May 2023

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**, 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.**

SOLUTION

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

COMPETITIVE ADVANTAGE

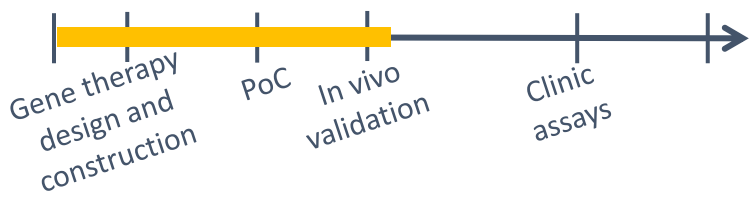
GA-I is currently treated by **dietary lysine restriction and carnitine supplementation**. Unfortunately, almost **one-third** of affected children poorly respond to therapy and experience striatal degeneration despite careful clinical management, clearly showing **the need of a development of more effective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

INTELLECTUAL PROPERTY

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

DEVELOPMENT

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



LOOKING FOR...

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

THE TEAM

Dr. Cristina Fillat
Group Leader

Dr. Antonia Ribes
Emeritus Researcher

Dr. Judit Garcia
Accredited Researcher

Eulalia Segur
Researcher

Anna Mateu
Researcher

CONTACT DETAILS
Knowledge and Technology Transfer Office
innova@recerca.clinic.cat