Thursday, 3 July 2025

Dear Noventia Pharma Team,

We are families affected by Lafora disease who are holding on to hope through your work. Your dedication to developing a treatment for Lafora means everything to us. We were excited when you shared the news of the ION283 license purchase and your intentions to create a clinical trial last year, but we are anxiously awaiting more details. We know that any future progress with ION283 will come from your company, so we kindly request a public update on what is next for the drug's development after the safety study at UTSW.

As you know, the current ION283 safety study includes only 10 children. While we are grateful this study is happening, many more families were heartbroken to learn our children could not participate. These families are now left waiting, watching their children's condition progress, and praying for the next opportunity. We are counting on Noventia to help make that opportunity possible.

For many of us, your work is the only hope we have left. Our children do not have time to wait. Lafora disease is aggressive and unforgiving—we are pleading for a chance to save our children's lives. We desperately urge Noventia to share your plans and move forward quickly with a clinical trial or expanded access program so more children can receive this potentially life-saving treatment. You have our full support in this mission, and we are willing to assist however we can.

We would greatly appreciate it if you could share any updates with us or join our community for a Q&A. Some of our questions include:

- How soon would expanded access be available for those not included in the trial?
- What will be the initial phase of the clinical trial conducted by Noventia?
- Which international sites will be involved?
- Are those sites accredited to conduct a clinical trial?

We look forward to your prompt response and a clear outline of Noventia's intentions regarding ION283. We are grateful for everything you do for the rare disease community and remain hopeful that Noventia will act swiftly to help more families in this fight against Lafora disease.

With hope,

The Lafora patient community

Patient organizations:

Asociación CEL LUZ
a.i.la. Associazione Italiana Lafora ODV
Chelsea's Hope Lafora Children Research Fund
France LAFORA Vaincre la maladie
LAFORA Corrientes - Argentina Luchemos juntos alias: lafora.corrientes
aevel
TEMPO ZERO

Lafora warriors and their families:

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Maggie Blatz
Anthony Caycho
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Camila Bicca Oliveira
Caroline Bicca Oliveira
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أم حسين الطباطبائي
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Bies Mahabier
Jamie Hennon
Marianne Rodriquez
Marina Weiss & Gajic Family
Georgianna
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Sami Memon
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Sheila Barter
Cheryl Bentley
Nicoletta Berti
Jette Daubjerg
Vanessa Lucas
Christophe Lucas
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