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FOR IMMEDIATE RELEASE

FIRST PATIENTS RECEIVE ION283 DOSING FOR LAFORA DISEASE

The groundbreaking safety study is happening at UTSW Medical Center in Dallas.

SACRAMENTO, Calif. – February 20, 2025 – The first-ever Lafora disease safety study at the University of Texas Southwestern Medical Center (USTW) will continue enrolling patients in March.

Lafora is a neurodegenerative disease affecting an estimated 250 children worldwide. It is a genetic condition in which patients cannot maintain a normal glycogen concentration, resulting in a toxic accumulation of glycogen, or Lafora Bodies, in the heart, spine, and brain. Symptoms include epilepsy, ataxia, childhood dementia, and difficulty speaking, walking, and eating. There is no cure, although the safety study is providing hope for the worldwide community.

In December 2024, the first patient received their first dose of ION283, a treatment designed to halt disease progression. In January 2025, the first patient completed their one-month check-up, and no safety issues were detected. This allowed a second patient to receive their first dose of ION283 in January. In February, the second patient completed their one-month safety check, and the safety board reviewed the data.

Thanks to approval from the safety board, the ION283 study will continue as planned. The team at UTSW may now enroll up to 10 patients in the safety study. Currently, patients three and four will begin treatment in March. The families selected for the study will receive notification from the team at UTSW when there is enough money available to enroll their loved one.

We must continue raising funds so that all 10 patients can receive treatment. The data collected from this study will help accelerate the approval process, allowing us to expand access for other Lafora patients to receive this drug. However, we need to complete the enrollment for this study first to obtain the data we need.

“Our Lafora families are facing a new sense of optimism today that their children could be saved from this terminal prognosis,” shares the President of the Board and mother, Jenifer Merriam.

“It’s what our community has been waiting so long to hear. The approval of the ION283 safety study gives us hope in fighting this horrific disease, and we are grateful to everyone who has been instrumental in getting it approved. Together, we will continue to work towards getting all 10 patients enrolled.”

So far, the Lafora community has raised more than $977,000, enough for six patients to receive treatment. To fully fund the study, we need to raise 1.5 million dollars by May 2025. We must reach our goal to build a brighter future for all affected by Lafora disease. Join us in fundraising for a cure: https://givebutter.com/LaforaSafetyStudy.

We will continue to keep the Lafora community informed about the progression of the Safety Study. Any questions about the study can be directed to Chelsea's Hope at info@chelseashope.org.

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Chelsea's Hope was created in 2007 when Linda Gerber and a small group of dedicated friends developed a website to share her daughter Chelsea's Lafora story. Today, the mission of Chelsea's Hope Lafora Children Research Fund is to improve the lives of those affected by Lafora disease and help accelerate the development of treatments. Contact info@chelseashope.org for press inquiries and any questions about the safety study.