



Ziopharm Oncology Granted Rare Pediatric Disease Designation for Controlled IL-12 for the Treatment of DIPG

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BOSTON, Sept. 14, 2020 (GLOBE NEWSWIRE) -- [Ziopharm Oncology](#), Inc. ("Ziopharm" or "the Company") (Nasdaq:Ziop) today announced that the U.S. Food and Drug Administration (FDA) has granted Rare Pediatric Disease Designation to Ad-RTS-hIL-12 with veledimex (Controlled IL-12) for the investigational treatment of diffuse intrinsic pontine glioma (DIPG), a lethal brain tumor occurring in the pontine region of the brain. DIPG accounts for approximately 10 to 15 percent of all cases of brain tumors in children. The Rare Pediatric Disease Designation program is intended to encourage the development of new drugs and biologics for the prevention and treatment of rare pediatric diseases.

"We are delighted to have received the Rare Pediatric Disease Designation for Controlled IL-12 from the FDA. This milestone for Ziopharm emphasizes the significant unmet need for children living with DIPG," said Laurence Cooper, M.D., Ph.D., Chief Executive Officer of Ziopharm. "Currently, there are no viable treatment options for this type of brain tumor. We are working with the FDA to advance Controlled IL-12 as a new gene therapy for this aggressive disease, which has historically been largely seen as incurable."

The FDA grants Rare Pediatric Disease Designation for serious and life-threatening diseases that primarily affect children ages 18 years or younger and fewer than 200,000 people in the United States. If Ziopharm's Biologics License Application (BLA) for Controlled IL-12 in DIPG is approved, the Company may be eligible to receive a priority review voucher from the FDA, which can be redeemed to obtain priority review for any subsequent marketing application or may be sold or transferred to another company for their program.

About DIPG

In children, the incidence of brain cancer is approximately 4.84 per 100,000, according to the National Cancer Institute. Glioma in the pontine region of the brain, or DIPG, accounts for approximately 10-15 percent of all cases of pediatric brain tumors, with about 150-300 new diagnoses per year in the United States.¹ Median survival ranges from 8-11 months.² There are no curative options.

About Controlled IL-12 (Ad-RTS-hIL-12 plus veledimex)

Ziopharm's Controlled IL-12 platform is an investigational gene therapy designed to induce and control the production of human interleukin 12 (hIL-12), a master-regulator of the immune system. The Company has treated more than 175 patients, including more than 125 patients with rGBM, with Ad-RTS-hIL-12 plus veledimex and administered more than 1,300 doses of veledimex across three types of solid tumors, building a significant safety profile, mechanistic dataset and evidence of anti-tumor effects.

Controlled IL-12 is being studied in a phase 1/2 trial (NCT03330197) designed to evaluate the safety and tolerability of a single intratumoral injection of Ad-RTS-hIL-12 given with up to 14 days of oral veledimex in children with gliomas. Up to 12 patients with DIPG may be enrolled in phase 1 of the study, which is being conducted at leading pediatric cancer centers across the United States, including Lurie Children's Hospital in Chicago, Dana-Farber Cancer Institute in Boston and University of California in San Francisco.

About Ziopharm Oncology, Inc.

Ziopharm is developing non-viral and cytokine-driven cell and gene therapies that weaponize the body's immune system to treat the millions of people globally diagnosed with a solid tumor each year. With its multiplatform approach, Ziopharm is at the forefront of immuno-oncology with a goal to treat any type of solid tumor. Ziopharm's pipeline is built for commercially scalable, cost effective T-cell receptor T-cell therapies based on its non-viral *Sleeping Beauty* gene transfer platform, a precisely controlled IL-12 gene therapy, and rapidly manufactured *Sleeping Beauty*-enabled CD19-specific CAR-T program. The Company has clinical and strategic partnerships with the National Cancer Institute, The University of Texas MD Anderson Cancer Center and others. For more information, please visit www.ziopharm.com.

Forward-Looking Statements Disclaimer

This press release contains forward-looking statements as defined in the Private Securities Litigation Reform Act of 1995, as amended. Forward-looking statements are statements that are not historical facts, and in some cases can be identified by terms such as "may," "will," "could," "expects," "plans," "anticipates," and "believes." These statements include, but are not limited to, statements regarding the potential approval of a BLA for Controlled IL-12 for the treatment of DIPG and the receipt and benefits of a priority review voucher from the FDA. Although Ziopharm's management team believes that the expectations reflected in such forward-looking statements are reasonable, investors are cautioned that forward-looking information and statements are subject to various risks and uncertainties, many of which are difficult to predict and generally beyond the control of Ziopharm, that could cause actual results and developments to differ materially from those expressed in, or implied or projected by, the forward-looking information and statements. These risks and uncertainties include among other things, changes in regulations that limit the benefits of receiving a priority review voucher, the uncertainties inherent in research and development, future clinical data and analysis, including whether any of Ziopharm's product candidates such as Controlled IL-12 will advance further in the preclinical research or clinical trial process, including receiving clearance from the U.S. Food and Drug Administration or equivalent foreign regulatory agencies to conduct clinical trials and whether and when, if at all, they will receive final approval from the U.S. FDA or equivalent foreign regulatory agencies for the treatment of DIPG; the strength and enforceability of Ziopharm's intellectual property rights; competition from other pharmaceutical and biotechnology companies as well as risk factors discussed or identified in the public filings with the Securities and Exchange Commission made by Ziopharm, including those risks and uncertainties listed in Ziopharm's Quarterly Report on Form 10-Q filed by Ziopharm with the Securities and Exchange Commission. We are providing this information as of the date of this press release, and Ziopharm does not undertake any obligation to update or revise the information contained in this press release.

whether as a result of new information, future events or any other reason.

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¹ Source: DIPG.org. Accessed June 30, 2020.

² Hargrave D, Bartels U, Bouffet E. Diffuse brainstem glioma in children: critical review of clinical trials. Lancet Oncol. 2006 Mar;7(3):241-8.



Source: ZIOPHARM Oncology Inc