December 2024

Dear Prion Disease Community,

We are writing today to inform you that Ionis' Phase 1/2a clinical trial of ION717 (NCT06153966), called PrProfile, in people with symptomatic prion disease has achieved full enrollment. The purpose of PrProfile is to evaluate the safety, tolerability, pharmacokinetics and pharmacodynamics of intrathecal (IT) delivery of ION717. While enrollment is now complete, dosing and data collection will continue for the duration of the trial.[1]

Ionis also recently updated PrProfile to include a 70-week open-label extension (OLE) period as part of the trial. Individuals who complete the double-blind placebo control period may transition into the OLE. All participants will receive ION717 during the OLE. Individuals who participate in the OLE will continue to be monitored by study doctors.[1]

Ionis recognizes the considerable time and effort each person with prion disease and their caregivers have invested and continue to invest by participating in PrProfile. Your courage and commitment to advancing medical research is truly inspiring. We recognize the urgent need for advancing treatments and remain dedicated to completing this trial as quickly as possible. Ionis will provide the prion disease community with updates as appropriate in the future.

Sincerely,
The Ionis ION717 Team

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When do you expect results from this clinical trial?

The Primary Completion Date occurs when the last person accepted into PrProfile completes study related procedures and assessments for the primary outcome measure. With the enrollment of the final person having just occurred, Ionis can estimate that the Primary Completion Date to be July 2025. Due to the complex nature of clinical development, this date is subject to change. If the completion date changes, we will provide an update on clinicaltrials.gov. [1,2]

We will share results from the clinical trial when it is appropriate to do so in the future.

Why did Ionis add an open-label extension period to PrProfile?

The addition of an OLE is common with trials like this one and is done for many reasons. In the case of PrProfile, the OLE will enable researchers to collect additional, longer-term data associated with exposure to ION717.

ION717 remains an investigational medicine only, without any established clinical evidence of safety or efficacy.

Is there a way to receive ION717 outside the clinical trial, such as for people not eligible for this clinical trial?

Evaluation of the safety and efficacy of ION717 in clinical trials is essential to establishing whether ION717 can help people diagnosed with symptomatic prion disease. For this reason, ION717 is not available outside this clinical trial. We are working diligently to conduct the clinical trials necessary to evaluate ION717 in individuals with symptomatic prion disease. For additional information about Ionis' expanded access policy, which also includes Right To Try and Compassionate Use, click here.[3]

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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.

References:

1. ClinicalTrials.gov Listing: https://clinicaltrials.gov/study/NCT06153966 (Accessed September 23, 2024)
2. ClinicalTrials.gov Glossary of Terms: https://clinicaltrials.gov/study-basics/glossary (Accessed September 23, 2024)
3. Ionis Expanded Access Policy: https://www.ionis.com/patients/expanded-access-policy/ (Accessed September 23, 2024)