

Feb 11, 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 a pediatric immunologist, who has been involved in hematopoietic stem cell transplantation for 49 years since I did my first transplant at The Childrens Hospital in Boston in 1969. I pioneered the use of hematopoietic stem cell transplantation for the treatment of genetic diseases in my successful transplantation of patients with the Wiscott-Aldrich syndrome in 1978. A major limitation to the successful use of hematopoietic stem cell transplantation to treat children with genetic abnormalities of their immune and hematological system is the toxicities associated with the pre-transplant chemotherapy, which is required to achieve donor hematopoietic stem cell engraftment. Therefore, I have been excited by the preliminary results of Dr. Shizuru and her colleagues, who have shown that the administration of a monoclonal antibody to CD117 (c-kit) can result in donor hematopoietic stem cell engraftment without the administration of chemotherapy. The results of their dose escalation of the antibody dose suggests that clinically significant levels of donor hematopoietic stem cell can be achieved. Besides the use of the antibody for the treatment of patients with severe combined immune deficiency (SCID), I envisage the future use of the antibody in the clinical trials of the transplantation of gene corrected autologous hematopoietic stem cells for diseases like sickle cell disease and Fanconi anemia where there is resistance to the use of chemotherapy before transplantation. I, therefore, fully support CLIN2-11431. Please feel free to contact me if you have any questions.

Sincerely yours,



Robertson Parkman, M.D.
Adjunct Professor of Pediatrics
Stanford University
rparkman@stanford.edu