Charcot-Marie-Tooth Association

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08 December, 2025

RE: Letter of support for CLIN2-19061

Dear CIRM board,

On behalf of the Charcot-Marie-Tooth Association (CMTA), I am writing to express our strong and unequivocal support for the CLIN2-19061 application advancing AAV9/FIG4 gene therapy for CMT4J. As the leading patient advocacy organization dedicated to improving the lives of individuals affected by Charcot-Marie-Tooth disease, the CMTA has spent decades accelerating research, supporting affected families, and helping drive promising therapeutic programs toward the clinic.

CMT4J is one of the most devastating and rapidly progressive forms of CMT. Caused by biallelic pathogenic variants in FIG4, the disease results in severe motor decline, muscle weakness, loss of ambulation, respiratory involvement, and profound disability—often beginning in early childhood. For these children, the window for therapeutic intervention is short, and once neurological damage occurs, it is irreversible. Today, there are no approved treatments, and families are left to watch their children decline without any disease-modifying options.

The CMTA works directly with numerous CMT4J families who reach out to us immediately after diagnosis—often in crisis, searching for hope and for expertise. These families demonstrate extraordinary resilience, but they face an overwhelming clinical and emotional burden. The lack of available therapies for CMT4J represents one of the most urgent unmet needs in the broader CMT community. The development of AAV-based FIG4 gene replacement therapy is the only therapeutic strategy currently capable of addressing the root cause of this disorder.

Over the past several years, the CMTA has followed the progress of the ELP-02 AAV9/FIG4 gene therapy program closely. We have been encouraged by the strength of the scientific rationale, the rigor of the preclinical evidence, and the careful, responsible planning that has guided this program to the threshold of first-in-human clinical translation. The investigators have demonstrated deep scientific and ethical commitment, and the program is now ready to advance, pending the funding required to initiate the clinical trial.

CLIN2-19061 represents a critical, time-sensitive opportunity to deliver a first-in-human therapy to children living with CMT4J. Without CIRM's support, this program may face significant delays, during which affected children will continue to lose function and face irreversible neurological progression. With CIRM's support, however, this program has the potential to transform the lives of families who have no other therapeutic options.

The CMTA strongly urges CIRM to fund CLIN2-19061. This program aligns directly with CIRM's mission to enable transformative, high-impact treatments for rare diseases, especially for pediatric patients who urgently need lifesaving therapies. The CMT4J community is ready, engaged, and deeply supportive of this effort. Advancing this gene therapy into the clinic would mark a historic milestone for CMT4J and a major step forward for the broader CMT community.

Thank you for your consideration and for your leadership in advancing therapies for rare and underserved patient populations.

Your sincerely,

Suzanne L. Bruhn, PhD

CEO, CMTA