ProNAi Therapeutics Reports Anti-Tumor Activity from Ongoing Phase II Clinical Study of PNT2258, a Novel BCL2-Inhibitor, at ASH Annual Meeting

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PLYMOUTH, Mich.--(BUSINESS WIRE)--ProNAi Therapeutics, Inc., a leader in developing nucleic acid therapeutics, presented safety and efficacy data from its ongoing Phase II study yesterday at the 55th Annual Meeting of the American Society for Hematology (ASH) in New Orleans, Louisiana. Phase II findings on the company's first-in-class BCL2 targeted IV drug were presented in an oral session on Novel Therapies for Lymphoma, titled "The BCL2 Targeted Deoxyribonucleic Acid Inhibitor (DNAi) PNT2258 Is Active In Patients With Relapsed Or Refractory Non-Hodgkin's Lymphoma." PNT2258's unique mechanism of action targets the BCL2 gene directly, rather than the BCL2 protein, to inhibit cancer cell proliferation, drive cell death (apoptosis) and minimize off-target effects.

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PNT2258 Phase II Data Presented at ASH on December 8, 2013 (Abstract #88):

- PNT2258, a first-in-class BCL2 targeted drug, exhibits single agent anti-tumor activity in patients

with recurrent or refractory NHL.

- 82% of patients had tumor shrinkage when receiving single-agent therapy with PNT2258. To date, overall response rate in patients with follicular lymphoma (FL) is 40% and in patients with diffuse large B-cell lymphoma (DLBCL) is 50%.
- Several patients have elected to receive additional maintenance therapy after their planned 6 treatment cycles.
- PNT2258 is safe at a dose of 120 mg/m2 IV administered for 2-3 hours on days 1-5 of a 21-day schedule. No tumor lysis syndrome or major organ toxicities were observed. No occurrences of elevated liver enzymes, hyperkalemia, hyperphosphatemia, hypocalcemia, renal failure/dysfunction, or infections were noted. Additionally, no Grade 4 toxicities.
- PNT2258 drug exposures levels (AUC) exceeded by at least four-fold that required for anti-tumor activity in xenograft studies of human tumors, consistent with the Phase I study.
- Preliminary pharmacodynamic markers demonstrated on-target BCL2 activity, including lymphocyte and platelet effects.
- With the promising results in DLBCL and FL patients, additional PNT2258 single-agent and combination studies are planned.

“Patient with symptomatic disease experienced significant improvement in quality of life as their disease responded to treatment. Infusions were very well tolerated without any significant immediate or cumulative toxicities. The complete responses and durability of responses observed to date suggest that PNT2258 may become a valuable new therapy for NHL patients who have very limited treatment options,” commented study investigator Dr. Wael Harb, MD, Horizon Oncology Research, Lafayette, Indiana.

“These results reinforce our enthusiasm for PNT2258 and its potential to treat hematologic tumors,” said Mina Sooch, CEO of ProNAi. “In particular, the data presented at ASH are the first to show systemic, clinical activity by a DNAi drug. We look forward to expanding the current trial and initiating new studies in NHL patients in 2014. In addition, our first peer-reviewed publication has just been accepted this December in Cancer Chemotherapy and Pharmacology titled: *A Phase 1 study of the BCL2 targeted deoxyribonucleic acid inhibitor PNT2258 in patients with advanced solid tumors*. 2013 was a milestone year for ProNAi as we clinically validated our DNAi drug delivery platform and advanced a new BCL2 targeted drug candidate with potential synergies to current and many new lymphoma therapies.”

About PNT2258

PNT2258 is a 24-base, single-stranded, chemically-unmodified DNA oligonucleotide called PNT100 that is encapsulated in a specialized anionic and pH “tunable” liposome (SMARTICLES®). Robust preclinical activity of PNT2258 has been demonstrated as a single agent and in combination with CD20-targeted

antibody therapy and other chemotherapeutic agents in a variety of hematological and solid tumor xenograft models. PNT2258 exhibits broad and safe systemic exposure and cellular uptake, resulting in cell death by modulation of the BCL2 gene.

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. Beyond PNT2258, the company has a broad pipeline of DNAi leads for over 30 cancer and non-cancer targets, including CMYC and KRAS. ProNAi's business strategy is to establish multiple 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.

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