# Chimerix Announces Positive ONC201 Data in Recurrent H3 K27M-mutant Diffuse Midline Glioma to Be Presented at the Society for Neuro-Oncology Annual Meeting

November 19, 2021

*– Plenary Session Presentation on Saturday, November 20 –*

*– Company to Host Conference Call at 8:30 a.m. ET on Monday, November 22 –*

DURHAM, N.C., Nov. 19, 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 announced the presentation of positive data from its 50-patient efficacy analysis of ONC201 for the treatment of recurrent H3 K27M-mutant diffuse midline 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 data will be presented by Isabel Arrillaga-Romany, MD, PhD, Director of Neuro-Oncology Clinical Trials, Massachusetts General Hospital Cancer Center tomorrow, November 20, 2021, during a plenary session at the Society for Neuro-Oncology (SNO) Annual Meeting in Boston, MA. In addition, Dr. Arrillaga-Romany will join management for a conference call on Monday, November 22, 2021 to give an additional overview of the data.

**Key Data Highlights**

ONC201 monotherapy exhibited durable and clinically meaningful efficacy in recurrent H3 K27M-mutant diffuse midline glioma (DMG) patients:

- Response Assessment in Neuro-Oncology Criteria for High Grade Gliomas (RANO-HGG) criteria assessed by dual reader blinded independent central review (BICR)
    - Overall response rate (ORR): 20% (95% CI: 10 – 34%); including one complete response
    - Median duration of response (DOR): 11.2 months (95% CI: 3.8 – not reached)
    - Median time to response: 8.3 months (range: 1.9 – 15.9)
    - Disease control rate (DCR): 40% (95% CI: 26 – 55%)
    - Progression-free survival (PFS): 35% (95% CI: 21 – 49%) at 6 months; 30% (95% CI: 17 – 44%) at 12 months
- Response Assessment in Neuro-Oncology Criteria for Low Grade Gliomas (RANO-LGG) criteria assessed by dual reader BICR
    - ORR 26% (95% CI: 15 – 40%)
- Among evaluable patients (those receiving at least 4mg of dexamethasone daily at baseline), 46.7% achieved at least a 50% confirmed reduction in corticosteroid dose
- Among evaluable patients (those with a baseline performance status (KPS/LPS) score of 80 or lower), 20.6% achieved a confirmed improvement, indicative of improved quality of life.
- Overall survival
    - 12 months: 57% (95% CI: 41 – 70%)
    - 24 months: 35% (95% CI: 21 – 49%)

"The ONC201 data to be presented at SNO show impressive and consistent results in a disease where life expectancy is exceedingly limited. There currently are no effective therapeutic options for patients with recurrent disease after radiation other than palliation. ONC201 results are particularly notable in light of extended wash-out periods required to ensure isolation of ONC201 single agent effect. It sets the stage for future study of ONC201 earlier in treatment," said Mike Sherman, Chief Executive Officer of Chimerix. "This mutation is considered high grade regardless of histology when present in diffuse midline gliomas and yet the consistency in response between RANO-HGG and -LGG criteria is important in that responses in both enhancing and non-enhancing measures of disease confer benefit to patients. Importantly, tumor responses were quite durable and associated with other measures of clinical benefit including sustained reduction in corticosteroid use, performance status improvement, and survival beyond 24 months. In fact, 11 of the 12 evaluable RANO responders (HGG or LGG) also had accompanying corticosteroid reduction or performance status improvement. In the context of an oral therapy that has demonstrated an attractive safety profile in prior reports, we are very excited about the potential of ONC201 to help children and adult patients."

"Given the very limited treatment options for patients with recurrent H3 K27M-mutant glioma, we are encouraged by the durable tumor regressions seen in some patients treated with ONC201," said Dr. Arrillaga-Romany.

This cohort was comprised of the first 50 patients enrolled across five ONC201 clinical studies who met specific criteria based on feedback from the FDA. These patients were two years of age or older, had a measurable diffuse midline glioma with 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.

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 U.S. Food and Drug Administration (FDA) granted ONC201 Fast Track Designation for the treatment of adult recurrent H3 K27M-mutant HGG, 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.

Chimerix plans to meet with the FDA 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 H3 K27M-mutant glioma is a brain cancer with a particularly poor prognosis. Pediatric patients with recurrent glioma that carries the H3 K27M mutation have an even worse prognosis. Diffuse midline gliomas with this mutation are classified as Grade IV by the World Health Organization. Grade IV gliomas represent the highest grade with the worst prognosis.

**Conference Call and Webcast**

Chimerix will host a conference call and live audio webcast to discuss these data on Monday, November 22 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 9048088. The call will reference slides that can be accessed on our website under the investor tab.

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 results of the 50-patient efficacy analysis of ONC201. 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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