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GAVIN HERBERT EYE INSTITUTE

Department of Ophthalmology

University of California, Irvine
School of Medicine
850 Health Sciences Road
Irvine, CA 92697-4375
OFFICE (949) 824-6211
FAX (949) 824-4015

Jonathan Thomas, Chairman
Independent Citizens Oversight Committee
California Institute for Regenerative Medicine
1999 Harrison St, Ste. 1650
Oakland, CA 94612

RE: CLIN2-11472, "A Phase 2 Study of the Safety of Repeat Intravitreal Injection of Human Retinal Progenitor Cells in Adult Subjects with Retinitis Pigmentosa"

October 25, 2019

Dear Chairman Thomas and ICOC members,

On behalf of our team, I would like to thank you and the citizens of California, for your ongoing support of our clinical stage project, from DT1 Planning, through ET2, DT2, and prior CLIN2 Awards. Without the financial backing and expert guidance from the CIRM team and CIRM consultants, we would not have been able to move our program forward on such a strong foundation and at such a rapid pace. We also thank the scientific reviewers for recommending that our grant be awarded. With this critical support we can complete the milestones needed in order to launch a future phase 3 study without delay.

Unmet Medical Need: Retinitis pigmentosa (RP) is a heritable blinding condition characterized by progressive loss of photoreceptors (rods and cones) and an inexorable decent into blindness. There is no treatment, beyond gene therapy for a very rare subset of patients.

The Team: Human retinal progenitor cells (jCell) technology was developed in our laboratories (UC Irvine, UC Santa Barbara) and licensed by the California startup jCyte, specifically founded to propel this therapy to commercial availability. jCyte's CEO is dedicated to finding a treatment for his daughter who has a form of RP.

The Treatment: jCell is easily delivered to the eye by injection in an office setting. The cells enhance photoreceptor survival and function via secreted factors, independent of the underlying gene mutation.

Re-Treatment: jCell provides sustained effect (> a year) and--unlike alternate approaches--can be re-injected when treatment effect dissipates.

Clinical Progress: With CIRM support, this project progressed well through the clinic with a favorable safety profile

IND: April 2015, FDA approval
Phase 1/2a Clinical Trial: 28 subjects, 4 dose levels
Completed July 2017

Favorable safety profile, indications of treatment benefit for visual acuity Regenerative Medicine Advanced Therapy (RMAT) designation from FDA

Phase 1/2a Extension Study: 22 subjects, fellow eye treatment
Completed October 2018.

Phase 2b Clinical Trial: 80 subjects completed the study
Fully enrolled June 2018 (1 year ahead of CIRM milestone)
Three arms – 2 dose groups and a control group
Favorable safety profile, as with prior study (including higher dose)
Review of recently received efficacy data shows encouraging results and provides key trial design information to advance the project to a pivotal trial

Manufacturing: In parallel to our clinical program, tech transfer was completed to a commercial contract manufacturer in Q2 2018, and is now in the process of scale up and qualification.

Current status: We have currently treated 130 eyes in 108 patients, with a favorable safety record as well as clinical data consistent with a treatment signal through phase 2b. Simultaneously, we have moved forward with commercial manufacturing in order to provide the cell product required for a phase 3 pivotal study.

Next Steps: Our goal is to achieve phase 3 readiness on the shortest possible timeline. To do that we need to fund the following critical path activities:

Redosing Trial: The expected duration of treatment effect is 12-18 months and patients in the first trial received their most recent injection a year or more ago and their first injection up to 4 years ago. As anticipated, improvements have started to fade and patients have signaled their strong interest in repeat dosing. Moreover, the FDA has requested redosing data. Delays in initiating this study would be inconsistent with CIRM's mission of accelerating therapies for patients.

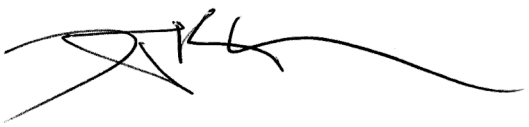
Manufacturing: jCyte and FDA have agreed to test commercially manufactured cell product as part of a phase 3 pivotal study. This product must be available for use.

Completion of these specific clinical and CMC activities is urgent. Doing so will address the stated regulatory requirements and allow us to proceed to phase 3 testing, thereby accelerating the program towards marketing approval.

Our Commitment: $4,405,728 in co-funding from jCyte.

We thank CIRM for your partnership, guidance, and support in this effort to bring a novel cell-based therapy for RP patients to fruition.

Sincerely,



Henry Klassen, MD, PhD
Professor