



October 15, 2018

Attention: Maria Bonneville (mbonneville@cirm.ca.gov)
Independent Citizens' Oversight Committee (ICOC)
California Institute for Regenerative Medicine (CIRM)
1999 Harrison Street, Suite 1650 Oakland, CA 94612

Re: Letter of Support for CIRM TRAN1-11259 (Developing engineered autologous leukemia vaccines to target residual leukemic stem cells, Gaensler, P.I.)

Dear Members of the California Institute for Regenerative Medicine Governing Board and Independent Citizens' Oversight Committee:

As the director of the CIRM Alpha Stem Cell Clinic recently established at UCSF and its 4 clinical sites at Zuckerberg SF General Hospital, at the Mission Bay and Parnassus sites and at Benioff Children's Hospital, Oakland, I am writing this letter in strong support of Dr. Gaensler's TRAN1 application to develop a patient-derived leukemia vaccine that potentially targets leukemia stem cells. She has brought together a group of investigators with extensive experience in bringing gene therapy-based therapeutics to the clinic. These physicians and scientists include Dr. Donald Kohn (UCLA) and Dr. Farzin Farzaneh (King's College, London) as well as physicians and translational scientists from UCSF, UC Davis, and Stanford University. The group has long-standing interactions and a well-organized plan for pursuing pre-clinical Pre-IND studies. Development of this vaccine program has the full support of the Division of Hematology/Oncology and the Helen Diller Family Comprehensive Cancer Center.

As you know, the mission of the Alpha Stem Cell Clinic Network is to accelerate the development, launch and completion of clinical cellular therapy trials. In this regard, we are pleased to extend our infrastructure resources to assist Dr. Gaensler in the regulatory requirements and compilation of the pre-IND packet, act as a resource in identifying the optimal GMP manufacturing venue, and assist if requested to help draft a clinical trial protocol and consent for this important project. We will also assist in developing biospecimen handling requirements for the clinical phase of the project.

In conclusion, this is a timely and outstanding application with great potential for clinical translation in the near future. It deserves CIRM support.

Thank you for your consideration,



Sincerely yours,

A handwritten signature in black ink, appearing to read "Mark C. Walters". The signature is fluid and cursive, with a long horizontal stroke at the end.

Mark C. Walters, MD
Adjunct Professor of Pediatrics, UCSF School of Medicine
Jordan Family Director, Blood and Marrow Transplant Program
Interim Director of Research

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