.**

The Solution

A **gene therapy** strategy has been developed based on the **administration of the GCDH gene.**

Innovative Aspects

- GA-I is currently treated by **dietary lysine restriction and carnitine supplementation**. Unfortunately, almost **one-third** of affected children poorly respond to therapy and experience striatal degeneration despite careful clinical management, clearly showing the need to develop more effective therapies.

Stage of Development:

Pre-clinical assays have been performed with mice models and showed promising results to accomplish clinical trials.

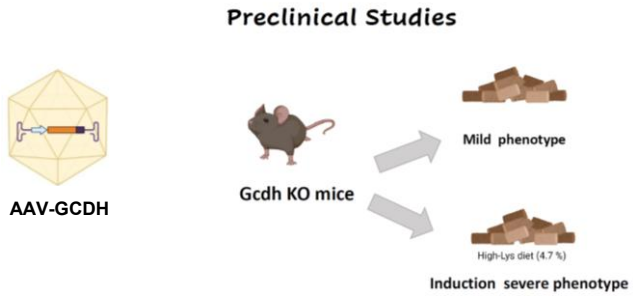

Fig 1. Basis of the preclinical studies performed. Severe phenotype in KO mice and scheme of AAV used.

Fig 2. Developmental stage of the project

Intellectual Property:

- Orphan Drug Designation (EMA): EMAOD0000171786

Aims

Looking for a partner interested in a license and/or a collaboration agreement to develop and exploit this asset.

Contact details

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