

Solve GNE Raises \$2.5 Million, Secures Leading Researchers to Help Develop Cure for HIBM

August 1, 2023 – Solve GNE, a Los Angeles based non-profit organization, has raised over \$2.5 million and negotiated sponsored research agreements with multiple leading medical researchers to help develop a cure for Hereditary Inclusion Body Myopathy (HIBM).

Also known as GNE Myopathy (GNEM), HIBM is a muscular disorder that causes progressive muscle weakness which manifests in early adulthood – generally starting with the legs – until reaching eventual complete loss of mobility. While rare, this disease has a remarkably high incidence among Iranians of Jewish descent – with an estimated 10 to 15% of the population carrying at least a single copy of the mutated gene.

Solve GNE has been actively raising funds to support a consortium of academic and private sector researchers tasked with the development of various gene therapies. Solve GNE has committed to funding experts in HIBM in four leading organizations: Genosera, Gradalis, Johns Hopkins University School of Medicine and Stanford University. These agreements include detailed development budgets and a commitment from Genosera and Gradalis to start human clinical trials within 12 to 18 months.

The Solve GNE consortium is focusing on a common goal but implementing a parallel track strategy that should allow them to approach the FDA with multiple options to get patients enrolled in Phase 1 trials as rapidly as possible.

Each consortium participant brings a unique perspective to the collaboration, and Solve GNE funding will be allocated accordingly to the following projects:

- [Genosera](#), an Ohio-based biotechnology startup, is an offshoot of [Nationwide Children's Hospital](#), the leading gene therapy research and development center. Co-founders, Dr. Gidon Akler (Aghlar) – a geneticist from the Iranian Jewish community in Great Neck, NY – and Dr. Paul Martin – a pioneer in gene therapy – are leading experts in HIBM. Dr. Martin developed Genosera's "bicistronic technology" – a proprietary gene therapy that combines a correct copy of the GNE gene with an additional muscle growth factor gene to help build back muscle. This process incorporates a novel AAV delivery system, which has been successfully applied in patients with other muscular diseases. The company's platform includes cutting-edge HIBM cellular and animal models that are critical for the approval of an Investigational New Drug (IND) application, the gateway to human clinical trials. Genosera's bicistronic technology is the only gene therapy that is designed to stop the progression of the disease and allow patients to regain their physical capabilities. The approach was designed to be broadly applicable to patients at any stage of disease progression.

- **Gradalis, Inc.**, an established Texas-based biotechnology company, specializes in novel genetic therapies for cancer. In 2010, Gradalis co-founder and chief scientific officer, Dr. John Nemunaitis, carried out the first and (currently) only gene therapy clinical trial for HIBM in a single patient. Published data showed activity in this early form of HIBM gene therapy that was well tolerated. In addition, there were measurable improvements in certain muscle groups. Financial commitment from Solve GNE will allow dosing of additional patients using an improved liposomal formulation of the original therapy. A successful outcome will stop the progression of the disease and offer an alternative to patients who may not qualify for AAV based gene therapies.
- **Johns Hopkins University School of Medicine** researchers spearheaded by [Dr. Greg Newby](#) are the recipients of a multi-year grant from Solve GNE. Using a novel precise CRISPR therapy known as prime editing – a pioneering technology developed over the past five years by Harvard Professor David Liu – the team will attempt to directly correct the genetic point mutation in laboratory models. If successful in future clinical trials of patients, the approach could potentially be most useful for younger patients who are not yet showing any disease symptoms.
- **Stanford University** researchers led by gene therapy experts [Dr. Natalia Gomez-Ospina](#), [Dr. John Day](#) and [Dr. Fraser Wright](#), have developed a sophisticated MRI technique for analyzing the skeletal musculature of HIBM patients with stunning detail, allowing the researchers to follow the progression of the disease more accurately during clinical trials. Additionally, the team is developing a novel Adeno-Associated Virus (AAV) delivery system, which is safer and more efficient than current gene therapy viral delivery systems, potentially allowing a larger percentage of patients to qualify for an HIBM viral gene therapy.

Additionally, Solve GNE has already identified Nationwide Children's Hospital in Ohio as their primary clinical site. Stanford has agreed to serve as the secondary clinical site on the West Coast. Solve GNE is still researching alternative clinical sites in Los Angeles and New York in order to facilitate enrollment of patients who live in those cities.

Additional details will be provided by Solve GNE and researchers from Stanford, Johns Hopkins, Gradalis and Genosera during a global webcast presentation followed by a Q&A session on Sunday September 10th, 2023 at 11A New York time (8A Los Angeles time, 6P Tel Aviv time). For those who are unable to attend the live presentation, a recording of the webcast will be made available.

For more information and a link to the webcast, please visit www.solvegne.org or follow them on Facebook.