Subject: Update from Elly's Team on International Participation and Next Steps

Dear Families,

Thank you for your continued patience and support. Elly's Team's Board of Directors and Patient Selection Committee met last week and would like to share the following important updates:

Compassionate Use

After careful consideration, the Board and Patient Selection Committee have decided that we will not be moving forward with a compassionate use program. Given the severity and progressive nature of NEDMSS, nearly all patients would meet compassionate use criteria. However, our current supply of the investigational gene therapy is limited, and all remaining doses will be reserved for a U.S.-based clinical trial. This trial is being designed with the intention of maximizing the resources available to Elly's Team and moving the therapy as efficiently as possible toward regulatory approval pathways.

International Clinical Trial Possibility

We recognize and deeply value the interest from families outside the United States and are committed to exploring the possibility of an international clinical trial. In order to responsibly advance this, several critical steps must be addressed:

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1. Manufacturing a Second Batch of Gene Therapy
To support an international trial, a new batch of the gene therapy (Batch #2) would need to be manufactured.

- We estimate this cost at approximately **$1.3–$1.5 million**, inclusive of vector production and additional plasmid manufacturing.
- We are currently awaiting a formal quote from Andelyn for vector manufacturing, incorporating requirements for European release standards, as well as pricing anticipated from Charles River for plasmid manufacturing.

- Once finalized, Elly's Team can establish a dedicated fund to support this effort. Fundraising would need to be led by the international patient community. Production could begin once the full amount is secured.

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2. Clear Regulatory Pathway in Europe
To proceed responsibly, we require a detailed understanding of the regulatory requirements necessary to obtain approval to treat patients in Spain (or more broadly in Europe).

- We kindly ask for your assistance in outlining the steps needed for regulatory authorization to conduct a clinical trial involving gene therapy.
- This information is essential for ensuring that the trial is conducted with the same scientific and ethical rigor as the trial within the US.

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3. Hospital Partnership and Oversight
It will be necessary to identify and assess the clinical site(s) that would conduct the trial. Specifically, we need the following details:

- The hospital(s) willing and able to run the clinical trial, including **per-patient cost estimates**
- Confirmation of hospital capabilities, particularly their experience with gene therapy and pediatric rare disease research
- Identification of the surgeon(s) who would perform the **intracisternal magna (ICM)** injections, including their training, experience, and qualifications

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Elly's Team is fully committed to maintaining the highest standards of safety, scientific rigor, and oversight as this program moves forward. We are eager to collaborate with international partners to advance this effort, while also ensuring that it aligns with our overarching clinical development strategy and does not compromise the ongoing work occurring in the US-based trial.

We look forward to hearing from you with updates on the steps outlined above and are happy to support your efforts where possible.

Warm regards,
Elly's Team