Krystal Biotech Reports Second Quarter 2019 Financial and Operating Results

August 5, 2019

FDA grants RMAT designation for KB103 to treat dystrophic epidermolysis bullosa (DEB)

Announced positive results from Phase 2 trial ("GEM-2") of KB103

Submitted an IND application to initiate a Phase 1/2, first in-human trial of KB105 to treat patients with TGM1-deficient autosomal recessive congenital ichthyosis (ARCI).

PITTSBURGH, Aug. 05, 2019 (GLOBE NEWSWIRE) -- Krystal Biotech Inc., ("Krystal") (NASDAQ: KRYS), a gene therapy company developing medicines to treat dermatological diseases, announced financial results for second quarter 2019, recent corporate highlights and upcoming milestones.

"We are pleased with the results from the Phase 2 trial of KB103 and delighted that the FDA has recognized our KB103 program with an RMAT designation. We are now focused on advancing KB103 into a pivotal trial that is anticipated to start in Q4 2019, following discussions with the FDA," said Krish S. Krishnan, chairman and chief executive officer of Krystal Biotech.

Mr. Krishnan continued, "Our team has done an exceptional job in getting our manufacturing facility, Ancoris, ready in a timely fashion to provide clinical materials for our pivotal study. Finally, we are thrilled to have submitted an IND for KB105 to treat ARCI and look forward to commencing a Phase 1/2 clinical trial in the second half of this year."

Recent Corporate Highlights

- In June 2019, Krystal announced positive results from Phase 2 clinical trial ("GEM-2 study") of KB103 that commenced in December 2018 at Stanford University. Safety data from all patients showed that KB103 was well tolerated with no adverse events reported. The Phase 1 portion of the trial commenced in May 2018 at Stanford University, and we announced positive interim results from this clinical study on two patients in October 2018. In the combined Phase 1 and Phase 2 study, 7 out of 8 wounds treated with KB103 closed completely (100%). The average time to 100% wound closure on all KB103 treated wounds in combined Phase 1 and Phase 2 study (7 out of 8) was 20.14 days (median 20 days). In the Phase 1 study, the duration of wound closure on two patients following 100% wound closure as of the last follow up was 184 days (6.6 months) and 174 days (6.2 months). In the Phase 2 study, preliminary results indicate that duration of wound closure at 120 day timepoint was 101 days. We anticipate commencing pivotal Phase 3 clinical trials for KB103 in Q4 2019.

- In June 2019, the U.S. Food and Drug Administration (FDA) granted Regenerative Medicine Advanced Therapy ("RMAT") designation to KB103 for the treatment of DEB. Established under the 21st Century Cures Act, RMAT designation is a program designed to expedite the development and approval of regenerative medicine products, including gene therapy products. The designation includes all the benefits of the FDA's Fast Track and Breakthrough Therapy designations and enables the ability to work more closely and frequently with the FDA to discuss surrogate or intermediate endpoints to support the potential acceleration of approval and satisfy post-approval requirements.

- In June 2019, we submitted an Investigational New Drug (IND) application with the FDA to initiate a Phase 1/2, first-in-human trial of KB105, an HSV-1 based gene therapy engineered to deliver a human transglutaminase-1 (TGM1) gene to patients with TGM1-deficient autosomal recessive congenital ichthyosis (ARCI). There are currently no treatments for this debilitating disease that affects approximately 20,000 patients worldwide. The FDA has granted KB105 orphan drug designation and rare pediatric designation for the treatment of ARCI.

- In June 2019, we announced an underwritten public offering of 2,500,000 shares of our common stock, at a public offering price of $40.00 per share. The underwriters exercised a 30-day option in July 2019 to purchase an additional 353,946 shares of our common stock at the same price per share. The gross proceeds to us from this offering were approximately $114 million, before deducting underwriting discounts and commissions and other offering expenses. The offering closed on June 27, 2019. In connection with the public offering, we suspended our "at-the-market" equity offering program that had previously been put in place in March 2019.

Upcoming Milestones

- **KB103 Phase 3 trial in the U.S.** (DEB): Expected to initiate in 2H 2019
- **KB103 Clinical trial in EU** (DEB): Expected to initiate in 1H 2020
- **KB105 Phase 1/2 trial in the U.S.** (ARCI): Expected to initiate in 2H 2019
- **KB301 IND filing in the U.S.** (Aesthetics): Expected to file in 2H 2019

- **KB104 IND filing in the U.S.** (Netherton): Expected to file in 1H 2020

Financial results for the quarter ended June 30, 2019

- Cash, cash equivalents and short-term investments totaled $195.5 million on June 30, 2019.
- Research and development expenses for the second quarter ended June 30, 2019 were $4.2 million, compared to $1.5 million for second quarter 2018.
- General and administrative expenses for the second quarter ended June 30, 2019 were $1.7 million, compared to $0.9 million for second quarter 2018.
- Net losses for the quarters ended June 30, 2019 and 2018 were $5.3 million and $2.3 million, or ($0.37) and ($0.22) per common share (basic and diluted), respectively.

For additional information on the Company's financial results for the year ended December 31, 2018, refer to form 10K filed with the SEC.

