GenGA

Gene Therapy for Glutaric Aciduria

👉 Treatment for **Glutaric Aciduria type I** (GA-I) based on **gene therapy**.

CLINICAL NEED

Glutaric Aciduria type I (GA-I), classified as an **orphan disease** (1:100.000), 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 a development of a **complex movement disorder and premature death.** It is detected during the **heel prick test**.

SOLUTION

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

COMPETITIVE ADVANTAGE

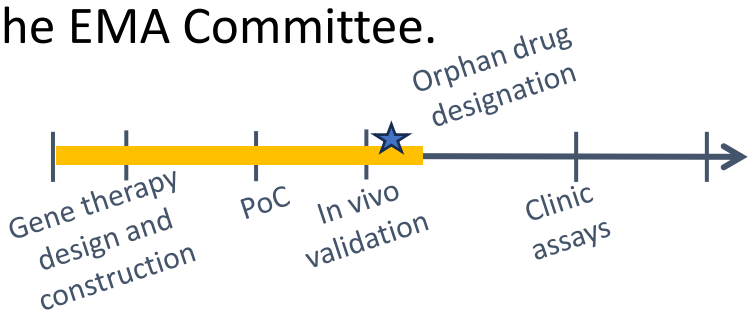
GA-I is currently treated by **dietary lysine restriction and carnitine supplementation**. Unfortunately, almost **one-third** of affected children poorly respond to therapy and experience striatal degeneration despite careful clinical management, clearly showing **the need of a development of more effective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

INTELLECTUAL PROPERTY

European patent (EP23382397) application was submitted 27 of April 2023. Hospital Clínic de Barcelona, CIBER and FRCB-IDIBAPS share joint ownership. **EESR and ISR** have been positive.

DEVELOPMENT

Pre-clinical assays have been performed with mice models and showed **promising results to accomplish clinical trials.** Moreover, has **received Orphan Drug Designation from the EMA**, following a positive opinion from the EMA Committee.



LOOKING FOR...

Partners for **license agreement** or **co-development.**

THE TEAM

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