Catalyst Biosciences Reports First Quarter 2020 Operating & Financial Results and Provides a Corporate Update

May 11, 2020

MarzAA Phase 3 study first patient enrollment targeted before year end following FDA and EMA regulatory feedback

DalcA Phase 2b study completed, final data to be presented in the second quarter

SOUTH SAN FRANCISCO, Calif., May 11, 2020 (GLOBE NEWSWIRE) -- Catalyst Biosciences, Inc. (NASDAQ: CBIO) today announced its operating and financial results for the first quarter ended March 31, 2020 and provided a corporate update.

“Despite disruptions in clinical and preclinical research operations affecting the biotechnology industry as the result of the COVID-19 pandemic, we have continued to make significant progress in both our MarzAA and DalcA programs,” said Nassim Usman, Ph.D., president and chief executive officer of Catalyst. “We have incorporated guidance from FDA and EMA in our Phase 3 trial design for MarzAA and anticipate enrolling the first patient before the end of the year. We completed our Phase 2b DalcA study and will present the final data before the end of the quarter.” Dr. Usman continued, “As we look forward, our solid financial position and progress across our portfolio give us confidence in our ability to advance our programs through several important milestones. I would like to thank our study participants, collaborators, contractors and employees for their dedication and flexibility that has allowed us to advance the development of our new medicines as we face these trying times together.”

Recent Milestones:

- **Marzeptacog alfa (activated) – MarzAA,** a subcutaneously (SQ) administered next-generation engineered coagulation Factor VIIa (FVIIa): The Company received guidance from the FDA and the EMA on a pivotal Phase 3 trial design for 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.

- **Dalcinonacog alfa – DalcA,** a subcutaneously administered next-generation engineered coagulation Factor IX (FIX): The open-label Phase 2b study was designed to evaluate the ability of DalcA to maintain steady state protective FIX levels above 12% in six individuals with severe hemophilia B. Each subject received a single intravenous dose, followed by daily SQ doses of DalcA for 28 days. Data presented at the European Association for Haemophilia and Allied Disorders (EAHAD) Congress in February showed that daily SQ dosing of DalcA achieved effective prophylaxis with FIX activity levels ranging from 14 to 28% and zero bleeds. No neutralizing antibodies were detected and the treatment was well tolerated. The half-life of SQ DalcA ranged from 70 to 112 hours, suggesting the potential for lower or less frequent dosing.

- **Factor IX gene therapy construct – CB 2679-GT,** The Company’s proprietary FIX gene therapy construct CB 2679d-GT is being developed for the treatment of hemophilia B and has demonstrated superiority compared with the Padua variant in preclinical models. The Company presented preclinical data at EAHAD in February 2020 demonstrating that a proprietary chimeric AAV capsid licensed from Stanford University expressing CB 2679d-GT FIX variant may significantly reduce the vector dose required of a gene therapy treatment while maintaining high factor activity levels.

Expected Milestones:

- **MarzAA:** Report final data in the second quarter of 2020 from a MarzAA Phase 1 pharmacokinetic and pharmacodynamic study to support future SQ treatment of bleed studies. Initiate a Phase 3 open-label trial in 2020, evaluating the efficacy of SQ MarzAA to treat episodic bleeding in individuals with hemophilia A or B with inhibitors, subject to the readiness of trial sites and improvements in the current effects of COVID-19 on clinical trial execution.

- **DalcAA**: Report final Phase 2b trial data in the second quarter of 2020.

- **CB 2679d-GT (**FIX Gene Therapy): Report initial non-human primate data in the second quarter of 2020.

First Quarter 2020 Results and Financial Highlights:

- Cash, cash equivalents and short-term investments, as of March 31, 2020 were $104.5 million.

- Research and development expenses were $13.3 million and $12.0 million during the three months ended March 31, 2020 and 2019, respectively, an increase of $1.3 million, or 10%. The increase was due primarily to an increase of $0.9 million in preclinical spending, an increase of $0.2 million in personnel costs and $0.1 million in clinical and manufacturing costs.

