



August 25, 2026

Dear Prion Disease Community,

We are writing to address the heartfelt inquiries regarding ION717 that Ionis has received over the past several days related to Lito Sousa. We recognize the profound impact that prion disease has on individuals and their loved ones and supporters, and the urgency to explore all options to participate in clinical research and access investigational medicines.

ION717 is an investigational medicine being studied in PrProfile, an early-stage (phase 1/2a) clinical trial that is evaluating the safety, tolerability, pharmacokinetics, and pharmacodynamics in people with prion disease. What we learn from the PrProfile study will help inform the next steps for ION717.

At this time, the PrProfile study is no longer recruiting new participants, and we anticipate that enrollment will complete soon. We will provide an additional update to the prion disease community when that milestone occurs.

The safety and efficacy of ION717 in people with prion disease have not been established and ION717 is not currently available outside the PrProfile clinical trial. For additional information about Ionis' expanded access policy, please visit the policy webpage (<https://ionis.com/patients-and-advocacy/expanded-access-policy>).

Ionis expresses sincere gratitude to all PrProfile participants, their caregivers, site investigators and staff for their contributions to the study. We also wish to express our thanks to the global prion disease community for your collaboration and support over many years to advance research for people with prion disease.

Sincerely,

The ION717 Team

The [CJD Foundation](#) and [CJD International Support Alliance](#) are community resources that provide education and support to those affected by prion disease. For more information about Ionis, visit www.ionis.com or email padvocacy@ionis.com.

ionis.com

2855 Gazelle Court
Carlsbad, CA 92010

(760) 931-9200