



Curtana Pharmaceuticals Receives FDA Rare Pediatric Disease Designation for CT-179 in Pediatric High-Grade Glioma

Designation for first-in-class OLIG2 inhibitor highlights the urgent need for new therapies in aggressive pediatric brain cancers and qualifies Curtana for a potential Priority Review Voucher upon FDA approval.

Austin, TX – April 23, 2026 – Curtana Pharmaceuticals, a clinical-stage biotechnology company focused on improving outcomes for brain cancer patients, today announced that the U.S. Food and Drug Administration (FDA) has granted Rare Pediatric Disease Designation (RPDD) to the company's lead therapeutic candidate, CT-179, for the treatment of pediatric-type diffuse high-grade gliomas (pHGG). CT-179 is a first-in-class, brain-penetrant, orally bioavailable small-molecule inhibitor targeting the oligodendrocyte transcription factor 2 (OLIG2).

Pediatric-type diffuse high-grade gliomas, which include H3 K27-altered Diffuse Midline Gliomas (DMG), are universally fatal World Health Organization (WHO) Grade 4 malignancies. The FDA's decision to grant this designation is based on the finding that pHGG is a serious or life-threatening disease in which the life-threatening manifestations, such as mortality, primarily affect individuals from birth to 18 years of age, and that the disease meets the statutory definition of a rare condition. The prognosis for these patients is devastating, with the median survival for DMG patients typically falling under one year.

Research has shown that OLIG2 is highly expressed in primary brain cancers, including pHGG, and acts as an essential factor in tumorigenesis by driving tumor growth, invasion into normal brain tissue, and recurrence. In these aggressive pediatric tumors, OLIG2 functions as a master regulator that maintains neural stem cell pluripotency and prevents terminal differentiation. CT-179 is specifically designed to disrupt this process by acting as a highly potent and selective inhibitor of OLIG2, thereby targeting the resilient cancer stem cells that evade standard therapies.

The FDA grants RPDD to incentivize the development of novel therapeutics for serious and rare pediatric diseases. Under this program, if a New Drug Application (NDA) for CT-179 is approved for this indication, Curtana may be eligible to receive a Rare Pediatric Disease Priority Review Voucher (PRV). A PRV can be redeemed to obtain expedited priority review for any subsequent marketing application, or it may be sold or transferred to another sponsor. Recent secondary market transactions for PRVs have consistently valued these vouchers between \$150 million and \$200 million, representing a transformative, non-dilutive monetization pathway for the company.

This marks the second Rare Pediatric Disease Designation granted to CT-179, following a prior designation for the treatment of medulloblastoma. This dual-designation status underscores the broad therapeutic potential of OLIG2 inhibition across multiple devastating pediatric brain cancers.

This regulatory milestone strongly aligns with Curtana's strategic clinical roadmap. To accelerate time to clinical proof-of-concept, the company is currently in discussions with the Pacific Pediatric Neuro-Oncology Consortium (PNOC) on how best to advance CT-179 into clinical development for pediatric brain cancers. Collaborating with a premier research network will be crucial to addressing the profound unmet medical needs of this vulnerable patient population.



Leadership Perspectives

"Receiving Rare Pediatric Disease Designation from the FDA is a significant regulatory milestone for Curtana and a critical validation of our approach to treating some of the most aggressive and intractable pediatric brain cancers" said Gregory Stein, M.D., M.B.A., Chief Executive Officer of Curtana Pharmaceuticals. "Children diagnosed with high-grade gliomas and diffuse midline gliomas are in desperate need of innovative treatments. CT-179 targets the fundamental drivers of these tumors, and this designation accelerates our momentum as we work to advance CT-179 into pediatric clinical trials to improve outcomes for these patients."

About Curtana Pharmaceuticals

Curtana Pharmaceuticals, founded in 2013, is a privately held, clinical-stage biopharmaceutical company headquartered in Austin, Texas. Current investors include Thynk Capital, angelMD, Biosense Global, DEFTA Partners, and other anonymous investors. The company was also awarded a \$7.6 million grant from the Cancer Prevention and Research Institute of Texas (CPRIT). Curtana focuses on the development of novel first-in-class, small molecule therapeutics targeting cancer stem cells in the central nervous system for the treatment of glioblastoma, low grade glioma, medulloblastoma, diffuse midline glioma, and other brain cancers. For more information, visit <https://www.curtanapharma.com/>.

Forward-Looking Statements

This press release contains forward-looking statements regarding the clinical development and potential commercialization of CT-179. These statements include projections regarding the launch of the multi-site Phase 1 OPAL study evaluating CT-179 in adult patients with glioblastoma, the clinical development trajectory of CT-179 for pediatric brain cancers, and the potential award and monetization of a Rare Pediatric Disease Priority Review Voucher upon future FDA approval. These forward-looking statements involve risks and uncertainties, including the inherent challenges of patient accrual in rare disease populations, the complexities of clinical trial execution, and the necessity of securing targeted capital raises to fund ongoing operations and clinical milestones.