- General and administrative expenses were $3.7 million and $3.7 million during the three months ended March 31, 2020 and 2019, respectively.

- Interest and other income, net was $1.0 million and $0.6 million during the three months ended March 31, 2020 and 2019, respectively. The increase was primarily due to a $0.7 million final contingent payment from a prior asset sale offset by decrease in interest income of approximately $0.3 million.

- Net loss attributable to common stockholders for the three-months ended March 31, 2020 was $4.1 million, or ($0.28) per basic and diluted share, compared with $15.1 million, or ($1.26) per basic and diluted share, for the prior year period.

- As of March 31, 2020, the Company had 17,419,313 shares of common stock outstanding.

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 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 delivery 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 FDA and EMA, we recently announced the design of a Phase 3 registration study that is planned for late 2020. 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. 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 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 plans to enroll the first patient in the Phase 3 trial of MarzAA before year-end, plans to present final results from the Phase 2b clinical trial of DalcA and non-human primate data of CB 2679d-GT during the second quarter of 2020, the potential for positive results from the Phase 2b clinical trial of DalcA and for SQ DalcA to change the treatment paradigm for hemophilia B, the potential uses and benefits of MarzAA and DalcA to effectively and therapeutically treat hemophilia subcutaneously, the superiority of CB 2679d-GT over other gene therapy candidates 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 complete data from the Phase 2b trial of DalcA may not replicate previously reported partial results or 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 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 with them, 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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Catalyst Biosciences, Inc.
Condensed Consolidated Balance Sheets
(In thousands, except share and per share amounts)

	March 31, 2020 (Unaudited)	December 31, 2019
Assets		
Current assets:		
Cash and cash equivalents	$ 70,364	$ 15,369
Short-term investments	34,164	61,496
Accounts receivable	1,366	15,000
Prepaid and other current assets	2,381	4,201
Total current assets	108,275	96,066
Other assets, noncurrent	197	257

Right-of-use assets	1,795	1,927
Property and equipment, net	296	304
Total assets	$ 110,563	$ 98,554
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 1,316	$ 4,279
Accrued compensation	1,268	2,106
Deferred revenue	—	15,000
Other accrued liabilities	8,774	7,031
Operating lease liability	495	483
Total current liabilities	11,853	28,899
Operating lease liability, noncurrent	1,191	1,319
Total liabilities	13,044	30,218
Stockholders' equity:		
Preferred stock, $0.001 par value, 5,000,000 shares authorized; zero shares issued and outstanding	—	—
Common stock, $0.001 par value, 100,000,000 shares authorized; 17,419,313 and 12,040,835 shares issued and outstanding at March 31, 2020 and December 31, 2019, respectively	17	12
Additional paid-in capital	359,935	326,810
Accumulated other comprehensive income	140	34
Accumulated deficit	(262,573)	(258,520)
Total stockholders' equity	97,519	68,336
Total liabilities and stockholders' equity	$ 110,563	$ 98,554

Catalyst Biosciences, Inc.
Condensed Consolidated Statements of Operations
(In thousands, except share and per share amounts)
(Unaudited)

	Three Months Ended March 31, 2020	Three Months Ended March 31, 2019
License	$ 15,045	$ —
Collaboration	1,321	—
License and collaboration revenue	16,366	—
Operating expenses:		
Cost of license	3,047	—
Cost of collaboration	1,432	—
Research and development	13,264	12,027
General and administrative	3,691	3,687
Total operating expenses	21,434	15,714
Loss from operations	(5,068)	(15,714)
Interest and other income, net	1,015	631
Net loss	$ (4,053)	(15,083)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.28)	$ (1.26)
Shares used to compute net loss per share attributable to common stockholders, basic and diluted	14,592,451	11,963,586

Source: Catalyst Biosciences, Inc.