FDA Grants Fast Track Designation to NKT Therapeutics’ NKTT120 for the Treatment of Sickle Cell Disease

• Dosing in Phase 1b Trial Complete
• Plan to Advance into Phase 2b Studies with Endpoints Demonstrating Clinical Efficacy in 2015

WALTHAM, MA (October 2, 2014): NKT Therapeutics today announced that the U.S. Food and Drug Administration has granted Fast Track designation to NKTT120, the company’s lead therapeutic being developed for the treatment of sickle cell disease. NKTT120 is a humanized monoclonal antibody that specifically depletes iNKT cells, a regulatory T cell that has been demonstrated to be a key mediator of organ damage in preclinical models of sickle cell disease. Fast Track designation is intended to facilitate development and expedite review of drugs intended to treat serious or life-threatening medical conditions and demonstrate the potential to address unmet medical needs. The FDA previously granted Orphan Drug designation to NKTT120 for the treatment of sickle cell disease.

NKT Therapeutics also announced that the company has completed dosing in a Phase 1b trial of NKTT120 in patients with sickle cell disease. The primary goal of the trial is to determine the safety of NKTT120 in such patients. Secondary endpoints include effects on markers of inflammation, daily pain scores, and Quality of Life. Further details of the trial can be found at clinicaltrials.gov. NKT Therapeutics plans to present updated results of its clinical trial at a major medical conference later this year.

iNKT cells have been demonstrated to be key mediators of the inflammation that can result in pain and organ damage in sickle cell disease. Treatment with NKTT120 reduces iNKT cell-mediated inflammation.

“We look forward to working with the FDA on the design of our efficacy trials for NKTT120,” stated Robert Mashal, chief executive officer of NKT Therapeutics. “Fast Track status will help us reach our goal of bringing this new therapy to patients as rapidly as possible.”
About NKT Therapeutics

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.

Contacts:

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

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