NKT Therapeutics Reports First Clinical Results with anti-iNKT Antibody in Patients with Sickle Cell Disease

- Complete Peripheral Depletion of iNKT Cells Observed
- No Dose Limiting Toxicities (DLTs); Maximal Tolerated Dose (MTD) not reached

WALTHAM, MA (December 7, 2013): NKT Therapeutics today announced the presentation of initial clinical results with NKTT120, a monoclonal antibody designed to suppress the chronic inflammation associated with sickle cell disease (SCD) pathology. Interim results from an ongoing Phase 1 safety and dose escalation study of NKTT120 in adults with stable SCD demonstrated the intended complete and specific depletion of invariant Natural Killer T (iNKT) cells from the peripheral blood. No significant adverse events (SAEs) have been observed in the data reported. The results were presented as a poster at the 55th American Society of Hematology (ASH) meeting

“Much evidence exists that iNKT cells generate an inflammatory cascade that promotes and sustains the painful vascular events suffered by patients with sickle cell disease, and researchers have shown in animal models of SCD that depleting iNKT cells decreases inflammation and prevents organ damage,” said Joshua Field of the Blood Center of Wisconsin. “Results from the initial participants in this ongoing Phase 1 study of NKTT120 provide evidence of both the safety and tolerability of this antibody-based therapy at the doses studied, and also confirm the antibody’s ability to quickly deplete iNKT cells from the peripheral circulation of SCD patients.”

The poster presentation included data from the first 9 patients treated in the Phase 1 safety and dose escalation study of NKTT120, three of whom received the antibody at the 0.001 mg/kg, 3 who received the 0.003 mg/kg dose, and 3 who received the 0.01 mg/kg dose. The primary end point for the study was safety. Secondary endpoints included daily measurements of pain, analgesic use, and respiratory symptoms captured via daily smartphone eDiary use and via standard questionnaires administered at clinic visits. All patients showed a reduction in the iNKT cells within 6 hours following treatment with NKTT120. All of the patients treated at the 0.01 mg/kg dose experienced complete peripheral depletion of iNKT cells lasting at least 2 days. The effects of NKTT120 were specific to iNKT cells, with T cell, B cell and NK cell percent of lymphocytes remaining unaffected by the treatment. The patients tolerated NKTT120 treatment well, with no serious adverse events occurring in the study in the data reported. Moreover, the investigators reported excellent patient compliance with the daily electronic data capture of pain and symptom scores.

“Results to date support the continued investigation of NKTT120 as a potential treatment to reduce the painful and damaging inflammation associated with SCD,” said Robert Mashal, MD, President and Chief
Executive Officer of NKT Therapeutics. “We are continuing enrollment into this dose escalation trial in order to determine the optimal dose for our planned Phase 2 studies.”

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.

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