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We Treat Kids Better

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February 12, 2019

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 Michael Pulsipher, currently the Section Head of BMT and Cellular Therapy at the Children's Hospital Los Angeles. A large focus of my work is transplantation of children with non-malignant disorders, including children with severe combined immunodeficiency (SCID). We have focused on this disorder for many years and are one of the major programs treating this challenging disease in Southern California. One of the major challenges in our field is reliance on toxic chemotherapeutic agents to deplete a patient's own stem cells in order to allow donated stem cells to engraft. Because these children are now diagnosed by newborn screening, we are performing transplants as early as 2-3 months of age. Our hope is to use only the smallest amounts of chemotherapy on these infants, but studies have shown that when unrelated or haploidentical donors are used (up to 90% of patients), high dose preparative regimens are needed to ensure both T and B cell reconstitution. These chemotherapy regimens lead to infertility, growth issues, and many other long-term consequences.

I wrote a strong support letter earlier and 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. Because I have been assisting CIRM as an advisor, I have seen in detail data showing that the antibody is accomplishing its intended goal of safely depleting recipient blood stem cell and allowing replacement by donor blood stem cells in the small number of patients that have been treated. This approach is applicable to not only standard allogeneic transplants from donors but use of the antibody may eventually be applicable to the future trials of autologous gene therapy. If their study continues to demonstrate efficacy, it would prove practice changing for our field, as we would be able to achieve cure of this disease without the unwanted and toxicity of chemotherapy.

Thank you for your consideration.

Sincerely,

Michael A. Pulsipher, MD
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