NeOnc Technologies Reports Positive Topline Phase 2a Results for Intranasal NEO100 in Recurrent IDH1-Mutant High-Grade Glioma

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Company to request a Type B meeting with the FDA to align on a registrational path in a setting with no approved targeted therapy

— Study met its primary endpoint: six-month progression-free survival of 48.9% versus a pre-specified 20% benchmark for standard of care (p = 0.0047)

— Median overall survival of 26.09 months; 86.7% of patients alive at six months

— Five of 24 patients remain on treatment, with one partial response ongoing beyond 114 days

— No major toxicities reported across the cohort; adverse events predominantly low-grade

— Company to host investor conference call and webcast today at 8:30 a.m. Eastern Time

CALABASAS, Calif., Aug. 12, 2026 (GLOBE NEWSWIRE) -- NeOnc Technologies Holdings, Inc. (Nasdaq: NTHI) ("NeOnc" or the "Company"), a multi-Phase 2 clinical-stage biopharmaceutical company developing novel therapies for central nervous system (CNS) cancers, today announced positive topline results from the Phase 2a portion of NEO100-01, an open-label study of intranasal NEO100™ (purified perillyl alcohol) in patients with recurrent or progressive Grade III and Grade IV IDH1-mutant glioma.

The study met its primary endpoint. Six-month progression-free survival (PFS-6) was **48.9%** (95% CI: 26.3-68.1) by RANO 2.0 criteria using Kaplan-Meier estimation, compared with the 20% rate pre-specified in the study design as the expectation for standard of care (p = 0.0047).

"These results represent an important milestone for NeOnc and, more importantly, a source of hope for patients with recurrent high-grade glioma who currently have very limited treatment options. NEO100 met the study's primary endpoint, demonstrated encouraging survival outcomes, and was administered intranasally by patients at home with no major toxicities reported. We believe these findings support the potential of our intranasal delivery platform to address one of the greatest challenges in treating brain cancer - the blood-brain barrier. Our priority now is to engage with the FDA and align on the most efficient path toward a registrational study," said Amir F. Heshmatpour, Executive Chairman, President and Chief Executive Officer.

Secondary survival endpoints were supportive. Median overall survival was **26.09 months**, with 12 deaths among 24 patients. Overall survival was **86.7%** at six months (95% CI: 64.3-95.5), 60.9% (95% CI: 36.4-78.4) at 12 months and 54.1% (95% CI: 29.5-73.4) at 24 months.

"We believe that these results are a powerful validation of the science behind NEO100," said Thomas C. Chen, MD, PhD, Founder, Chief Medical Officer and Chief Scientific Officer of NeOnc. "We set out to reach the brain directly, delivering therapy through the nose and along the olfactory pathway rather than forcing a drug past the blood-brain barrier, and what we are seeing in Phase 2a is what our biology predicted and what we saw in Phase 1, where every long-term survivor carried an IDH1 mutation. We believe that survival of this duration in twice-treated, high-grade disease, achieved with a therapy patients take at home and with no major toxicity, is the kind of result that justifies a much larger study."

Evidence of durable disease control was observed across the cohort. Five of 24 patients remain on active treatment. One patient has remained progression-free for approximately 19 months. A second patient achieved a partial response sustained for 114 days through the end of Cycle 8 and remains on treatment in response. Objective response rate was 8.3% (two of 24 patients) by RANO 2.0.

"In recurrent high-grade glioma, the outcome that matters is how long a patient can hold the disease at bay and still live their life," said Josh Neman, PhD, Chief Clinical Officer of NeOnc. "Five of our 24 patients remain on therapy, one progression-free approaching 19 months and another in an ongoing response approaching four months, and they are taking this treatment at home rather than in an infusion chair. Durability and tolerability together are rare at recurrence, and we believe that combination is what these data point to. We look forward to discussing the findings with the FDA."

The data suggests that NEO100 was well tolerated. No major toxicities were reported across the cohort, and adverse events to

date have been predominantly low-grade. The tolerability profile is consistent with the Phase 1 portion of the study, in which the data suggested that NEO100 was well tolerated at all dose levels, with no severe or dose-limiting toxicities observed. The absence of significant toxicity is notable in a population receiving continuous therapy (several patients have now remained on daily intranasal dosing for well over a year) and reflects a delivery route designed to reach the brain without systemic cytotoxic exposure.

NEO100-01 is directed at a population that existing IDH-targeted therapies do not serve. Approved and late-stage IDH-targeted agents for glioma have been developed in the front-line setting for lower-grade disease, typically Grade 2, non-enhancing tumors in patients who have undergone surgery but have not yet received radiation or chemotherapy.

NEO100-01 enrolled the opposite population: patients with Grade III and Grade IV IDH1-mutant tumors that have recurred or progressed after radiation and temozolomide. There is no approved targeted therapy for these patients. Approximately 90% of high-grade glioma patients recur within six to nine months of maximal therapy, and at recurrence, surgery is often not repeatable and systemic agents face rapid resistance and cumulative toxicity.

