

January 27, 2019

Ms. Maria Bonneville

California Institute for Regenerative Medicine (CIRM)

CIRM 1999

Harrison Street Suite 1650,

Oakland, CA 94612

**Re: Letter of Support for CLIN1-10953: "An hESC-derived hNSC Therapeutic for Huntington's Disease"
(PI: Leslie Thompson)**

Dear Members of the California Institute for Regenerative Medicine (CIRM) Governing Board and Independent Citizens' Oversight Committee,

On behalf of the Huntington's Disease Society of America (HDSA), I am delighted to write to you to express our strong support of the CIRM grant application (CLIN1-10953: An hESC-derived hNSC Therapeutic for Huntington's Disease) from Dr. Leslie Thompson and her colleagues.

The overall goal of this proposal is to complete GLP preclinical studies and file an IND to permit the initiation of a clinical trial using human-derived neural stem cells in Huntington's disease (HD) patients. Over the past couple years, we have seen great progress in the development of novel therapies to treat HD. While several huntingtin lowering drugs have entered the clinic, to date we lack *any* evidence that these approaches will produce clinically meaningful benefits in man. While the current therapies may lower huntingtin, it is possible that they will be unable to restore lost neural connectivity and improve many of the cognitive and psychiatric symptoms that most impact HD patients and their caregivers. That is why it is so critical that we continue to pursue any and all innovative approaches, such as the use of implanted hNSCs.

As one of the cruelest diseases known to man, it is not surprising that the people suffering with HD are willing to subject themselves to a high degree of risk in the hope of some relief from their devastating symptoms. To help capture this feedback, HDSA has formed a coalition with HD organizations from around the world called the HD Coalition for Patient Engagement (HD-COPE). HD-COPE provides the HD patient and caregiver perspective to any group interested in developing an HD therapeutic. We have found that after generations of suffering within families, the aversion to potential research risks in the HD community is low and they would welcome a stem cell based approach to treat HD.

Finally, HDSA currently supports nearly 50 Centers of Excellence across the United States where HD patients and families go to receive expert multidisciplinary care ranging from genetic testing and counseling to physical therapy, to experimental medicine and HD clinical trial participation. The Clinical Directors at these academic medical centers are highly committed key opinion leaders in the field of HD care and research. At any point during the development of this research project, HDSA is committed to providing Dr. Thompson and her team at UC-Irvine open access to all of the medical and research resources at our disposal to facilitate the development of this novel therapeutic approach. Thank you for your consideration.

Sincerely,



Louise Vetter
President and CEO
Huntington's Disease Society of America

ABOUT HDSA: *HDSA is a national, non-profit, voluntary health agency dedicated to finding a cure for HD and to supporting the individuals and families impacted by HD. HDSA strives to improve the lives of individuals with HD, as well as their families, by providing them with access to care, support, and educational information. Our national network of 54 volunteer-based chapters and affiliates, 60+ social workers, 49 HDSA Centers of Excellence for Family Services, and more than 160 support groups across the United States offer resources and guidance to families affected by HD.*