

Department of Pediatrics

February 12, 2019

Research Units
Pathobiology Unit
Patient Oriented Research Unit
Developmental Biology and Genetics Unit

John Thomas, PhD, JD
Chair, Governing Board of the CIRM (ICOC)
210 King Street
San Francisco, CA 94107

Re: CLIN2-11431: A monoclonal antibody that depletes blood stem cells and enables chemotherapy free transplants

Dear Dr. Thomas,

I am writing to support additional funding for the trial of anti-CD117 in children with the lethal disease severe combined immunodeficiency (SCID). I am a pediatric hematologist whose research and clinical background includes the transplantation of children with non-malignant disorders, including those with primary immunodeficiency. I am also an advisor to the Pediatric Immunodeficiency Treatment Consortium. SCID is only curable by blood stem cell transplantation. Depletion of the patient's own marrow stem cells prior to transplantation is a critical step in facilitating engraftment of donated stem cells. Identifying more selective approaches for HSC cytoablation that spare young children the global toxicity of chemotherapy- or irradiation-based regimens will greatly advance in the field.

I wish to affirm my support for the above referenced CIRM sponsored study, which is testing a unique monoclonal antibody directed against CD117, to determine if it can be used to replace chemotherapy as conditioning for transplant. I understand the early data suggests that the antibody is accomplishing its intended goal of safely depleting recipient marrow stem cell and allowing replacement by donor blood stem cells. This approach is applicable to not only standard allogeneic transplants from donors but use of the antibody may eventually be useful for future trials of autologous gene therapy.

If their study continues to demonstrate efficacy, it would prove transformative. We would be able to achieve cure of inherited immunodeficiencies and other blood cell disorders and eliminate the unwanted and unnecessary toxicity of damaging chemotherapy for marrow transplantation.

Thank you for your consideration.

Sincerely,



Mary C. Dinauer, MD, PhD
Fred M. Saigh Distinguished Chair of Pediatric Research
Professor of Pediatrics and Pathology & Immunology
Scientific Director, Children's Discovery Institute

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