NKT Therapeutics Presents Data on Potential Antibody Treatment for Sickle Cell Disease

Preclinical Study on NKTT120 To Be Presented at American Society of Hematology

WALTHAM, MA (December 8, 2012): NKT Therapeutics, Inc., a privately held biotechnology company, announced preclinical study results supporting the potential of its lead therapeutic candidate, NKTT120, as an innovative approach to the treatment of sickle cell disease. Preclinical studies of NKTT120, a humanized monoclonal antibody, showed its ability to generate a safe, rapid, specific and complete depletion of invariant Natural Killer T cells (iNKT cells) 24 hours post dosing, thus offering a means to reduce the chronic inflammation associated with sickle cell disease pathology. NKT Therapeutics plans to initiate a clinical trial of NKTT120 in patients with stable sickle cell disease in the first quarter of 2013.

The study will be presented on December 9 at the American Society of Hematology meeting in Atlanta as a poster presentation entitled, “The Safety Evaluation of NKTT120, an iNKT Cell Depleting Antibody in the Cynomolgus Monkey.” An abstract of the study has been published online at https://ash.confex.com/ash/2012/webprogram/Paper50259.html

NKTT120 is a humanized monoclonal antibody that results in a safe, rapid, specific and complete depletion of iNKT cells. The presented research showed that a specific and complete depletion of iNKT cells was observed at the earliest post dosing measurement at 24 hours, and was maintained throughout the 85 day study. Investigators observed no adverse events, infections, or cytokine release. T cell-dependent antibody response to KLH antigen was not affected by NKTT120 treatment.

Chronic inflammation is associated with the pathology of sickle cell disease, and studies by NKT Therapeutics and others have found an increased ratio of activated iNKT cells in the peripheral blood of patients with sickle cell disease. These findings suggest that iNKT cell reduction and/or depletion would be effective in reducing the damaging inflammation associated with sickle cell disease.

“Depletion of iNKT cells represents an exciting new approach to the treatment of sickle cell disease, and we are encouraged that this mechanism has the potential to meaningfully improve the lives of patients with this condition,” stated Dr. Robert Schaub, Chief Scientific Officer at NKT Therapeutics.

“The safety and pharmacodynamic profile of NKTT120 demonstrated in this study are extremely encouraging, and we look forward to testing this investigational agent in patients with stable sickle cell disease,” added Dr. Joshua Field, Medical Director of the Adult Sickle Cell Disease Clinic at the Medical College of Wisconsin, and Principal Investigator of the planned study.

About NKT Therapeutics, Inc.
NKT Therapeutics, Inc. is a privately held biotechnology company focused on developing therapeutics based on unique immune cells called natural killer T (NKT) cells. The company’s mission is to use its expertise to develop a pipeline of first-in-class NKT-based therapeutics to treat sickle cell disease, autoimmune and inflammatory diseases, and cancer. For more information on the company, please visit http://www.nktrx.com.

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**Contacts:**

Robert Mashal  
NKT Therapeutics  
(781) 684 0290  
info@nktrx.com

Joan Kureczka  
Kureczka/Martin Associates  
(415) 821 2413  
Joan@kureczka-martin.com