Catalyst Biosciences Announces Pivotal Phase 3 Study Design for MarzAA in Individuals with Hemophilia A or B with Inhibitors

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SOUTH SAN FRANCISCO, Calif., April 06, 2020 (GLOBE NEWSWIRE) -- Catalyst Biosciences, Inc. (NASDAQ: CBIO), a biopharmaceutical company that is developing novel subcutaneous (SQ) therapies for hemophilia and other inherited bleeding disorders, today announced that it has received guidance from the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA) on a pivotal Phase 3 trial design for Marzeptacog alfa (activated) – MarzAA. The open-label trial will evaluate the efficacy of SQ MarzAA to treat episodic bleeding in individuals with hemophilia A or B with inhibitors.

"Now that we have received regulatory feedback, we have initiated preparations for a Phase 3 trial of MarzAA in individuals with hemophilia A or B with inhibitors," said Nassim Usman, Ph.D., president and chief executive officer of Catalyst. "We plan to enroll the first patient before year end, however that date is dependant on the readiness of our trial sites and improvements in the current effects of COVID-19 on clinical trial execution. At present, manufacturing and drug supply are unaffected for our clinical trials."

The Phase 3 **Crimson-1** *(Sub**c**utaneous Mar**z**eptacog Alfa (Act**i**vated) For On de**m**and Treatment and Control of Bleeding Epi**s**odes in Subjects with Hem**o**philia A or Hemophilia B with I**n**hibitors)* study will enroll individuals who experience episodic bleeding. Crimson-1 will be an open-label global trial, evaluating the safety and efficacy of SQ MarzAA in the treatment of approximately 230 bleeding episodes in approximately 75 patients, compared with their prior standard of care in a similar number of bleeding episodes.

The study will assess the effectiveness of SQ MarzAA, using up to 3 doses to treat a bleeding episode. The primary endpoint will be hemostatic efficacy using a standard 4-point assessment scale.

About Marzeptacog alfa (activated) MarzAA
Marzeptacog alfa (activated), or MarzAA, is a potent, subcutaneously (SQ) administered, next-generation recombinant Factor VIIa variant. Catalyst completed a Phase 2 open-label SQ prophylaxis trial of MarzAA which met the trial's primary and all of the secondary endpoints in 2019. The Company's preclinical and clinical data support MarzAA's potential use for treatment of episodic bleeding episodes and supports further clinical testing for on-demand treatment of bleeds in individuals with hemophilia, Glanzmann Thrombasthenia, Factor VII deficiency and other bleeding disorders.

About Catalyst Biosciences
Catalyst is a research and clinical development biopharmaceutical company focused on addressing unmet needs in rare diseases and systemic complement mediated disorders. Our protease engineering platform includes development programs in hemophilia and a research program on subcutaneous (SQ) systemic complement inhibitors. One of our key competitive advantages is that our systemically dosed product candidates, due to the improvements we have made using our protease engineering platform, can be delivered SQ, which is less invasive, faster to treat, and more convenient than intravenous (IV) drugs currently on the market. Our lead asset, MarzAA has completed Phase 2 development in prophylaxis, having met its primary endpoint of significantly reducing the annualized bleed rate (ABR) in individuals with hemophilia A or B with inhibitors. Our second hemophilia asset, SQ dalcinonacog alfa (DalcA) is being developed for the treatment of hemophilia B and has demonstrated efficacy and safety in a Phase 2b clinical trial that has completed dosing and all participant activities. We have an early stage Factor IX gene therapy construct - CB 2679d-GT - for Hemophilia B that has demonstrated superiority compared with the Padua variant in preclinical models. We also have a global license and collaboration agreement with Biogen for the development and commercialization of pegylated CB 2782 for the potential treatment of geographic atrophy-associated dry age-related macular degeneration. For more information, please visit www.catalystbiosciences.com

Forward-Looking Statements
This press release contains forward-looking statements that involve substantial risks and uncertainties. Forward-looking statements include statements about the potential uses and benefits of MarzAA and DalcA to effectively and therapeutically treat hemophilia subcutaneously, the potential market opportunity for MarzAA, plans to begin enrollment for a Phase 3 pivotal study for MarzAA by year end, and potential future milestone and royalty payments from Biogen. Actual results or events could differ materially from the plans, intentions, expectations and projections disclosed in the forward-looking statements. Various important factors could cause actual results or events to differ materially, including, but not limited to, the risk that trials and studies may be delayed as a result of the COVID-19 virus and other factors, that trials may not have satisfactory outcomes, that additional human trials will not replicate the results from earlier trials, that potential adverse effects may arise from the testing or use of DalcA or MarzAA, including the generation of antibodies, which has been observed in patients treated with DalcA, the risk that costs required to develop or manufacture the Company's products will be higher than anticipated, including as a result of delays in development and manufacturing resulting from COVID-19 and other factors, the risk that Biogen will terminate Catalyst's agreement with them, competition and other risks described in the "Risk Factors" section of the Company's annual report filed with the Securities and Exchange Commission on February 20, 2020, and in other filings with the Securities and Exchange Commission. The Company does not assume any obligation to update any forward-looking statements, except as required by law.

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