Day One Announces FDA Acceptance of NDA and Priority Review for Tovorafenib in Relapsed or Progressive Pediatric Low-Grade Glioma (pLGG)

October 30, 2023

Priority review granted with PDUFA target action date of April 30, 2024

BRISBANE, Calif., Oct. 30, 2023 (GLOBE NEWSWIRE) -- Day One Biopharmaceuticals (Nasdaq: DAWN) (“Day One” or the “Company”), a clinical-stage biopharmaceutical company dedicated to developing and commercializing targeted therapies for people of all ages with life-threatening diseases, today announced that the U.S. Food and Drug Administration (FDA) accepted its New Drug Application (NDA) for tovorafenib as a monotherapy in relapsed or progressive pediatric low-grade glioma (pLGG). The FDA has granted priority review and assigned a Prescription Drug User Fee Act (PDUFA) target action date of April 30, 2024. The FDA is not currently planning to hold an advisory committee meeting to discuss the application.

pLGG is the most common brain tumor diagnosed in children, with patients suffering profound tumor and treatment-associated morbidities that can impact their life trajectory. For the vast majority of patients in the relapsed setting, there is no standard of care and no approved therapy.

“We are pleased to be one step closer to achieving our mission of bringing a novel targeted therapy to children whose low-grade gliomas with BRAF alterations have relapsed or progressed,” said Jeremy Bender, Ph.D., chief executive officer of Day One. “We are grateful to the patients and their caregivers who participated in the FIREFLY-1 trial and look forward to continuing to collaborate with the FDA as we prepare to make this treatment more broadly available to those who need it.”

The NDA is based on results from the open-label, pivotal Phase 2 trial evaluating tovorafenib as a once-weekly monotherapy in patients aged 6 months to 25 years with relapsed or progressive pLGG.
Updated data was recently disclosed when the Company announced the completion of its rolling NDA submission on September 11, 2023. New, detailed data is expected to be presented at an upcoming medical conference.

Under the FDA’s Rare Pediatric Disease Priority Review Voucher program, the Company may receive a voucher if it receives an approval for an eligible indication, which can be redeemed to obtain priority review for a subsequent marketing application for a different product candidate.

Tovorafenib is an investigational therapy that is not approved for commercial use in any country.

About Pediatric Low-Grade Glioma
Pediatric low-grade glioma (pLGG) is the most common brain tumor diagnosed in children, accounting for 30% - 50% of all central nervous systems tumors. BRAF wild-type fusions are the most common cancer-causing genomic alterations in pLGG. These genomic alterations are also found in severe adult and pediatric solid tumors.

Pediatric low-grade glioma can impact a child’s health in many ways depending on tumor size and location, including vision loss and motor dysfunction. There are no approved therapies for the vast majority of patients with pLGG, and current treatment approaches are associated with potential acute and life-long adverse effects. While most children with pLGG survive their cancer, children who do not achieve remission following surgery may face years of increasingly aggressive treatment. Due to the indolent nature of pLGG, patients generally receive multiple years of systemic therapy.

About FIREFLY-1
FIREFLY-1 is evaluating tovorafenib as once-weekly monotherapy in patients aged 6 months to 25 years with relapsed or progressive pLGG harboring a known activating BRAF alteration. The trial is being conducted in collaboration with the Pacific Pediatric Neuro-Oncology Consortium (PNOC). The primary endpoint is overall response rate (ORR), defined as the proportion of patients with confirmed response based upon Response Asessment for Neuro-Oncology High Grade Glioma (RANO-HGG) criteria. Secondary and exploratory endpoints include the overall response rate based on Response Assessment in Pediatric Neuro-Oncology Low-Grade Glioma (RAPNO-LGG) criteria, Response Assessment for Neuro-Oncology Low-Grade Glioma (RANO-LGG) criteria and volumetric analyses, progression-free survival, safety, functional outcomes, and quality of life measures. RANO-HGG, RANO-LGG and RAPNO-LGG are assessed by blinded independent central review. Additional information about FIREFLY-1 may be found at ClinicalTrials.gov, using Identifier NCT04775485.

