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Mark H. Tuszynski, M.D., Ph.D.
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July 17, 2019

CIRM Independent Citizens Oversight Committee

Re: TRAN-11579, Human Embryonic Stem Cell-Derived Neural Stem Cells for Severe Spinal Cord Injury (SCI)

Dear ICOC Members:

I am grateful for the opportunity to relay a few points related to the evaluation of our project for CIRM funding.

• **Programmatic Impact:** We are the only stem cell project in the CIRM portfolio that aims to treat severe SCI. We aim to re-connect damaged circuits in the spinal cord, addressing a great unmet medical need that currently has no treatment. Other SCI-related projects in CIRM's portfolio target the small population of uninjured connections that remain after a spinal cord injury ("remyelination"), an approach that may not impact the majority of SCI patients who have severe injuries. Our program significantly widens the potential scope and impact of CIRM funding.

• **Patient Impact:** This program has been supported by CIRM from its earliest stages to the present level of translational readiness. Upon completing work in this TRAN grant, we will be ready to initiate human clinical trials in SCI. This TRAN grant will produce our clinical trial cell line and establish standards for testing and releasing these cells to patients and physicians.

• **Effectiveness in Large Animal Models:** We have shown the effectiveness of this stem cell therapy for SCI in several rat experiments, and now in large animal models that are the best predictive model of a benefit to humans. Our demonstration of effectiveness in large animals is unique among stem cells programs for neurological disease, and strongly supports moving this program to patients.

• **Strength of the Science and Experience of the Research Team:** We have published results from this program in the top journals of science, including 6 papers in top Nature journals in just the last year, supporting the quality and depth of this program. Moreover, we have a successful track record of moving research programs to first-in-human clinical trials in neurological disorders. We have already involved clinicians and surgeons in our work, and we are ready to initiate clinical trials with an extensive collective body of experience and dedication.

Sincerely,

A handwritten signature in black ink that reads "Mark H. Tuszynski".

Mark H. Tuszynski, MD, PhD