Dear Lafora Families,

My name is Terry Pirovolakis. On April 2, 2019, my son Michael was diagnosed with SPG50, a devastating genetic disease. We were told to take him home, love him, he will never walk, never talk, have limited brain function and will not live an average lifespan, cherish the time we had because there was nothing that could be done. Refusing to accept that answer, our family worked with incredible scientists and physicians around the world to develop a gene therapy specifically for Michael. On March 24, 2022, he became the first child in the world to receive that treatment.

That experience changed my life. I made a promise that if we could save Michael, we would do everything possible to help other children facing devastating genetic diseases. That promise led to the creation of **Elpida Therapeutics**, a non-profit biotechnology organization dedicated to developing treatments for children with ultra-rare diseases. Since then, we have treated 14 children across two diseases, and this year we expect to treat more than 36 children across the United States, Europe, and the United Kingdom through our SPG50, CLN7, and CMT4J programs and hopefully a BLA application for SPG50 in 2028.

Ever since meeting children and families affected by Lafora disease, our team has quietly been exploring every possible way to help. Several months ago, we learned that ION283 had been returned to Ionis. Like many of you, we hoped another company would continue developing the program through a formal clinical trial. Unfortunately, given the current biotech environment and the lack of commercial interest in ultra-rare diseases, that did not happen. This is a reality our own SPG50 program has faced many times.

We partnered with **Fundación Columbus** and **Sant Joan de Déu Barcelona Children's Hospital** to create a path forward. Together with the exceptional work being led by Dr. Berge Minassian and his team, our immediate goal is to initiate a clinical trial evaluating the safety and efficacy of investigational ION283 in individuals living with Lafora disease and advance the program as quickly and responsibly as possible.

At the same time, we are working closely with physicians, researchers, hospitals, regulators, and funding partners around the world to advance the clinical development of investigational ION283 and support the successful execution of the clinical trial.

Many of you will ask why this cannot happen sooner. The answer is simply that we must balance urgency with safety. The current supply of drug expires in August 2027 and can only be used for the patients already receiving treatment at The University of Texas Southwestern Medical Center (UT Southwestern). A new manufacturing batch is now being produced, but it will not be available until approximately the end of the first quarter of 2027. In parallel, Dr. Minassian is working closely with the FDA to identify the fastest and safest path to dose escalation so we can determine the most effective dose for future patients.

Once the new batch is available, our greatest challenge will likely not be drug supply. We expect to have enough drug to treat approximately 200 patients over five years. The challenge will be funding the clinical programs, hospital costs, monitoring, regulatory requirements, and patient care needed to deliver those treatments. We are actively pursuing grants, philanthropic support, and partnerships to maximize the number of children we can help and sites to treat patients.

We also want to be transparent about an important decision. Until the new batch has been manufactured and additional strong, robust safety data have been generated, we will **not** be expanding treatment through compassionate use or into broader, more advanced patient populations. We know this will be disappointing for many families, but we believe it is the most responsible path to ensure the program can continue safely and successfully for the entire community without potentially halting the entire program

As we begin this next chapter following the transfer of the ION283 program from Ionis, we are committed to communicating openly, honestly, and transparently with our community. We will provide updates at key milestones, including manufacturing progress, regulatory and clinical developments, site activation, and other meaningful program advancements. There will undoubtedly be difficult decisions ahead, but we firmly believe that every family deserves clear, timely, and transparent communication at every step of this journey.

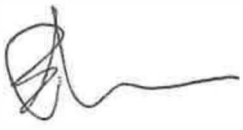
We would like to thank Ionis Pharmaceuticals for their support, collaboration, and partnership in helping advance the continued clinical development of investigational ION283 for individuals living with Lafora disease.

Finally. Our vision is to generate the clinical evidence needed to advance this therapy through the regulatory process and ultimately achieve FDA approval and approvals in other countries around the world. We believe that approval is the only sustainable path to ensuring that every child diagnosed with Lafora disease has the opportunity to access treatment, not just those fortunate enough to enroll in a clinical trial. While we cannot promise that this journey will be easy or happen overnight, we can promise that we will work tirelessly alongside the patient community, physicians, researchers, regulators, and our partners until we have done everything possible to make this treatment available to every child who may benefit.

Regards
Terry Pirovolakis

Signed
Terry Pirovolakis
CEO & Founder
Elpida Therapeutics

Signed
Javier Garcia
Co-Founder
Fundacion Columbus



Signed
Dr. Berge Minassian
Professor and Chief of Pediatric Neurology
University of Texas Southwestern and
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