Plus Therapeutics Granted U.S. FDA Orphan Drug Designation to REYOBIQ™ in Pediatric Malignant Gliomas

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HOUSTON, April 08, 2026 (GLOBE NEWSWIRE) -- Plus Therapeutics, Inc. (Nasdaq: PSTV) (the "Company"), a clinical-stage pharmaceutical company developing targeted radiotherapeutics with advanced platform technologies for central nervous system (CNS) cancers, today announced that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation (ODD) to REYOBIQ™ (rhenium Re186 obisbemeda) for the treatment of pediatric malignant gliomas.

Pediatric malignant gliomas are rare, aggressive brain tumors with limited treatment options and poor outcomes, where current standards of care—including surgery and radiation—often fail to prevent recurrence.

Notably, the FDA granted orphan designation for malignant glioma more broadly than originally requested, encompassing pediatric ependymoma.

"Receiving orphan drug designation for REYOBIQ in pediatric malignant gliomas, including the broader scope for progressive pediatric ependymoma, is an important milestone and further validates our approach to delivering targeted radiotherapy directly to CNS tumors," said Marc Hedrick, M.D., President and Chief Executive Officer of Plus Therapeutics. "We believe REYOBIQ's ability to deliver high-dose radiation precisely to tumor sites while minimizing exposure to healthy brain tissue has the potential to meaningfully improve outcomes in this underserved patient population. This orphan designation reinforces the potential applicability of REYOBIQ across a wider range of CNS tumor indications and our continued advancement of REYOBIQ across multiple CNS cancer indications."

Orphan Drug Designation is granted by the FDA to investigational therapies intended to treat rare diseases affecting fewer than 200,000 people in the United States. The designation provides several potential benefits, including seven years of market exclusivity upon approval, tax credits for qualified clinical trial expenses, and exemptions from certain regulatory fees.

The ODD for pediatric malignant gliomas builds on recent regulatory and clinical progress for REYOBIQ, including completion of a Type B meeting with the FDA supporting development plans in leptomeningeal metastases (LM), encouraging clinical data from the ReSPECT-LM trial, and ongoing advancement of Phase 1 and Phase 2 studies. In addition, the Company has received FDA clearance of its Investigational New Drug (IND) application to evaluate REYOBIQ in pediatric patients with high-grade glioma and ependymoma.

About Pediatric Malignant Gliomas
Pediatric malignant gliomas are high-grade brain tumors that form from glial cells in the central nervous system that tend to grow quickly in children. Pediatric high grade gliomas (HGG), including ependymoma, are rare (approximately 3.3 cases per 100,000 persons) but aggressive brain tumors with limited treatment options and poor prognosis, particularly in recurrent settings. Standard treatments, including surgical resection and external beam radiation therapy, often fail to prevent recurrence, with 5-year survival rates as low as 22% for HGG, depending on tumor grade and resection extent.

About Leptomeningeal Metastases
LM is a rare complication of cancer in which the primary cancer spreads to the cerebrospinal fluid (CSF) and leptomeninges surrounding the brain and spinal cord. All malignancies originating from solid tumors, primary brain tumors, or hematological malignancies have this LM complication potential with breast cancer as the most common cancer linked to LM, with 3-5% of breast cancer patients developing LM. Additionally, lung cancer, GI cancers and melanoma can also spread to the CSF and result in LM. LM occurs in approximately 5% of people with cancer and is usually terminal with 1-year and 2-year survival of just 7% and 3%, respectively. The incidence of LM is on the rise, partly because cancer patients are living longer and partly because many standard chemotherapies cannot reach sufficient concentrations in the spinal fluid to kill the tumor cells, yet there are no FDA-approved therapies specifically for LM patients, who often succumb to this complication within weeks to several months, if untreated.

