



April 7, 2021

Dear Prion Disease Community,

We are writing to share with you an update on our progress towards initiating clinical trials and to emphasize our commitment to developing a treatment for prion disease.

As you may be aware, in September 2020, we announced at the CJD Foundation Family Conference that we were conducting tests on a potential drug candidate and intended to initiate a clinical trial in the second half of 2021. Since then, we have identified another drug candidate that we believe will be better suited to treat prion disease, and we have decided that this is the drug we will advance toward the clinic. We anticipate initiating a clinical trial in late 2022. Our goal at Ionis is to rapidly bring the best drug forward to the patient community, and we remain committed to this. We are continuing to conduct necessary animal studies and have initiated other important activities, such as research to better understand the needs and priorities of the patient community.

We understand that it's important to communicate often with the community and we are committed to providing regular updates on our progress as this work advances. Our goal is to keep you well-informed on the clinical trial process, opportunities to engage in research on the needs and priorities of patients, the activities related to the study start up, and expected timelines for results. Going forward, we anticipate releasing a community update periodically throughout the year.

We are very grateful to all of the families who volunteer to take part in any research and drug development efforts. It is only with your support that we can advance potential new therapies towards clinical trials. We look forward to engaging with you this year as we make progress towards a treatment for those affected by prion disease.

Sincerely,  
The Ionis Prion Disease Team

For additional questions, please contact: [patients@ionisph.com](mailto:patients@ionisph.com)