



Day One Receives Orphan Designation from the European Commission for DAY101 for the Treatment of Glioma

Jun 21, 2021

SOUTH SAN FRANCISCO, Calif., June 21, 2021 (GLOBE NEWSWIRE) -- Day One Biopharmaceuticals, a clinical-stage biopharmaceutical company dedicated to developing and commercializing targeted therapies for patients of all ages with genetically defined cancers, today announced that the European Commission has granted orphan designation for the Company's lead product candidate, DAY101, for the treatment of glioma. The designation was based on a positive opinion from the European Medicines Agency Committee for Orphan Medicinal Products.

DAY101 is an oral, brain-penetrant, highly-selective type II pan-RAF kinase inhibitor designed to target a key enzyme in the MAPK signaling pathway. Day One is conducting a pivotal Phase 2 clinical trial (FIREFLY-1) of DAY101 in pediatric, adolescent and young adult patients with recurrent or progressive low-grade glioma (pLGG) harboring a known BRAF alteration.

"Orphan designation for DAY101 from the European Commission exemplifies the urgent medical need for additional treatment options for glioma and builds upon the previous Breakthrough Therapy and Orphan Drug designations from the U.S. Food and Drug Administration for DAY101," said Davy Chiodin, PharmD, chief development officer of Day One. "We look forward to continue enrolling patients in the pivotal Phase 2 FIREFLY-1 study which has the potential to make DAY101 the first approved therapy for patients with pLGG."

About European Commission Orphan Designation

Orphan designation is granted by the European Commission to encourage development of medicines intended to treat a seriously debilitating or life-threatening condition that affects fewer than 5 in 10,000 people in the European Union. Orphan designation by the European Commission provides companies with certain benefits and incentives, including protocol assistance, reduced regulatory fees and up to 10 years of market exclusivity following regulatory approval.

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 system tumors. BRAF wild-type fusions are the most common cancer-causing genomic mutations in pediatric low-grade gliomas. These genomic alterations are also found in several adult solid tumors. Currently approved BRAF inhibitors are only active in tumors harboring BRAF V600 mutations, exhibit limited activity in brain tumors, and cannot be used in patients harboring BRAF fusions.

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 pLGG and current treatment approaches are associated with significant acute and life-long adverse effects. While most children with pLGG survive their cancer, children who do not achieve a cure following surgery face years of increasingly aggressive therapies that can have lasting effects on learning, cognition, and quality of life. Due to the indolent nature of pLGG, patients receive multiple years of systemic therapy.

About DAY101

DAY101 is an oral, brain-penetrant, highly-selective type II pan-RAF kinase inhibitor designed to target a key enzyme in the MAPK signaling pathway. Studies have shown DAY101 has high brain distribution and exposure in comparison to other MAPK pathway inhibitors, thus potentially benefiting patients with primary brain tumors or brain metastases of solid tumors. DAY101 is a type II RAF inhibitor found to selectively inhibit both monomeric and dimeric RAF kinase, which may broaden its potential clinical application to treat an array of RAF-altered tumors.

DAY101 has been studied in over 250 patients, and as a monotherapy demonstrated good tolerability and encouraging anti-tumor activity in pediatric and adult populations with specific MAPK pathway-alterations. In November 2020, Day One announced [preliminary results from PNOC014](#), an ongoing Phase 1 Pacific Pediatric Neuro-Oncology Consortium (PNOC) network study with DAY101 sponsored by the Dana-Farber Cancer Institute, in patients under 18 years of age with relapsed low-grade glioma. Preliminary results demonstrated that of the eight patients in the study with RAF fusions, two patients achieved a complete response by Response Assessment for Neuro-Oncology (RANO), three had a partial response, two achieved prolonged stable disease, and one experienced progressive disease. DAY101 also demonstrated a tolerable safety profile.

DAY101 has been granted Breakthrough Therapy designation by the U.S. Food and Drug Administration (FDA) for the treatment of patients with pLGG harboring an activating RAF alteration who require systemic therapy and who have either progressed following prior treatment or who have no satisfactory alternative treatment options. In addition, DAY101 has received Orphan Drug designation from the FDA for the treatment of malignant glioma and orphan designation from the European Commission for the treatment of glioma.

Day One is conducting a pivotal Phase 2 trial (FIREFLY-1) of DAY101 in pediatric, adolescent and young adult patients with pLGG. Day One also plans to study DAY101 alone or in combination with other agents that target key signaling nodes in the MAPK pathway, such as the Company's MEK inhibitor pimasertib, in patient populations where various RAS and RAF alterations are believed to play an important role in driving disease.

About Day One Biopharmaceuticals

Day One Biopharmaceuticals is a clinical-stage biopharmaceutical company dedicated to developing and commercializing targeted therapies for patients of all ages with genetically defined cancers. Day One was founded to address a critical unmet need: children with cancer are being left behind in a cancer drug development revolution. Our name was inspired by the "The Day One Talk" ¹ that physicians have with patients and their families about an initial cancer diagnosis and treatment plan. We aim 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, DAY101, is an oral, highly-selective type II pan-RAF kinase inhibitor, and is being evaluated in a pivotal Phase 2 clinical trial (FIREFLY-1) in pediatric, adolescent and young adult patients with recurrent or progressive low-grade glioma (pLGG). The Company's pipeline also includes the investigational agent pimasertib, a clinical-stage, oral, small molecule found to selectively inhibit mitogen-activated protein kinase kinases 1 and 2 (MEK). Through Day One and its collaborators, cancer drug development comes of age. Day One is based in South San Francisco. For more information, please visit www.dayonebio.com.

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 clinical trial for DAY101 as designed, any expectations about safety, efficacy, timing and ability to complete clinical trials and to obtain regulatory approvals for DAY101 and other candidates in development, and the ability of DAY101 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, including Day One's ability to develop, obtain regulatory approval for or commercialize any product candidate, Day One's ability to protect intellectual property, the potential impact of the COVID-19 pandemic and the sufficiency of Day One's cash, cash equivalents and investments to fund its operations. 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.

¹Jennifer W. Mack and Holcombe E. Grier; Journal of Clinical Oncology 2004 22:3, 563-566

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