Neon Therapeutics Logo

Neon Therapeutics Presents Updated Data from Ongoing Phase 1b NT-001 Clinical Trial of Personal Neoantigen Vaccine NEO-PV-01 at AACR 2019 Annual Meeting

March 31, 2019

Post-Vaccine Immune and Pathologic Markers Associated with Durable Clinical Benefit in Metastatic Melanoma Patient Cohort

Top-line Results, Including 52-Week Clinical Outcomes, from Full NT-001 Trial in Melanoma, Non-Small Cell Lung and Bladder Cancers Expected Later in 1H 2019

CAMBRIDGE, Mass., March 31, 2019 (GLOBE NEWSWIRE) -- Neon Therapeutics, Inc. (Nasdaq: NTGN), a clinical-stage immuno-oncology company developing neoantigen-based therapeutics, today presented updated data describing the immune and pathologic markers associated with durable clinical benefit in patients enrolled in NT-001, an ongoing Phase 1b clinical trial evaluating NEO-PV-01 in combination with nivolumab (OPDIVO® or anti-PD-1 therapy). The data were highlighted in an oral presentation titled, "*The Personalized Vaccine, NEO-PV-01 with Anti-PD-1, Induces Neoantigen-Specific De Novo Immune Responses in Patients with Advanced or Metastatic Melanoma: Association with Clinical Outcomes*," at the American Association for Cancer Research (AACR) Annual Meeting in Atlanta.

NEO-PV-01 is a personal neoantigen vaccine custom-designed and manufactured based on the neoantigens identified by Neon's proprietary bioinformatics engine, RECON®, as being the most therapeutically relevant for an individual patient. The data presented today, which reflect a cutoff date of August 31, 2018, include 23 patients with metastatic melanoma who received at least one dose of NEO-PV-01 and who either remained progression-free or had progressed by week 36 after the initiation of anti-PD-1 therapy. Patients who did not progress by 36 weeks were classified as having durable clinical benefit (DCB). The data indicate that RECON-based prediction of neoantigen quality correlates with DCB and serves as clinical validation for RECON's ability to identify therapeutically relevant neoantigens.

"Our new analysis has shown two post-vaccine markers, epitope spread and tumor pathology, that clearly associate with durable clinical benefit. Of the patients tested, we observed epitope spread – which is a cascade of neoantigen recognition beyond those included in the vaccine – in all patients with DCB. Furthermore, the majority of patients with DCB displayed marked reductions in tumor content only after vaccination. We are encouraged by these associations and look forward to evaluating patient outcomes, including clinical responses and progression-free survival, in the more mature 52-week topline data set expected mid-year," said Richard Gaynor, M.D., President of Research and Development at Neon Therapeutics.

The analysis presented at AACR both confirms mechanism of action of NEO-PV-01 and highlights new data on the relationships between multiple histological and molecular markers and DCB in the melanoma cohort. Together these findings show that:

- RECON-based prediction of high-quality neoantigens was correlated with DCB
- Durable, neoantigen-specific peripheral immune responses were found only post-vaccine
- Vaccine-induced T cells trafficked to the tumor and were capable of killing tumor cells
- In patients tested, epitope spread was detected in all patients with DCB and only post vaccine
- Post-vaccine biopsies showed marked reduction in tumor content, which was associated with DCB
- Tumor microenvironment analysis indicated that major histocompatibility complex class II expression, B cell gene signature, a natural killer cell gene signature and TCF7-positive CD8 cells were all correlated with DCB, and may play an important role for patient selection in future studies
- Several metrics that typically correlate with patient response to checkpoint inhibitors, including tumor mutation burden and tumor PD-L1, were not correlated with DCB in this analysis

Each of the Company's presentations at AACR 2019 will be made available in the Publications section of Neon Therapeutics' website at **neontherapeutics.com/publications.**

About NEO-PV-01

NEO-PV-01 is a fully personal cancer vaccine targeting neoantigens that is custom-designed and manufactured for each individual patient based upon the tumor's unique mutational fingerprint. The neoantigen-targeting peptides in NEO-PV-01 are intended to generate an anti-tumor immune response that directs patients' T cells to target and kill their cancer cells. NEO-PV-01 is being studied in multiple ongoing Phase 1 clinical trials.

About NT-001

NT-001 is a Phase 1b study evaluating Neon Therapeutics' personal neoantigen vaccine, NEO-PV-01, in combination with nivolumab in checkpoint-naïve patients with metastatic melanoma, smoking-associated NSCLC or bladder cancer. The primary endpoint of the study is safety. In addition, Neon is evaluating immune responses and clinical outcomes, including clinical responses and progression-free survival.

About Neon Therapeutics

Neon Therapeutics is a clinical-stage immuno-oncology company and a leader in the field of neoantigen-targeted therapies, dedicated to transforming the treatment of cancer by directing the immune system towards neoantigens. Neon is using its neoantigen platform to develop both vaccine and T cell therapies, including NEO-PV-01, a clinical-stage neoantigen vaccine for the treatment of metastatic melanoma, non-small cell lung cancer, and bladder cancer; NEO-PTC-01, a neoantigen T cell therapy for the treatment of solid tumors; and NEO-SV-01, a neoantigen vaccine for the treatment of a subset of estrogen-receptor-positive breast cancer.

For more information, please visit **www.neontherapeutics.com**.

Forward-Looking Statements

This press release contains “forward-looking statements” of Neon Therapeutics, Inc. within the meaning of the Private Securities Litigation Reform Act of 1995. These forward-looking statements may include, but may not be limited to, express or implied statements regarding our ability to obtain and maintain regulatory approval of our product candidates; the potential timing and advancement of our preclinical studies and clinical trials and related regulatory submissions; the potential timing of data readouts from our ongoing and planned clinical trials; the design and potential efficacy of our therapeutic approaches; the ability and willingness of our third-party research institution collaborators to continue research and development activities relating to our product candidates; our ability and the potential to successfully manufacture and supply our product candidates for clinical trials; our ability to replicate results achieved in our preclinical studies or clinical trials in any future studies or trials; regulatory developments in the United States and foreign countries; our expectations regarding our ability to obtain and maintain intellectual property protection for our product candidates; and our expectations regarding our uses of capital, expenses, future accumulated deficit and other financial results. Any forward-looking statements in this press release are based on management’s current expectations and beliefs of future events, and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in or implied by such forward-looking statements. These risks and uncertainties include, but are not limited to: uncertainties related to the initiation, timing and conduct of studies and other development requirements for our product candidates; the risk that any one or more of our product candidates will not be successfully developed and commercialized; the risk that the results of preclinical studies and clinical trials will be predictive of future results in connection with future studies or trials; the risk that Neon’s collaborations will not continue or will not be successful; risks related to our ability to protect and maintain our intellectual property position; and risks related to the ability of our licensors to protect and maintain their intellectual property position. For a discussion of these and other risks and uncertainties, and other important factors, any of which could cause Neon’s actual results to differ from those contained in the forward-looking statements, see the section entitled “Risk Factors” in Neon’s most recent Annual Report on Form 10-K, as filed with the Securities and Exchange Commission, as well as discussions of potential risks, uncertainties, and other important factors in Neon’s other filings with the Securities and Exchange Commission. All information in this press release is as of the date of the release, and Neon undertakes no duty to update this information unless required by law.

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