



May 29, 2024

Subject: Letter of Support for CIRM Grant #TRAN1-16026

Dear Members of the Application Review Subcommittee,

I am writing to express our support for the grant submitted by Dr. Gomez-Ospina to the California Institute for Regenerative Medicine TRAN1 Program on behalf of the Gaucher Community Alliance (GCA).

The Gaucher Community Alliance is a 501(c)(3) non-profit organization founded by patients, for patients. Our mission is to provide unwavering support to individuals and families impacted by Gaucher disease. We do this through a variety of means, including peer-to-peer support, education, advocacy, resource provision, and fostering connections within the Gaucher community. Our commitment extends to advancing safe and effective treatments for these rare diseases, achieved through grant support, educational initiatives, and collaborative efforts. Consequently, we are tremendously excited about Dr. Gomez-Ospina's innovative approach to advance into clinical trials.

We firmly believe that increased collaboration and emotional support within the Gaucher patient community can help alleviate the considerable burdens associated with this rare disorder. Our primary objectives revolve around education and support. By facilitating mutual assistance and establishing peer-to-peer connections, the GCA aims to ensure that no family faces the challenges of Gaucher disease in isolation. As the needs of the Gaucher community evolve, we stand ready to advocate for and work alongside patients to address the ever-changing difficulties they encounter. Through collaborative efforts with patients, the GCA is committed to empowering all those affected to lead fulfilling lives.

As one of the foremost organizations globally dedicated to Gaucher disease, we closely monitor the progress of various therapies in development, with a keen awareness of their potential and challenges. We are genuinely excited about Dr. Gomez-Ospina's approach, utilizing genome editing to modify a patient's own blood stem cells, which we believe holds great promise and clinical feasibility for this patient population. We eagerly anticipate collaborating with Dr. Gomez-Ospina as her strategy approaches clinical testing, with a strong focus on crafting a clinical protocol that prioritizes patients' needs.

Dr. Gomez-Ospina has done outstanding work in the Gaucher disease field. The GCA is eager to follow the trajectory of her research and hopes for the emergence of a future clinical trial for this complex disease. Equally important, we aspire to see her studies yield positive outcomes for Gaucher disease type 1 and envisage the potential for her platform to be readily applicable to other forms of Gaucher disease, where a substantial unmet medical need persists.

The Gaucher Community Alliance (GCA) strongly encourages your support for this grant. Your backing is crucial in advancing efforts to enhance the quality of life for those affected by Gaucher disease.

Sincerely,



Cynthia J. Frank
Co-Founder, Co-President