Krystal Biotech's KB105 Granted Orphan Drug Designation by the FDA to Treat Patients With TGM-1 Deficient Autosomal Recessive Congenital Ichthyosis

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PITTSBURGH, Aug. 09, 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 & Drug Administration (FDA) has granted Orphan Drug Designation to the Company's second product candidate, KB105, currently in preclinical development for treatment of patients with transglutaminase 1 (TGM-1) deficient autosomal recessive congenital ichthyosis ("ARCI"). There are currently no treatments for this disease that affects approximately 20,000 patients worldwide.

"We are pleased to receive Orphan Drug Designation for KB105 to treat ARCI as this is an important step forward in our efforts to bring hope to ARCI patients and their families," said Suma M. Krishnan, founder and chief operating officer of Krystal Biotech. "We are excited by the results of the pharmacology data in animal models to date for ARCI that we submitted to the Office of Orphan Product Development. We are on track to file an IND for KB105 in the fourth quarter of 2018."

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 Krystal Biotech to be eligible for a seven-year period of U.S. marketing exclusivity upon approval of KB105, tax credits for certain clinical research costs, and a waiver of the Prescription Drug User Fee Act (PDUFA) filing fees, subject to certain conditions.

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 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 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 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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