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CIRM ICOC Board Members c/o Stuart A. Lipton, MD, PhD Hannah and Eugene Step Chair Departments of Medicine and Neuroscience Scripps Research Institute 11119 North Torrey Pines Road, Suite 125 La Jolla, CA 92037

August 10, 2018

Re: Funding CIRM Application DISC2-11070 for the Treatment of Autism, PI Stuart Lipton

Dear CIRM ICOC Board Members:

As Director of the Neurogenetics Program and the Center for Autism Research and Treatment at the at UCLA School of Medicine, I write in support of application DISC2-11070, "Drug Development for Autism Spectrum Disorder Using Human Patient iPSCs," which received a score of 87. Specifically, I want to highlight the importance of this work for development of a therapeutic for citizens in California with autism spectrum disorder (ASD), for which there is no current treatment or cure. This is probably the most common affliction that you will deal with, now estimated to affect 1 in 59 children according to CDC statistics for 2018. Dr. Lipton discovered a gene that causes ASD called MEF2C. In my laboratory, we independently then found that MEF2C is a regulatory factor driving transcription of many other genes involved in autism. Hence, finding a treatment for the MEF2C form of autism, as proposed in the current application, may well form the basis of a treatment for many other forms of autism. As such, I strongly encourage you to reconsider your July Board Meeting decision to defer funding of this application and instead to fund it forthwith. It is extremely important for the citizens of California that you do this, as this is probably the most common disease that has been presented for funding to your Board, and the current application outlines a pathway for novel treatment. Developing treatments for ASD is risky and not guaranteed, but it is an enormous unmet need that this project addresses quite effectively. For these reasons, I strongly support this application.

Yours Sincerely,

Daniel H. Geschwind, MD, PhD

Gordon & Virginia MacDonald Distinguished Professor

Departments of Neurology, Psychiatry and Human Genetics

Director, Center for Autism Research and Treatment

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