Catalyst Biosciences Presents Preclinical FIX Gene Therapy Data in an Oral Presentation at the World Federation of Hemophilia Virtual Summit 2020

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CB 2679d-GT demonstrates robust expression of FIX in multiple preclinical models

SOUTH SAN FRANCISCO, Calif., June 19, 2020 (GLOBE NEWSWIRE) -- Catalyst Biosciences, Inc. (NASDAQ: CBIO), today presented data from preclinical studies of its hemophilia B gene therapy CB 2679d-GT at the World Federation of Hemophilia Virtual Summit, taking place from June 14 -19, 2020.

The oral presentation, entitled: “Combination of a Novel Chimeric AAV Capsid and Potency Enhanced FIX Variant for Hemophilia B Gene Therapy,” given by Dr. Grant Blouse, senior vice president of translational research, provided preclinical results of CB 2679d-GT, the company’s novel FIX gene therapy. CB 2679d-GT was designed to achieve clinically relevant FIX levels at a reduced viral load by combining engineered AAV capsids with Catalyst’s novel high potency FIX transgene.

“The preclinical data from our constructs demonstrated a strong dose response and improved reduction in bleeding relative to the Padua variant,” said Nassim Usman, Ph.D., president and chief executive officer of Catalyst. “The enhanced FIX activity and reduced viral dose may offer advantages over current AAV-based gene therapies in clinical development.”

Studies of CB 2679d-GT in hemophilia B mice have demonstrated a 4-fold reduction in blood loss and an 8-fold reduction in bleeding time when compared with the same dose of the Padua variant of FIX. Furthermore, when packaged in a proprietary chimeric AAV capsid, CB 2679d-GT demonstrated a clear dose response of high stable FIX levels across the three dose levels in hemophilia B mice.

A pilot non-human primate study compared the expression and tolerability of CB 2679d-GT in the novel chimeric capsid KP1 with the LK03 capsid. The study demonstrated that CB 2679d-GT was well tolerated with high FIX expression that stabilized to approximately 25% to 50% FIX above baseline levels at the 6-week interim data cutoff. The novel chimeric capsid had differentiated and superior response to anti-capsid neutralizing antibodies than that observed for the LK03 comparator during the screening of non-human primates for the study.

A copy of the presentation slides can be accessed on the Events and Presentations section of the Catalyst website.

About Catalyst Biosciences
Catalyst is a research and clinical development biopharmaceutical company focused on addressing unmet needs in rare hematologic and systemic complement-mediated disorders. Our protease engineering platform includes development programs in hemophilia, a research program on subcutaneous (SQ) systemic complement inhibitors and a partnered preclinical development program with Biogen for dry age-related macular degeneration (AMD). One of our key competitive advantages is that the product candidates generated by our protease engineering platform have improved functionality and potency. These characteristics allow for improved dosing of our candidates including SQ systemic administration of recombinant coagulation factors and complement inhibitors, low-dose, high activity gene therapy constructs, and less frequently dosed intravitreal therapeutics. Our most advanced asset, SQ MarzAA has successfully completed Phase 2 development in prophylaxis, significantly reducing the annualized bleed rate (ABR) in individuals with Hemophilia A or B with inhibitors. Following regulatory guidance from the U.S. Food and Drug Administration and European Medicines Agency, we recently announced the design of a Phase 3 registration study that is planned for late 2020. Subcutaneous dalcinonacog alfa (DalcA) is being developed for the treatment of Hemophilia B and has demonstrated efficacy and safety in a Phase 2b clinical trial. We have a discovery stage Factor IX gene therapy construct - CB 2679d-GT - for Hemophilia B, that has demonstrated superiority compared with the Padua variant in preclinical models. Finally, we have a global license and collaboration agreement with Biogen for the development and commercialization of anti-complement Factor 3 (C3) pegylated CB 2782 for the potential treatment of geographic atrophy-associated dry AMD.

Forward-Looking Statements
This press release contains forward-looking statements that involve substantial risks and uncertainties. Forward-looking statements include statements about the superiority of CB 2679d-GT over the Padua variant, enhanced FIX activity of CB 2679d-GT, which may reduce viral dose and maintain high FIX activity levels while potentially decreasing liver toxicity, the chimeric capsid, which may have lower neutralization by pre-existing AAV antibodies, as well as plans for a Phase 3 trial of MarzAA in late 2020 and the Company’s collaboration with Biogen for the development and commercialization of pegylated CB 2782 for the potential treatment of geographic atrophy-associated dry age-related macular degeneration. 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 animal trials or earlier human trials, that potential adverse effects may arise from the testing or use of DalcA or MarzAA, including the generation of neutralizing 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, competition and other risks described in the “Risk Factors” section of the Company’s quarterly report filed with the Securities and Exchange Commission on May 11, 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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