Chimerix Announces Data Highlighting Clinical Efficacy and Molecular Mechanisms of Response to ONC201 Treatment of H3 K27M-Mutant Diffuse Midline Gliomas Published in "Cancer Discovery"

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Patients Treated with ONC201 Demonstrated Median Overall Survival (mOS) of 21.7 Months in the Front-Line Setting, Post Radiation, Versus 12 Months mOS Historical Control

ONC201 Treatment Disrupts Key Metabolic and Epigenetic Pathways

DURHAM, N.C., Aug. 16, 2023 (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 publication of data in support of the Company's first-in-class small molecule imipridone, ONC201, as a treatment for H3 K27M-mutant diffuse midline gliomas (H3K27M-DMG) in the peer-reviewed journal, *Cancer Discovery,* a journal of the American Association for Cancer Research.

The manuscript titled, *"Clinical efficacy of ONC201 in H3K27M-mutant diffuse midline gliomas is driven by disruption of integrated metabolic and epigenetic pathways,"* reports survival analyses of 71 patients with H3 K27M-DMG treated with ONC201 which demonstrated promising results in a patient population with a poor prognosis and few treatment options. In addition to assessing clinical outcomes, the study corroborated mechanistic findings from laboratory models in samples from treated patients that demonstrated the ability of ONC201 to disrupt metabolic pathways and reverse the epigenetic consequence (H3K27me3-loss) of the H3 K27M mutation.

"The survival analyses published today in *Cancer Discovery* reported that ONC201 frontline treatment, administered post radiation therapy, demonstrated a statistically significant increase in mOS from diagnosis versus historical controls (21.7 months mOS vs. 12 months mOS, p<0.0001). These data are particularly relevant given that enrollment in the ongoing ACTION study occurs in a similar population. Additionally, these data further elucidate the underlying novel mechanism of action for ONC201 in a patient population which has very limited treatment options," said Mike Andriole, Chief Executive Officer of Chimerix. "While these results require validation in prospectively designed studies, such as the Phase 3 ACTION study, they nevertheless provide ongoing confidence and rationale for ONC201's monotherapy treatment effect and offer hope to the patients, families and caregivers facing this challenging and life-threatening cancer."

"H3K27M-DMG represents one of the most difficult tumors to treat," said Carl Koschmann, M.D., Associate Professor of Pediatric Neuro-Oncology and Clinical Scientific Director of the Chad Carr Pediatric Brain Tumor Center at Michigan Medicine. "Prior to this study, there have been more than 250 clinical trials that have not been able to improve outcomes. These results are potentially a major crack in the armor."

The complete publication can be accessed here.

About the Phase 3 ACTION Study
The Phase 3 ACTION trial is actively recruiting patients in 11 countries across North America, Europe, the UK, Israel and Asia. The trial enrolls patients shortly after completion of front-line radiation therapy that is the standard of care. The study is designed to enroll 450 patients randomized 1:1:1 to receive ONC201 at one of two dosing frequencies or placebo. Participants are randomized to receive 625mg of ONC201 once per week (the Phase 2 dosing regimen), 625mg twice per week on two consecutive days or placebo. The dose will be scaled by body weight for patients <52.5kg. OS will be assessed for efficacy at three alpha-allocated timepoints: two interim assessments by the Independent Data Monitoring Committee (IDMC) at 164 events and 246 events, respectively, and a final assessment at 327 events. The final progression-free survival (PFS) analysis will be performed after 286 events, with progression assessed using RANO HGG criteria by blinded independent central review (BICR). Secondary endpoints include corticosteroid response, performance status response, change from baseline in quality of life (QoL) assessments and change from baseline in neurologic function as assessed by the Neurologic Assessment in Neuro-Oncology (NANO) scale. For more information, please visit clinicaltrials.gov.

About Chimerix
Chimerix is a biopharmaceutical company with a mission to develop medicines that meaningfully improve and extend the lives of patients facing deadly diseases. The Company's most advanced clinical-stage development program, ONC201, is in development for H3 K27M-mutant glioma.

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 relevance of the published survival analyses to the Phase 3 ACTION study, and the confidence and rationale in ONC201's treatment effect. Among the factors and risks that could cause actual results to differ materially from those indicated in the forward-looking statements are risks related to the timing, completion and outcome of the Phase 3 ACTION study of ONC201; risks associated with repeating positive results obtained in prior preclinical or clinical studies in future studies; 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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