



## **Curtana Pharmaceuticals' CT-179 Receives Rare Pediatric Disease Designation from the U.S. FDA for the Treatment of Medulloblastoma**

AUSTIN, TX – October 6, 2020 – Curtana Pharmaceuticals, a privately-held, preclinical stage biopharmaceutical company, today announced that the Food and Drug Administration (FDA) has granted Rare Pediatric Disease (RPD) designation to CT-179, the company's lead Olig2 inhibitor, for the treatment of medulloblastoma (MB). With the RPD designation, Curtana Pharmaceuticals may be eligible for a Priority Review Voucher (PRV) that can be used for a subsequent marketing application and may be sold or transferred. The US Government Accountability Office reports that, between 2017 and 2019, the sales price of a PRV has ranged between \$80 million and \$130 million.

Medulloblastoma (MB) is the most common type of primary brain cancer in children. According to the National Organization for Rare Disorders (NORD), approximately 1,000 new cases are diagnosed in children and adults each year in the US, with the American Society of Clinical Oncology (ASCO) estimating that between 250 and 500 medulloblastoma cases are diagnosed in children. While some patients do quite well, recurrence following initial treatment occurs in 30 to 40% of patients and is typically fatal. Overall, the reported 5-year survival rate after recurrence is only about 25%.

"We are pleased that the FDA has recognized the potential for CT-179 to provide a significant therapeutic benefit to patients with medulloblastoma," said Gregory Stein, M.D., M.B.A. and Chief Executive Officer, Curtana Pharmaceuticals. "Medulloblastoma is a devastating disease which can have a very poor prognosis, particularly in patients with recurrent disease. CT-179, projected to enter the clinic in the second half of 2021, has been shown to significantly prolong survival in relevant animal models, especially when combined with the standard of care treatment of radiation therapy."

CT-179 is a highly potent and selective small molecule inhibitor of oligodendrocyte transcription factor 2 (Olig2), a transcription factor that is essential to normal early brain development, but is not actively expressed in the vast majority of normal adult brain cells or in normal tissues outside the brain. Olig2-expressing progenitors are distinct tumor-initiating cells during the onset of primary and recurrent MB and these Olig2-expressing progenitors are highly enriched in therapy-resistant and recurrent MB. It has been shown that high levels of Olig2 are correlated with poor prognosis. In animal studies, experiments have demonstrated that elimination of Olig2-expressing cells blocks tumor progression in a mouse model of MB. Thus, targeting Olig2 is a promising, potential therapeutic avenue for the treatment of MB.



FDA Orphan Drug designation for the treatment of malignant gliomas, including glioblastoma (GBM), was granted to CT-179 in August 2017. The drug is orally bioavailable, readily crosses the blood-brain barrier, achieves very high concentrations in the brain, and significantly prolongs survival in animal models of brain cancer. CT-179 represents a novel agent which selectively targets Olig2-expressing brain cancer cells in preclinical studies with great potential as an adjunctive therapy in the treatment of GBM, MB, and other brain cancers. An investigational new drug (IND) application filing is planned for the second half of 2021.

#### **About Curtana Pharmaceuticals**

Curtana Pharmaceuticals, founded in 2013, is a privately held, preclinical-stage biopharmaceutical company headquartered in Austin, Texas. Current investors include [Thynk Capital](#), angelMD, Biosense Global, DEFTA Partners, and other anonymous investors. In 2014, the company was 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, medulloblastoma, and other brain cancers. For more information, visit [www.curtanapharma.com](http://www.curtanapharma.com).

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