NEO100 is administered intranasally by the patient at home, four times daily, in 28-day cycles, a delivery route designed to reach the brain directly while avoiding systemic cytotoxic exposure.

NeOnc intends to request a Type B meeting with the U.S. Food and Drug Administration to align on a registrational development path for NEO100 in recurrent IDH1-mutant high-grade glioma. Additional pre-specified analyses including the Grade III versus Grade IV subgroup analysis, pharmacokinetics, and quality-of-life measures are ongoing and will be reported separately. The Company expects to present the full Phase 2a dataset, including detailed safety, at a future medical meeting.

Conference Call and Webcast

NeOnc will host an investor conference call and webcast today at 5:30 a.m. Pacific Time / 8:30 a.m. Eastern Time to discuss these results, followed by a question and answer session. The live webcast can be accessed at https://www.webcaster5.com/Webcast/Page/3151/54410 or by visiting https://investors.neonc.com. A replay will be available at https://investors.neonc.com shortly following the conclusion of the call.

About the NEO100-01 Phase 2a study

NEO100-01 is an open-label, multi-center Phase 1/2a study of intranasal NEO100 in patients with radiographically confirmed progression of, or recurrence of, primary or secondary Grade IV glioma or Grade III astrocytoma harboring an IDH1 mutation. All patients had previously failed radiation or combined temozolomide and radiation. The Phase 2a portion enrolled 24 patients of a planned 28 at the recommended Phase 2 dose of 1,152 mg/day, self-administered intranasally four times daily in 28-day cycles until progression, death, or withdrawal.

The primary endpoint is the progression-free survival rate at six months. Secondary endpoints include objective response rate by RANO 2.0 criteria, progression-free survival, overall survival, safety and tolerability, pharmacokinetics, and quality of life. All MRI scans were read by an independent central reviewer. Biostatistical analysis was conducted by Anova Enterprises, Inc. Efficacy results are reported for the intent-to-treat population. Response and progression were assessed using RANO 2.0 criteria.

About NEO100

NEO100™ is a patented, ultra-pure pharmaceutical-grade formulation of perillyl alcohol, a naturally occurring monoterpene found in citrus and peppermint oils, produced through a proprietary crystalline synthesis process. Administered intranasally using a commercial nasal mask and nebulizer, NEO100 is designed to deliver therapy directly to the brain along olfactory and trigeminal pathways, bypassing the blood-brain barrier and avoiding first-pass metabolism and systemic toxicity. Data from preclinical studies suggests that NEO100 may transiently and reversibly open the blood-brain barrier, enabling brain entry of otherwise impermeable therapeutics.

About NeOnc Technologies Holdings, Inc.

NeOnc Technologies Holdings, Inc. is a clinical-stage life sciences company focused on the development and commercialization of central nervous system therapeutics that are designed to address the persistent challenges in overcoming the blood-brain barrier. The company's NEO™ drug development platform has produced a portfolio of novel drug candidates and delivery methods with patent protections extending to 2038. These proprietary chemotherapy agents have demonstrated positive effects in laboratory tests on various types of cancers and in clinical trials treating malignant gliomas. NeOnc's NEO100™ and NEO212™ therapeutics are in Phase II human clinical trials and are advancing under FDA Fast-Track and Investigational New Drug (IND) status. The company has exclusively licensed an extensive worldwide patent portfolio from the University of Southern California consisting of issued patents and pending applications related to NEO100, NEO212, and other products from the NeOnc patent family for multiple uses, including oncological and neurological conditions.

For more about NeOnc and its pioneering technology, visit https://neonc.com.

Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995,

including statements regarding the potential of NEO100, the interpretation and significance of the Phase 2a results, plans to engage with the FDA, the design and timing of future clinical trials, and the Company's development strategy. These statements are based on management's current expectations and are subject to known and unknown risks and uncertainties that may cause actual results to differ materially.

Such risks include, but are not limited to: the Phase 2a study was a single-arm, open-label study of 24 patients that did not reach its planned enrollment of 28 patients and was not powered as a confirmatory trial; results were not compared against a randomized control arm, and comparisons to historical data are inherently limited by differences in patient population, era, and assessment criteria; the primary efficacy analysis presented reflects RANO 2.0 criteria and Kaplan-Meier estimation, and results differ under the response criteria and estimation method specified in the study protocol; early-phase results frequently fail to replicate in larger, controlled studies; safety and tolerability findings in a 24-patient, open-label study may not predict the safety profile observed in larger or longer-duration studies, and additional adverse events may emerge with broader exposure; the FDA may not agree with the Company's proposed registrational path, endpoints, or analytical methods; and additional pre-specified analyses remain ongoing. Additional risks are described in the Company's filings with the U.S. Securities and Exchange Commission.

The Company undertakes no obligation to update any forward-looking statement except as required by law.

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