Chimerix Announces Positive Topline Results for ONC201 in Recurrent H3 K27M-mutant Glioma

November 4, 2021

– Blinded Independent Central Review (BICR) of ONC201 Cohort Reported 20.0% Objective Response Rate (ORR) by RANO-HGG Criteria –

– Compelling Durability of Responses with 11.2 Month Median Duration of Response (mDOR) in Addition to an 8.3 Month Median Time to Response (mTTR) –

– Additional Data to be Presented at the Society for Neuro-Oncology (SNO) Annual Meeting –

– Conference Call at 8:30 a.m. ET Today –

DURHAM, N.C., Nov. 04, 2021 (GLOBE NEWSWIRE) -- Chimerix (NASDAQ:CMRX), a biopharmaceutical company whose mission it is to develop medicines that meaningfully improve and extend the lives of patients facing deadly diseases, today reported positive topline data from its 50-patient efficacy analysis of ONC201 for the treatment of recurrent H3 K27M-mutant glioma. ONC201 is an orally administered small molecule dopamine receptor D2 (DRD2) antagonist and caseinolytic protease (ClpP) agonist for the treatment of recurrent gliomas that harbor the H3 K27M mutation.

“These results are exciting, especially for this patient population with no good systemic therapeutic options,” said Dr. Isabel Arrillaga-Romany, MD, PhD, Director of Neuro-Oncology Clinical Trials, Massachusetts General Hospital Cancer Center. “The durability of responses in patients we would otherwise expect to progress rapidly is compelling.”

“These data confirm our expectations for the potential benefit for patients with this devastating disease,” said Mike Sherman, Chief Executive Officer of Chimerix. “We look forward to sharing additional data at the SNO conference later this month. The durability of responses are complemented by the consistency of data across other clinical endpoints. We believe this represents an attractive risk – benefit for patients who otherwise receive palliative care. On behalf of the entire Chimerix team, we thank the clinical collaborators and their patients who participated in our clinical trials with the hope of improving not only their own outcomes, but also the outcomes of future patients. None of the progress we have made would be possible without their support.”

An efficacy analysis by blinded independent central review (BICR) of the registration cohort determined the overall response rate (ORR) to be 20.0% (95% Confidence Interval (CI): 10.0-33.7%) as determined by Response Assessment in Neuro-Oncology Criteria for High Grade Gliomas (RANO-HGG). The median duration of response (mDOR) was 11.2 months (95% CI: 3.8 - not reached) and the median time to response (mTTR) was 8.3 months.

The cohort for a potential registration of ONC201 was comprised of the first 50 patients enrolled across five ONC201 clinical studies who met certain criteria. These patients were two years of age or older, had measurable diffuse midline glioma, their tumor harbored the H3 K27M mutation and had evidence of disease progression following prior therapy with at least radiation completed at least 90 days prior to enrollment, among certain other criteria.

The full BICR analysis will be presented at the Society for Neuro-Oncology (SNO) Annual Meeting on November 20, 2021. The plenary presentation will include additional supporting evidence of disease control, clinical benefit, including neurological improvements as measured by performance status, reduction in the use of corticosteroids, and an analysis of overall survival.

One serious adverse event was attributed by an investigator as possibly related to ONC201. Full safety data collection and analysis for this cohort is ongoing. Prior safety review of ONC201 identified the most commonly reported adverse events as nausea/vomiting, fatigue and decreased lymphocyte counts.

The FDA granted ONC201 Fast Track Designation for the treatment of adult recurrent H3 K27M-mutant high-grade glioma, Rare Pediatric Disease Designation for treatment of H3 K27M-mutant glioma, and Orphan Drug Designations for the treatment of glioblastoma and for the treatment of malignant glioma.

The Company plans to meet with the U.S. Food and Drug Administration in 2022 following completion of ongoing chemistry, manufacturing and controls (CMC) clinical pharmacology studies and natural disease history evaluation.

About Recurrent H3 K27M-mutant Glioma

Recurrent high-grade glioma is a form of brain cancer with a particularly poor prognosis. Pediatric patients with recurrent glioma that carries the H3 K27M mutation have an even worse prognosis. Gliomas with this mutation are considered Grade IV by the World Health Organization regardless of patient age.

Conference Call and Webcast

Chimerix will host a conference call and live audio webcast to discuss these data along with third quarter 2021 financial results today at 8:30 a.m. ET. To access the live conference call, please dial 877-354-4056 (domestic) or 678-809-1043 (international) at least five minutes prior to the start time and refer to conference ID 5935686.

A live audio webcast of the call will also be available on the Investors section of Chimerix’s website, www.chimerix.com. An archived webcast will be

available on the Chimerix website approximately two hours after the event.

About Chimerix

Chimerix is a biopharmaceutical company whose mission is to develop medicines that meaningfully improve and extend the lives of patients facing deadly diseases. In June 2021, the U.S. Food and Drug Administration granted approval of TEMBEXA for the treatment of smallpox as a medical countermeasure. The Company has two other advanced clinical-stage development programs, ONC201 and dociparstat sodium (DSTAT). ONC201 is in development for recurrent H3 K27M mutant glioma. DSTAT is in development as a potential first-line therapy in acute myeloid leukemia.

Forward-Looking Statements
This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995 that are subject to risks and uncertainties that could cause actual results to differ materially from those projected. Forward-looking statements include those relating to, among other things, the status of Chimerix's oncology programs, and the potential benefits and government procurement of TEMBEXA. Among the factors and risks that could cause actual results to differ materially from those indicated in the forward-looking statements are risks that the current clinical study data for ONC201 will not support accelerated, or any, regulatory approval; the anticipated benefits of the acquisition of Oncoceutics may not be realized; the ability to generate positive results in a Phase 3 study in acute myeloid leukemia and subsequent approval for DSTAT; risks that Chimerix will not obtain a procurement contract for TEMBEXA in smallpox in a timely manner or at all; Chimerix's reliance on a sole source third-party manufacturer for drug supply; risks that ongoing or future trials may not be successful or replicate previous trial results, or may not be predictive of real-world results or of results in subsequent trials; risks and uncertainties relating to competitive products and technological changes that may limit demand for our drugs; risks that our drugs may be precluded from commercialization by the proprietary rights of third parties; and additional risks set forth in the Company's filings with the Securities and Exchange Commission. These forward-looking statements represent the Company's judgment as of the date of this release. The Company disclaims, however, any intent or obligation to update these forward-looking statements.

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