About KB103
KB103, Krystal's lead product candidate, currently in clinical development, seeks to use gene therapy to treat dystrophic epidermolysis bullosa, or DEB, an incurable skin blistering condition caused by a lack of collagen in the skin. KB103 is a replication-defective, non-integrating viral vector that has been engineered using the HSV-1 virus employing Krystal's STAR-D platform to deliver functional human COL7A1 genes directly to the patients' dividing and non-dividing skin cells. Krystal's vector can penetrate skin cells more efficiently than other viral vectors. Its high payload capacity allows it to accommodate large or multiple genes and its low immunogenicity makes it a suitable choice for direct and repeat delivery to the skin.

About Dystrophic Epidermolysis Bullosa, or DEB
Dystrophic epidermolysis bullosa, or DEB, is an incurable, often fatal skin blistering condition caused by a lack of collagen protein in the skin. It is caused by mutations in the gene coding for type VII collagen, or COL7, a major component of the anchoring fibrils, which anchor the epidermis to the underlying dermis, and provide structural adhesion in a normal individual. The lack of COL7 in DEB patients causes blisters to occur in the dermis as a result of separation from the epidermis. This makes the skin incredibly fragile, leading to blistering or skin loss at the slightest friction or knock. It is progressive and incredibly painful.

The most severe form of DEB is recessive DEB, or RDEB, which is caused by null mutations in the COL7A1 gene. DEB also occurs in the form of dominant DEB, or DDEB, which is considered to be a milder form of DEB. There are no known treatments which affect the outcome of either form of the disease, and the current standard of care for DEB patients is limited to palliative treatments. Krystal is developing KB-103 for the treatment of the broad DEB population, including both recessive and dominant forms of the disease.

About KB105
KB105, Krystal's second product candidate, currently in preclinical development, seeks to use gene therapy to treat patients with TGM1 deficient ARCI. KB105 is a replication-defective, non-integrating viral vector that has been engineered employing Krystal's STAR-D platform to deliver functional human TGM1 gene directly to the patients' dividing and non-dividing skin cells.

About Autosomal Recessive Congenital Ichthyosis
Transglutaminase 1 (TGM1) is an essential epidermal enzyme that facilitates the formation of the epidermal barrier, which prevents dehydration, and protects the skin from unwanted toxins and surface microorganisms. The loss of TGM1-activity results in the severe genetic skin disease autosomal recessive congenital ichthyosis (ARCI). Most patients with a TGM1-deficiency exhibit life-long pronounced scaling with increased transepidermal water loss (TEWL). The scales are plate-like, often of a dark color, and cover the whole body surface area. Erythroderma is either absent or minimal. Such patients usually have ectropion and, at times, eclabium, hypoplasia of joint and nasal cartilage, scarring alopecia, especially at the edge of the scalp, and palmoplantar keratoderma. Additional complications include episodes of sepsis, fluid and electrolyte imbalances due to impaired skin barrier function, and failure to thrive, especially during neonatal period and infancy. Severe heat intolerance, and nail dystrophy are also frequently observed. TGM1-deficient ARCI is associated with increased mortality in the neonatal period and has a dramatic impact on quality of life. No efficient treatment is available; current therapy only relieves some symptoms. There are approximately 23,000 cases of TGM1 deficient ARCI worldwide and about 400 new cases per year globally.

About the STAR-D Gene Therapy Platform
Krystal has developed a proprietary gene therapy platform, the Skin TARgeted Delivery platform, or STAR-D platform, that consists of an engineered HSV-1 viral vector and skin-optimized gene transfer technology, to develop off-the-shelf treatments for dermatological diseases. We believe that the STAR-D platform provides an optimal approach for treating dermatological conditions due to the nature of the vector. Certain inherent features of the HSV-1 virus, combined with the ability to strategically modify the virus in the form employed as a gene delivery backbone, provide the STAR-D platform with several advantages over other viral vector platforms for use in dermatological applications.

About Krystal Biotech
Krystal Biotech, Inc. (NASDAQ:KRYS) is a gene therapy company dedicated to developing and commercializing novel treatments for patients suffering from dermatological diseases. For more information, please visit http://www.krystalbio.com.

Forward-Looking Statements
This press release includes certain disclosures that contain "forward-looking statements," including, without limitation, statements regarding our intention to commence a pivotal study of KB103 in the second half 2019, our plans to commence a Phase 1/2 clinical trial of KB105 in the second half of 2019, the ability of KB103 to be a transformative treatment option for DEB patients and the ability of our Ancoris manufacturing facility to supply KB103 for the forthcoming clinical trial. You can identify forward-looking statements because they contain words such as "believes" and "expects." Forward-looking statements are based on Krystal's current expectations and assumptions. Because forward-looking statements relate to the future, they are subject to inherent uncertainties, risks and changes in circumstances that may differ materially from those contemplated by the forward-looking statements, which are neither statements of historical fact nor guarantees nor assurances of future performance. Important factors that could cause actual results to differ materially from those in the forward-looking statements are set forth in Krystal's filings with the Securities and Exchange Commission, including its registration statement on Form S-3, and in its Forms 10-K and 10-Q, as modified or supplemented from time to time, under the caption "Risk Factors."

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