

We Treat Kids Better

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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,

My name is Neena Kapoor. I am Director of Blood and marrow and cell therapy laboratory and attending physician of Blood and Marrow transplant and Cell therapy program at Children's Hospital Los Angeles and professor of Pediatrics at Keck school of Medicine at USC.

I am a Blood and Marrow stem cell transplanter with special interest in Primary immunodeficiency diseases and alternative donor stem cell transplants especially for severe combined immune-deficiency disorders. Over the years, the field has evolved from performing transplants from matched sibling donors to Haploidentical donors after T cell depletion, to unrelated donors. We have learnt the need for conditioning regimen for such transplants and issues associated with the pre transplant conditioning. It is utmost important for us to now address how can we now move to next step of abating the acute and chronic toxicities associated with conditioning regimen, without compromising engraftment of donor cells and complete reconstitution of immune system. The CIRM-sponsored study referenced.

Above, is based on use of a monoclonal antibody directed against CD117, represents the opportunity to deplete autologous hematopoietic stem cells without causing unwanted toxicity. I understand the early data gathered so far indicate that this approach is safe. It has been reported to be effective in depleting autologous stem cells while allowing engraftment of donor-derived hematopoietic stem cells. In the future, this may be applicable to treat other genetic diseases also. Continuation of this trial is extremely important to confirm efficacy without the unwanted and unnecessary toxicity associated with standard conditioning regimen. If proven, it will have wilder application in the field of transplantation.

Thank you for your consideration, Sincerely,

Neena Kapoor, MD

Director, Blood and Marrow Transplant Laboratory Professor of Pediatrics, Keck School of Medicine, USC Hematology, Oncology and Blood & Marrow Transplantation

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