Immune Deficiency Foundation

The National Patient Organization Dedicated to Advocacy, Education and Research for Primary Immunodeficiency Diseases

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 write to you on behalf of the Immune Deficiency Foundation (IDF), a national nonprofit patient organization dedicated to improving the diagnosis, treatment and quality of life of persons with primary immunodeficiency diseases (PI) – including Severe Combined Immunodeficiency (SCID) – through advocacy, education and research.

Beginning December 2018, all newborn children in the United States are being tested for SCID, a life-threatening disorder and one of the most severe forms of PI. This early identification of SCID can now make possible life-saving intervention – such as hematopoietic stem cell (HSC) transplantation – before infections occur. In order to prevent rejection, most patients require chemotherapy and/or radiation therapy to weaken their own residual immune system to prevent it from rejecting the transplanted HSCs. Unfortunately, the chemotherapy treatment may cause serious, even life-threatening side effects. These include transient loss of all of the cells of the bone marrow so the patient is very susceptible infections, anemia (low RBC) and bleeding problems due to low platelets. Chemotherapy also may cause severe blistering of the mouth or other mucous membranes that makes getting adequate hydration and nutrition very difficult.

As such, IDF strongly supports continued funding for the CIRM sponsored study, which is testing a unique monoclonal antibody directed against CD117, in order to determine if it can replace chemotherapy as conditioning for transplant. I understand the early data is promising in accomplishing its intended goal of safely depleting recipient blood stem cell and allowing replacement by donor blood stem cells. This approach may eventually be applicable to future trials of autologous gene therapy.

Thank you for your consideration,

Sincerely,

John G. Boyle
President & CEO
Immune Deficiency Foundation

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