Milan, 20th January 2026

Update on the ION283 Clinical Trial Pathway for Lafora Disease

Dear Members of the International Lafora Patient Community,

Following the many messages we have received from numerous families, we would like to share a few additional lines to provide greater clarity regarding what was already communicated to you on January 8th.

First and foremost, we wish to emphasize that we fully understand the suffering and sense of urgency experienced by families affected by Lafora disease. This is a situation we do not underestimate, and with regard to it we reaffirm our utmost commitment.

As we have already informed you, Noventia Pharma (an entity completely independent from Fondazione Telethon) has withdrawn abruptly from the Lafora disease project. Fondazione Telethon has taken note of Noventia's decision and has immediately sought to assess its implications: the withdrawal of a party that played a central and formal role in the previous action plan now makes it necessary to redesign the entire operational and legal framework.

It is important to clarify that the license for ION-283 is not held by Fondazione Telethon, but was returned by Noventia to IONIS. We are therefore actively engaging with IONIS to verify the conditions for a possible continuation of the clinical study project on which we have worked diligently over the past months. Any future development will depend on IONIS's decisions, as, at present, Fondazione Telethon has no formal authority to submit the protocol from which the study would originate.

We wish to highlight that the protocol on which we have worked (which, we reiterate, has not yet been submitted) provides for:

1. therapeutic continuity as an option for European patients who, having been enrolled in the U.S. study and upon completion of their participation in the

clinical trial, have derived a benefit such as to justify the continuation of treatment.

2. in addition to offering therapeutic continuity as described above, the protocol also includes a treatment arm to ensure that European patients enrolled in the U.S. study have the possibility of being treated in Italy, specifically in order to spare them the burden of long and repeated flights to the United States.

We further emphasize - in case of positive negotiation with Ionis - that we will not request any financial support from patient associations or families for the conduct of the trial or for participation in it.

Finally, we will organize meetings with patient communities in order to update them directly on developments, as soon as new information becomes available.

The sense of frustration and helplessness that Lafora families are being called upon to experience, particularly in light of the new complexities that have arisen, deeply pains us. We are working with a strong sense of urgency, with rigor, and in full compliance with the complex rules governing clinical research; however, we have not had—and will not have in the future—control over the multiple stakeholders involved and their decisions.

With kind regards,

Alessandra Camerini
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Fondazione Telethon