Elpida Therapeutics Announces Two Programs to be Advanced in Partnership with Bespoke Gene Therapy Consortium

Two of Elpida's pipeline programs for ultra-rare diseases, SPG50 and CMT4J, will get a significant boost having been included in the BGTC program portfolio.

Elpida Logo

LOS ANGELES, CA, USA, May 17, 2023 /EINPresswire.com/ -- After recently announcing the launch of Elpida Therapeutics (Elpida Tx), The Company has now announced that two of its 5 pipeline programs have been selected to be developed in partnership with the Bespoke Gene Therapy Consortium (BGTC) which is part of the Accelerating Medicines Partnership® (AMP® BGTC) at the Foundation for the National Institutes of Health (FNIH).

Managed by the FNIH, AMP® BGTC is a public-private partnership between the National Institutes of Health (NIH), U.S. Food and Drug Administration (FDA), biopharmaceutical and life science companies, and non-profit and other organizations. Elpida Tx has been voted in as a member of this esteemed consortium of companies.

Yesterday, at the American Society of Gene and Cell Therapy (ASGCT) Annual Meeting in Los Angeles, CA, the Foundation for the National Institutes of Health announced the AMP® BGTC selection of eight rare diseases for their BGTC clinical trial portfolio. Elpida Tx is pleased that two of its 5 programs were selected for this initial cohort. “The BGTC is excited to work with Elpida Therapeutics, a BGTC affiliated partner, that has taken on CMT4J and SPG50 which enabled the consortium to include 2 additional diseases in the portfolio,” said Brad Garrison BGTC Program Manager.

The selections for BGTC portfolio programs were made based on rigorous scientific and technical review by a panel of gene therapy and rare disease experts. 62 programs were nominated for the BGTC and after 18 months of extensive review and RFPs, 8 programs were selected for advancement in the program.

“We are thrilled to have two of Elpida’ s pipeline programs included in the AMP® BGTC announcement,” said Terry Pirovolakis, CEO of Elpida Tx. “This partnership will be key to advancing Elpida’s neurological programs for SPG50 and CMT4J so that we can not only develop a therapeutic for these ultra-rare diseases with significant unmet medical need, but so we can change and improve the development pathway that will allow us to best ensure equitable and affordable access for all treatable patients . The partnership with BGTC will pave the way in proving these pathways and will catalyze hope for patients and families with no other options.”

Elpida Tx was established with the goal of bringing a new approach to successfully address the hurdles related to gene therapy development for patient populations with significant unmet needs. As a Social Purpose Corporation, Elpida Tx looks to combine business sustainability balanced with broader socially responsible objectives. Elpida works with patient foundations to advance programs that would otherwise lag behind due to lack of financial interest and small patient populations.

"Elpida’s CEO, Terry Pirovolakis, was a Rare Disease Dad before he was a CEO. He understands the numerous challenges of bringing treatments forward for ultra-rare diseases--a rare gift that has shaped Elpida's business model and approach to ultra-rare disease drug development," said Jocelyn Duff, co-founder and Executive Director of CureCMT4J, and Mom to Talia, who has CMT4J. "Elpida feels more like a true partner, sharing urgency and alignment in our desire to ensure affordability and access to patients around the world. I am confident that this collaboration will advance our gene therapy program and bring immeasurable hope to our patient community."

Elpida Tx was launched with cash and in-kind funding commitments of over $20 million and has a significant cadre of partners and funders with the aim of expanding its partners for its operations in the near future. The BGTC announcement exemplifies the caliber of partners Elpida’ s model and mission is attracting.

More than 30 million people in the United States and over 400 million people worldwide live with the devastating effects of rare diseases. There are more than 10,000 rare diseases and it is estimated that more than 80% are caused by genetic defects. Gene therapy treatments are well suited as a potential treatment option for many of these diseases where there is currently no available treatment.

About Elpida Tx: Elpida’ s mission aims to address the current significant unmet medical needs of patients with ultra-rare diseases. Through leveraging scientific advancements and the now well-established safety and understanding of certain gene therapies, Elpida aims to put cures in reach of families and children who desperately and urgently need them. Elpida’ s business model focuses on partnerships that promote efficiency and the chance to treat a greater number of patients, while being self-sustaining and replicable. Elpida Tx specifically focuses on advancing programs that traditional biotech companies find difficult to bring through to completion. The company pipeline includes programs that were deprioritized by biotech companies or that did not receive biotech investment to advance due to small patient populations, but which have

excellent scientific data on which to build a program and springboard the science and opportunity. Elpida Tx plans to incorporate two more CNS programs into its initial program pipeline within the next six months through an application process designed for academic institutions and foundations.

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