NEW HAVEN, Conn.—September 28, 2015—Kolltan Pharmaceuticals, Inc. today announced the presentation of data from a preclinical study evaluating KTN0158 in dogs with spontaneous mast cell tumors. KTN0158 is a proprietary, humanized anti-KIT IgG1 monoclonal antibody drug candidate being developed as a potential therapy for cancer and mast cell-related diseases. Overall, substantial tumor shrinkage was observed during this study when KTN0158 was administered to dogs with spontaneous mast cell tumors, also known as mastocytomas. In addition, tumor shrinkage was observed at all dosing levels after one or two doses and in tumors with and without activating KIT mutations. KIT is a receptor tyrosine kinase (RTK) known to drive the growth of certain tumor types and is associated with activating mutations in both dog mastocytomas and human tumors such as gastrointestinal stromal tumors (GIST), melanoma and leukemias. Based on these results, and favorable results from a preclinical toxicology study in non-human primates, Kolltan plans to file an investigational new drug application (IND) for KTN0158 with the FDA in the fourth quarter of 2015.

The preclinical results were presented today in a poster titled, “KTN0158, a Humanized Anti-KIT Monoclonal Antibody, Demonstrates Antitumor Activity in Dogs with Mast Cell Tumors” (Poster #209, Abstract #202, Translational Research on Monday, September 28, 4:45 PM - 6:45 PM CEST, Location: C Hall at the European Cancer Conference 2015). The conference is being hosted by the European Society for Medical Oncology (ESMO) and being held in Vienna, Austria between September 25-29, 2015.

“We are excited about the preclinical data presented today and generated to date which support the evaluation of KTN0158 in the treatment of KIT-expressing tumors when wild-type or mutant KIT is expressed. We believe that KTN0158’s potential to treat both wild-type and mutant KIT-expressing tumors is related to its high potency and unique binding features, which block the dimerization and signaling of the KIT receptor,” said Gerald McMahon, Ph.D., President and Chief Executive Officer of Kolltan. “As a result, we believe that KTN0158 has the potential to be superior or complementary to small-molecule approaches to block the...
enzymatic function of this RTK. We are rapidly moving this program forward and are on track to file an IND this year in oncology. We plan to evaluate KTN0158 as a monotherapy in human cancers, such as GIST and other KIT-dependent tumors, and continue to explore it as a potential combination anti-cancer therapy.

“The IND for KTN0158 is supported by the favorable results from the nonclinical toxicology studies performed to date along with the activity of this antibody that may be due to the novel binding and blocking features of KTN0158, which has no agonist activity in vitro. Our studies further support evaluation of this product candidate to modulate mast cells, where KIT is expressed and active, in addition to blocking KIT as a tumor driver. We have generated preclinical data, and will be presenting new data at other scientific meetings, to support the use of KTN0158 to block mast cells, which in turn we expect will support applications in oncology, neurofibromatosis 1, and other diseases,” said Theresa LaVallee, Ph.D., Senior Vice President, Translational Medicine and Product Development at Kolltan.

The open-label, dose-escalating study of KTN0158 included 13 dogs with measurable spontaneous mast cell tumors (MCT), of which 12 were evaluable for efficacy, and was conducted by Dr. Cheryl London at The Ohio State University. Three dose levels and two schedules were tested. KTN0158 was given at 10 or 30 mg/kg as a single dose or given as two doses of 1 or 10 mg/kg three weeks apart. As part of the study, tumor biopsies and blood samples for PK and PD analysis were collected. The study also incorporated weekly assessments including physical examination and standard laboratory tests (serum chemistries, hematology profiles, and urinalyses) for toxicities and response. Antitumor efficacy was based on objective tumor assessments made according to established RECIST criteria for solid tumors in dogs. Adverse events (AEs) were recorded and graded according to VCOG-CTCAE v.1.1 criteria for AEs in dogs.

The presentation includes the following results and data:

- Clinical benefit of KTN0158 was observed in all dogs with MCTs at all dosing levels and in dogs with both wild-type and mutant expressing tumors:
  - Five animals showed partial responses and seven animals had stable disease; and
  - Histopathology after study completion showed a lack of neoplastic cells in primary tumors and/or lymph node samples from a total of four dogs.
- Reversible hematologic and biochemical effects were observed in dogs receiving either a 10 or 30 mg/kg/dose of KTN0158 with the maximum tolerated dose established at 10 mg/kg.
IND enabling toxicology studies in non-human primates revealed no significant findings, including hematologic effects, after repeat dosing of KTN0158, with a no observed adverse event level (NOAEL) of 75 mg/kg, the highest dose tested.

About KTN0158

KTN0158 is a proprietary, humanized monoclonal antibody designed using structure-based approaches to block the activation of KIT, an RTK that is expressed on many cancers and mast cells. Kolltan applied novel insights about the x-ray crystallographic structure of the KIT receptor to identify a unique way to inhibit the function of KIT through binding to the domain that is near the cell membrane and blocking dimerization. This targeting of the proximal membrane is a novel approach compared to targeting the ligand and led to Kolltan’s discovery of KTN0158.

There are currently no KIT-targeting antibodies on the market for any disease indication. In oncology, KIT is expressed in tumors such as GIST, melanoma, AML, SCLC, and others. There are several KIT-targeting small molecule drugs approved for use in GIST where mutant KIT is present. However, no KIT-targeting drugs are approved for non-GIST tumor types and treatment of GIST tumors does not always lead to long-term clinical benefit due to resistance, including secondary mutations that overcome small-molecule drug approaches.

As a monoclonal antibody, we believe KTN0158 is particularly suited to block KIT dimerization and inhibit activation and signaling of the receptor and therefore lead to potent inhibition of both wild-type and mutant KIT forms. Kolltan is planning to file an IND with the FDA for KTN0158 in 2015 followed by the initiation of clinical trials in oncology in 2016. A second IND filing for KTN0158 is anticipated in 2016 for neurofibromatosis 1 (NF1). KIT and mast cells have been associated with the etiology of NF1, an orphan disease afflicting approximately 100,000 individuals in the U.S.

About Kolltan Pharmaceuticals

Kolltan, a privately held clinical-stage company, is focused on the discovery and development of novel, best-in-class antibody-based drugs targeting receptor tyrosine kinases for the treatment of cancer and other diseases with significant unmet need. Kolltan’s founders and members of its management team have deep expertise and a proven track record in drug discovery, development, and commercialization of innovative therapeutics, including drugs targeting kinases. Kolltan is working in close collaboration with the laboratory of Kolltan Co-Founder, Dr. Joseph Schlessinger, as well as the Yale University medical and scientific community. The Company has a broad and novel portfolio of therapeutic biologics targeting multiple receptor
tyrosine kinases that are advancing in clinical and preclinical development and are progressing toward potential achievement of multiple near-term milestones. Kolltan’s most advanced product candidates include KTN3379, a human monoclonal antibody designed to block the activity of ErbB3 (HER3) which is in Phase 1b clinical trials in solid tumors, and KTN0158, a humanized monoclonal antibody designed to block the activation of KIT which is anticipated to enter clinical trials in early 2016.

Forward-Looking Statements

Any statements in this news release about future expectations, plans and prospects for Kolltan constitute forward-looking statements. Actual results may differ materially from those indicated by such forward-looking statements as a result of a variety of important factors. Kolltan anticipates that subsequent events and developments may cause its views to change. However, while Kolltan may elect to update these forward-looking statements in the future, Kolltan specifically disclaims any obligation to do so.

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