Krystal Biotechs' KB105 Receives Rare Pediatric Disease Designation from the FDA to Treat Patients with TGM-1-deficient Autosomal Recessive Congenital Ichthyosis

August 23, 2018

KB105 is Krystal's second "off-the-shelf" topical gene therapy candidate to receive Rare Pediatric Disease Designation for treating debilitating skin diseases

PITTSBURGH, Aug. 23, 2018 (GLOBE NEWSWIRE) -- Krystal Biotech, Inc. (Nasdaq: KRYS), a gene therapy company dedicated to developing and commercializing novel treatments for patients suffering from dermatological diseases, today announced that the U.S. Food and Drug Administration (FDA) has granted a Rare Pediatric Disease Designation ("RPDD") to the company's gene therapy candidate KB105, for the treatment of patients with TGM-1-deficient autosomal recessive congenital ichthyosis ("ARCI").

ARCI is associated with neonatal morbidity (including life threatening dehydration, respiratory problems, and systemic infection) and neonatal mortality as well as the following manifestations that primarily affect older infants and children: hypohidrosis, ectropion, alopecia, digital contractures, failure to thrive and short stature.

The FDA grants RPDD for serious or life-threatening diseases with manifestations in individuals aged from birth to 18 years, including access to the FDA's expedited review and approval process. The RPDD makes the KB105 program eligible for a Rare Pediatric Disease Priority Review Voucher upon approval of KB105 by the FDA.

"We are motivated by compassion and urgency for the patients, families and caregivers affected by ARCI and other serious, life-threatening conditions with high unmet needs," said Suma M. Krishnan, founder and chief operating officer of Krystal Biotech. "This designation, combined with our receipt of the FDA's Orphan Drug Designation for KB105 earlier this month, will help support our efforts to speed up the drug development efforts in this indication and, when successful, enable this new therapeutic candidate to be made available to patients as soon as possible."

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.

About KB105
KB105 is Krystal's second product candidate, currently in preclinical development, and seeks to use gene therapy to treat patients with TGM-1 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 TGM-1 gene directly to the patients' dividing and non-dividing skin cells. HSV-1 is Krystal's replication-deficient, non-integrating viral vector that 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 Autosomal Recessive Congenital Ichthyosis
Transglutaminase 1 (TGM-1) 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 TGM-1-activity results in the severe genetic skin disease autosomal recessive congenital ichthyosis (ARCI). Most patients with a TGM-1-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. TGM-1-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.

About Rare Pediatric Disease Designation
The FDA grants Rare Pediatric Disease Designations for serious or life-threatening diseases with manifestations in individuals aged from birth to 18 years, and that affect fewer than 200,000 people in the U.S. Under the FDA's Rare Pediatric Disease Priority Review Voucher program, a sponsor who receives an approval of a new drug application or biologics license application for a product for the prevention or treatment of a rare pediatric disease may be eligible for a voucher, which can be redeemed to obtain priority review for any subsequent marketing application, and may be sold or transferred.

About Orphan Drug Designation
The FDA's Office of Orphan Drug Products grants Orphan Drug Designation to support the development of medicines for underserved patient populations, or rare disorders, that affect fewer than 200,000 people in the United States. Orphan Drug Designation may allow a company to be eligible for a seven-year period of U.S. marketing exclusivity upon approval, tax credits for certain clinical research costs, and a waiver of the Prescription Drug User Fee Act (PDUFA) filing fees, subject to certain conditions.

Forward-Looking Statements
This press release includes certain disclosures which contain "forward-looking statements," including, without limitation, statements regarding the anticipated timing for Krystal's filing of an IND, prospects for the development and potential efficacy of KB105, and the benefits of receiving the "rare pediatric disease" indication. 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 or 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 annual report, on Form 10-K under the caption “Risk Factors.”

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