

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

February 11, 2019

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

Dear Dr. Thomas,

I am Kirk Schultz from British Columbia Children's Hospital. I am also past chair of the Pediatric BMT Consortium, current Director-at-large for Research for the Canadian BMT Group, and one of the founders of the Pediatric Immune Deficiency Therapeutic Consortium. My field of expertise is the transplantation of children with non-malignant disorders, including children with the lethal disease severe combined immunodeficiency (SCID). SCID is only curable by blood stem cell transplantation. One of the major challenges in our field is our reliance on toxic chemotherapeutic agents used to deplete a patient's own stem cells in order to allow donated stem cells to engraft. The consequences of this chemotherapy is particularly deleterious in children who may suffer short term, as well as long-term, deleterious consequences. 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 blood 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 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 and eliminate the without the unwanted and unnecessary toxicity of chemotherapy.

Thank you for your consideration,

Sincerely,



Kirk R. Schultz, MD, FCAHS  
Director, Michael Cuccione Childhood Cancer Research Program  
Professor of Pediatrics  
BC Children's Hospital and Research Institute