Stanford MEDICINE

Lucile Packard Children's Hospital Stanford

Department of Neurology and Neurological Sciences
Division of Neuromuscular Medicine

December 8, 2025

RE: Letter of support for CLIN2-19061

Dear CIRM board,

As Director of Stanford's Division of Neuromuscular Medicine, and Co-Director of Stanford's Neuro IGNITE Center for Innovative GeNetic Investigation and Treatment Evaluation, I have dedicated more than 35 years to designing and leading clinical trials in pediatric neuromuscular and neurodegenerative diseases, with a focus on advancing therapeutic options for rare and ultra-rare disorders with profound unmet medical need.

CMT4J is a devastating, autosomal-recessive neuropathy caused by pathogenic variants in FIG4, leading to loss of FIG4 function and dysfunctional endolysosomal trafficking. Clinical onset typically occurs in early childhood and is characterized by rapidly progressive motor decline, muscle weakness, distal and proximal denervation, respiratory compromise, and, ultimately, loss of ambulation and severe disabilities. For children with CMT4J, disease progression is not only relentless but often accelerates during key developmental windows. There are currently no course-modifying treatments available for CMT4J.

Our center receives referrals from families around the world with CMT and other forms of neuromuscular disorders, including newly diagnosed infants and children who are facing rapid functional deterioration. These families come to us searching for expertise, hope, and a path forward. After decades of working with children affected by rare neurological diseases, those affected by CMT4J clearly manifest an urgent therapeutic need. With a treatment for these patients possible, doing nothing is simply not an option.

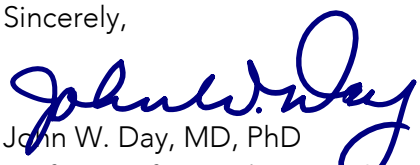
I have worked closely with the team developing the ELP-02 AAV9/FIG4 gene therapy program from its inception across both the preclinical and clinical domains. The preclinical science supporting FIG4 replacement has been compelling and consistent, including motor and pathological rescue resulting in survival of the CMT4J mouse model. The team has demonstrated scientific rigor, urgency, and deep commitment to the highest standards of safety, while making thorough and disciplined progress toward a clinical-ready vector, toxicology package, and regulatory engagement.

The goal of CLIN2-19061 is to launch a first-in-human clinical trial to treat CMT4J with AAV9-mediated FIG4 gene replacement. This trial represents the only realistic path to modifying the course of this disease and preventing irreversible neurological decline. The translational path is well-justified, the therapeutic rationale is strong, and the program has reached a stage where securing funding is critical and essential for advancing the program into the clinic.

Given the severity of CMT4J, the rapidity of disease progression, and the absence of any disease-modifying therapy, I am very excited by this program and strongly endorse it. I urge CIRM to support CLIN2-19061 so that we can bring this urgently needed therapy to the children under our care, as well as making it available to those who will be diagnosed in the years ahead. Funding this CLIN2-19061 proposal has the potential to transform what is currently a devastating, progressive, and life-limiting disease into one for which meaningful treatment is finally available.

Thank you for considering CLIN2-19061 and for your continued commitment to advancing treatments for rare and neglected diseases.

Sincerely,



John W. Day, MD, PhD
Professor of Neurology, Pediatrics, and Pathology
Director, Stanford Division of Neuromuscular Medicine
Co-Director, Stanford Neuro IGNITE Center
Email: jwday@stanford.edu
Phone: 650.725.7622