CLIVE SVENDSEN, PH.D.
PROFESSOR AND EXECUTIVE DIRECTOR
BOARD OF GOVERNORS REGENERATIVE MEDICINE INSTITUTE
KERRY AND SIMONE VICKAR FAMILY FOUNDATION
DISTINGUISHED CHAIR IN REGENERATIVE MEDICINE
CEDARS-SINAI MEDICAL CENTER

September 25th 2025

Ref: Develoment of iPSC-derived neural progenitors secreting GDNF for the treatment of ALS.

App Number: DISC2 - 16686

Dear CIRM Board,

We are very excited to have submitted a DISC2 proposal that describes a new iPSC derived neural progenitor prodcuct engineered to release the powerful growth factor GDNF to prevent motor neurons from dying in ALS. We are doing this now to ensure a continual pathway of cell product development following our very successful safety studies (completed or currently underway) using a very similar product but derived from a single unique source of fetal brain tissue. iPSCs will provide a similar cell product but completely renewable and much easier to commercialize.

We were the only grant to be to have a minority report as we had more than 35% of the committee recommend this grant for funding (6 were YES and 8 were NO). The major issue seemed to have been lack of novelty. We agree this product is similar to our last one – but very importantly it allows us to produce a renewable product that is expandable to treat thousands of patients and will have much more global impact if our early studies are successful.

We hope that there will be enough funding for this round to include this proposal that aims to deliver a unique combined cell and gene therapy product to patients with ALS.

Clive N. Svendsen, Ph.D

8700 BEVERLY BOULEVARD · ASHP A8400 · LOS ANGELES, CALIFORNIA 90048-1860
TELEPHONE: (310) 248-8072 FAX: (310) 248-8555 E-MAIL: CLIVE.SVENDSEN@CSHS.ORG