About REYOBIQ™ (rhenium Re186 obisbemeda)
REYOBIQ (rhenium Re186 obisbemeda) is a novel injectable radiotherapy specifically formulated to deliver direct targeted high dose radiation in CNS tumors in a safe, effective, and convenient manner to optimize patient outcomes. REYOBIQ has the potential to reduce off target risks and improve outcomes for CNS cancer patients, versus currently approved therapies, with a more targeted and potent radiation dose. Rhenium-186 is an ideal radioisotope for CNS therapeutic applications due to its short half-life, beta energy for destroying cancerous tissue, and gamma energy for real-time imaging. REYOBIQ is being evaluated for the treatment of recurrent glioblastoma, leptomeningeal metastases, and pediatric brain cancer in the ReSPECT-GBM, ReSPECT-LM, and ReSPECT-PBC clinical trials. ReSPECT-GBM is supported by an award from the National Cancer Institute (NCI), part of the U.S. National Institutes of Health (NIH), and ReSPECT-LM is funded by a three-year $17.6M grant by the Cancer Prevention & Research Institute of Texas (CPRIT). The Company's ReSPECT-PBC clinical trial for pediatric brain cancer is supported by a $3 million grant from the U.S. Department of Defense's Peer Reviewed Cancer Research Program.

About CNSide Diagnostic, LLC
CNSide Diagnostics, LLC is a wholly owned subsidiary of Plus Therapeutics, Inc. that develops and commercializes proprietary laboratory-developed tests, such as CNSide®, designed to identify tumor cells that have metastasized to the central nervous system in patients with carcinomas and melanomas. The CNSide® CSF Assay Platform enables quantitative analysis of the cerebrospinal fluid that informs and improves the management of patients with leptomeningeal metastases.

About Plus Therapeutics
Headquartered in Houston, Texas, Plus Therapeutics, Inc. is a clinical-stage pharmaceutical company developing targeted radiotherapeutics for difficult-to-treat cancers of the central nervous system with the potential to enhance clinical outcomes. Combining image-guided local beta radiation and targeted drug delivery approaches, the Company is advancing a pipeline of product candidates with lead programs in leptomeningeal metastases

(LM) and recurrent glioblastoma (GBM). The Company has built a supply chain through strategic partnerships that enable the development, manufacturing, and future potential commercialization of its products. For more information, visit https://www.plustherapeutics.com.

Forward-Looking Statements

This press release contains statements that may be deemed "forward-looking statements" within the meaning of U.S. securities laws, including statements regarding clinical trials, expected operations and upcoming developments. All statements in this press release other than statements of historical fact are forward-looking statements. These forward-looking statements may be identified by future verbs, as well as terms such as "expect" "potential," "anticipating," "planning" and similar expressions or the negatives thereof. Such statements are based upon certain assumptions and assessments made by management in light of their experience and their perception of historical trends, current conditions, expected future developments and other factors they believe to be appropriate.

The forward-looking statements included in this press release could differ materially from those expressed or implied by these forward-looking statements because of risks, uncertainties, and other factors that include, but are not limited to, the following: statements relating to the significance of the FDA ODD grant to the advancement of REYOBIQ as potential treatment of patients who suffer from for pediatric malignant gliomas; statements regarding the potential promise of REYOBIQ, including the next steps in developing the Company's product candidates; and, the Company's clinical trials, including statements regarding the Company's engagement with the FDA with respect to the possibility of defining a pivotal trial strategy for certain of the Company's clinical trials. This list of risks, uncertainties, and other factors is not complete. Plus Therapeutics discusses some of these matters more fully, as well as certain risk factors that could affect Plus Therapeutics' business, financial condition, results of operations, and prospects, in its reports filed with the SEC, including Plus Therapeutics' annual report on Form 10-K for the fiscal year ended December 31, 2024, quarterly reports on Form 10-Q, and current reports on Form 8-K. These filings are available for review through the SEC's website at www.sec.gov. Any or all forward-looking statements Plus Therapeutics makes may turn out to be wrong and can be affected by inaccurate assumptions Plus Therapeutics might make or by known or unknown risks, uncertainties, and other factors, including those identified in this press release. Accordingly, you should not place undue reliance on the forward-looking statements made in this press release, which speak only as of its date. The Company assumes no responsibility to update or revise any forward-looking statements to reflect events, trends or circumstances after the date they are made unless the Company has an obligation under U.S. federal securities laws to do so.

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