About the Pacific Pediatric Neuro-Oncology Consortium
The Pacific Pediatric Neuro-Oncology Consortium (PNOC) is an international consortium with study sites within the United States, Canada, Europe and Australia dedicated to bringing new therapies to children and young adults with brain tumors.

About Tovorafenib
Tovorafenib is an investigational, oral, brain-penetrant, highly-selective type II RAF kinase inhibitor designed to target a key enzyme in the MAPK signaling pathway, which is being investigated in primary brain tumors or brain metastases of solid tumors. Tovorafenib has been studied in over 325 patients to date and is currently under evaluation in two pivotal clinical trials for pLGG. Tovorafenib is also being evaluated alone or as a combination therapy for adolescent and adult patient populations with recurrent or progressive solid tumors with MAPK pathway aberrations (FIRELIGHT-1).

Tovorafenib has been granted Breakthrough Therapy and Rare Pediatric Disease designations by the U.S. Food and Drug Administration (FDA) for the treatment of patients with pLGG harboring an activating RAF alteration. Tovorafenib has also received Orphan Drug designation from the FDA for the treatment of malignant glioma, and from the European Commission (EC) for the treatment of glioma.

About Day One Biopharmaceuticals
Day One Biopharmaceuticals is a clinical-stage biopharmaceutical Company that believes when it comes to pediatric cancer, we can do better. We put kids first and are developing targeted therapies that deliver to their needs. Day One was founded to address a critical unmet need: the dire lack of therapeutic development in pediatric cancer. The Company's name was inspired by "The Day One Talk" that physicians have with patients and their families about an initial cancer diagnosis and treatment plan. Day One aims to re-envision cancer drug development and redefine what's possible for all people living with cancer—regardless of age—starting from Day One.

Day One partners with leading clinical oncologists, families, and scientists to identify, acquire, and develop important emerging cancer treatments. The Company's lead product candidate, tovorafenib, is an investigational, oral, brain-penetrant, highly-selective type II RAF kinase inhibitor. The Company's pipeline also includes pimasertib, an investigational, oral, highly-selective small molecule inhibitor of mitogen -activated protein kinases 1 and 2 (MEK-1/-2). Day One is based in Brisbane, California. For more information, please visit www.dayonebio.com or find the Company on LinkedIn or X/Twitter.

Cautionary Note Regarding Forward-Looking Statements
This press release contains "forward-looking" statements within the meaning of the "safe harbor" provisions of the Private Securities Litigation Reform Act of 1995, including, but not limited to: Day One's plans to develop cancer therapies, expectations from current clinical trials, the execution of the Phase 2 and Phase 3 clinical trials for tovorafenib as designed, any expectations about safety, efficacy, timing and ability to complete clinical trials, release data results, the ability of Day One to obtain regulatory approvals for and to commercialize tovorafenib and other candidates in development, and the ability of tovorafenib to treat pLGG or related indications.

Statements including words such as "believe," "plan," "continue," "expect," "will," "develop," "signal," "potential," or "ongoing" and statements in the future tense are forward-looking statements. These forward-looking statements involve risks and uncertainties, as well as assumptions, which, if they do not fully materialize or prove incorrect, could cause our results to differ materially from those expressed or implied by such forward-looking statements.

Forward-looking statements are subject to risks and uncertainties that may cause Day One's actual activities or results to differ significantly from those expressed in any forward-looking statement, including risks and uncertainties in this press release and other risks set forth in our filings with the Securities and Exchange Commission. These forward-looking statements speak only as of the date hereof and Day One specifically disclaims any obligation to update these forward-looking statements or reasons why actual results might differ, whether as a result of new information, future events or otherwise, except as required by law.

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