



August 15, 2024

Dear Prion Disease Community,

We are writing today to inform you that recruitment has resumed into Ionis' Phase 1/2a clinical trial of ION717 (NCT06153966), called PrProfile, in people with symptomatic prion disease.

Earlier this year a global recruitment pause was instituted per the study design to allow for the collection and review of data from the first group of individuals enrolled in PrProfile before allowing additional individuals into the clinical trial. The required data collection and review has been completed and global recruitment has resumed.

Ionis is also pleased to inform you that all planned clinical trial locations for PrProfile are now active, including several additional clinical trial sites that were not part of the earlier stage of enrollment. Additional information about the trial, as well as a list of clinical trial recruitment locations, is available at ClinicalTrials.gov. Individuals interested in participating in PrProfile should speak with their doctor. Individuals may also call +1-844-221-3587 or email PrionDisease@clinicaltrialmedia.com for additional information about clinical trial locations. Questions or concerns about health, treatments or medications should be directed to your health care team.¹

We express our deepest gratitude to the people with prion disease and their caregivers who are part of the PrProfile clinical trial. Your courage and commitment to advancing medical research is truly inspiring. We recognize the urgent need for supporting the advancement of medicine and remain dedicated to completing enrollment as quickly as possible. Ionis will provide the prion disease community with updates as appropriate in the future.

Sincerely,

Rob Goldstein-Mahon
Director, Patient Advocacy, Ionis

Below is additional information you may find helpful:

Why did the recruitment pause occur?

When the clinical trial was initiated, recruitment was anticipated to be slow allowing us more time to complete the required data collection and review without pausing enrollment. Due to rapid recruitment rate, enrollment was paused. The pause was required per the study's protocol and not due to any data collected in the study or per regulatory request.

How long will it take to complete enrollment in PrProfile?

While Ionis anticipates enrollment continuing at a rapid pace, many factors can impact how long it may take to complete enrollment. For example, prion disease is a rare disorder and only those in its early stages are eligible for the trial. The clinical trial team is working closely with site investigators to complete enrollment quickly as possible and will utilize ClinicalTrials.gov to communicate updates regarding the study's status and anticipated completion dates.

It is important to note that while enrollment may be completed rapidly, the trial itself will take time to complete. Ionis is required to disclose multiple dates on clinicaltrials.gov, including a [Primary Completion Date](#) (March 2025) and a [Study Completion Date](#) (October 2025). Dates are subject to change. Updates will be made available on ClinicalTrials.gov as appropriate in the future.^{1,2}

Where can people interested in participating in PrProfile go for more information?

A person's doctor should be their primary source of information regarding whether participating in a clinical trial may be an option. Details about the clinical trial, including a full list of active recruitment locations, are available at [ClinicalTrials.gov](#). Individuals may also call +1-844-221-3587 or email PrionDisease@clinicaltrialmedia.com to learn more about recruitment sites.

Why does clinicaltrials.gov still say that recruitment is paused?

As of today, the trial is actively recruiting again. Ionis anticipates that the trial's entry on ClinicalTrials.gov will be updated soon.

Did Ionis review any data during this recruitment pause?

The required data collection and review during the recruitment pause was used to inform appropriate next steps regarding exposing additional people to the investigational medicine being tested in this study, ION717. This data review is not designed to provide any conclusions about the safety or efficacy of ION717. Data reviews of this nature are common in early-stage clinical trials like PrProfile.

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 may be able to 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 visit: <https://www.ionis.com/patients/expanded-access-policy/>

The [CJD Support Group Network Australia](#), the [CJD Foundation](#) and the [CJD International Support Alliance](#) are community resources that provide education and support to those affected by prion disease. For more information or questions please email padvocacy@ionis.com.

References:

1. <https://clinicaltrials.gov/study/NCT06153966> (Accessed August 15, 2024)
2. <https://clinicaltrials.gov/study-basics/glossary> (Accessed August 15, 2024)