ProNAi to Present Phase II Clinical Data at ASH for PNT2258, a BCL2 Targeted Drug

Plymouth, MI, Nov. 7, 2013 — ProNAi Therapeutics, Inc. a leader in nucleic acid therapeutics, announced that data from its ongoing Phase II clinical study of PNT2258, a novel BCL2 inhibitor, will be presented at the 55th Annual Meeting of the American Society of Hematology (ASH), held in New Orleans December 7-10, 2013.

"We are very encouraged by the safety profile and the anti-tumor responses in patients with relapsed or refractory non-Hodgkin's lymphoma (NHL) treated with PNT2258," stated Rich Messmann, ProNAi's Chief Medical Officer. "We particularly look forward to presenting the updated safety and efficacy data on patients with follicular and diffuse large B-cell lymphoma and thank the participating patients, their families, and the study investigators Dr. Wael Harb, Dr. Nahal Lakhani and Dr. Ayad Al-Katib."

"PNT2258 is the first nucleic acid nanoparticle drug with Phase II data to be presented at a major cancer meeting." said Mina Sooch, ProNAi's Chief Executive Officer. She adds, "The unique design properties of the DNAi oligonucleotide and liposomal nanoparticle that comprise PNT2258 have allowed us to realize the promise of nucleic acid therapy without the liver toxicity or systemic delivery issues that limited the development of earlier drugs in this field. PNT2258 targets the BCL2 gene that causes cancer cells to avoid cell death and leads to resistance. While the study results show encouraging data against hematological cancers as a single agent, BCL2 also plays a role in cancers such as breast, lung, prostate, and sarcomas where a combination approach would be pursued. We look forward to designing and initiating further studies, either alone or with strategic partners."

Oral Presentation Details

Title: The BCL2 Targeted Deoxyribonucleic Acid Inhibitor (DNAi) PNT2258 Is Active In Patients With Relapsed Or Refractory Non-Hodgkin's Lymphoma (Abstract #88)
Time: Sunday, December 8, 2013 at 5:45 PM
Presenter: Ayad M. Al-Katib, MD, Study Principal Investigator and Medical Director, Cancer Center of Excellence, St. John Hospital
Location: Ernest N. Morial Convention Center, La Nouvelle Ballroom AB

Abstract Highlights

The abstract accepted for presentation at ASH includes an evaluation of anti-tumor activity and safety in patients with relapsed or refractory lymphoma undergoing treatment with PNT2258 for six cycles, or until disease progression or unacceptable toxicity. At the time of the data submission, 10 patients had been treated between January and August 2013. PNT2258 was administered at a dose of 120 mg/m^2 as a 3-hour IV infusion on days 1-5 of a 21-day cycle. Toxicity occurring in ≥ 2 patients included: grade 1 chills (n=2) and diarrhea (n=2); grade 2 nausea (n=3), tumor pain (n=2) and hypotension (n=2); the single grade 3 toxicity occurring in ≥2 pts was nausea (n=2); and no grade 4 toxicity was noted. Additionally, Tumor Lysis Syndrome has not been observed. Only 2 patients exhibited progression of disease. Antitumor activity was observed in all 5 patients with FL, which included 1 CR, 1 PR (with 80% reduction), and 3 with SD (all with net tumor shrinkage ranging from 5-15% at end-of-cycle 2 scan). Seven of 10 patients with DLBCL, FL, MCL and CLL remain on study and updated results will be presented at the meeting.

About PNT2258

PNT2258 is a 24-base, single-stranded, chemically unmodified DNA oligonucleotide called PNT100

that is encapsulated in specialized anionic and pH “tunable” liposomes (SMARTICLES®). Robust preclinical activity of PNT2258 has been demonstrated in a variety of hematological and solid tumor models. PNT2258 exhibits broad systemic exposure and cellular uptake, resulting in cell death by modulation of the BCL2 gene. A Phase I dose-escalation study of PNT2258 in patients with advanced solid tumors was completed in 2012 at South Texas Accelerated Research Therapeutics (START) under the direction of Dr. Anthony Tolcher. Twenty-two patients received over 300 doses of PNT2258 which was well-tolerated with dose proportional pharmacokinetics, and on-target BCL2 effects. The findings included four patients with stable disease and were presented at the NCI/AACR/EORTC 2012 and AACR 2013 Annual Meetings.

About ProNAi Therapeutics, Inc.

ProNAi Therapeutics, founded in 2004, has a proprietary and differentiated DNA interference (DNAi®) technology. DNAi utilizes single-stranded, unmodified, phosphodiester DNA sequences designed against genomic DNA to modulate gene transcription. In addition to PNT2258, the company has an expanding pipeline of DNAi leads for over 30 cancer and non-cancer targets, including CMYC and KRAS. ProNAi’s business strategy is to establish multiple pharmaceutical partnerships across its portfolio of DNAi drug candidates.

Forward-Looking Statements

This press release contains “forward looking statements” as that term is defined in the Private Securities Litigation Reform Act of 1995. These statements are based on management’s current expectations and involve significant risks and uncertainties that may cause results to differ materially from those set forth in the statements.

Source:

ProNAi Therapeutics, Inc.
Mina Sooch, CEO
248-681-9815
msooch@pronai.com
www.pronai.com