Krystal Biotech Receives Positive EMA Opinion on Orphan Designation for KB105 to Treat Patients With TGM1 Deficient Autosomal Recessive Congenital Ichthyosis

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PITTSBURGH, Oct. 10, 2019 (GLOBE NEWSWIRE) -- Krystal Biotech Inc., ("Krystal") (NASDAQ: KRYS), a gene therapy company developing medicines to treat rare skin diseases, today announced that the European Medicines Agency (EMA) Committee for Orphan Medicinal Products (COMP) issued a positive opinion on Krystal's application for orphan designation of KB105, for the treatment of transglutaminase-1 (TGM1) deficient autosomal recessive congenital ichthyosis (ARCI). KB105 was previously granted orphan drug designation by the United States Food and Drug Administration in August 2018.

"We are delighted with the EMA COMP's adoption of a positive opinion for KB105 orphan drug designation, which represents another important milestone for this clinical program," said Suma Krishnan, chief operating officer of Krystal Biotech. "We believe KB105 has the potential to be the first gene therapy to bring hope to ARCI patients and their families and we look forward to announcing interim clinical results on two adult patients, currently enrolled in our Phase 1/2 clinical trial in 1H 2020."

The positive opinion issued by COMP will be sent to the European Commission which is expected to grant the orphan designation within 30 days. Orphan designation in the EU allows Krystal Biotech to benefit from a number of key incentives, including reduced regulatory fees, protocol assistance, and market exclusivity, to develop a medicine for the treatment of a rare disease affecting not more than five in 10,000 people in the European Union.

TGM1-deficient ARCI is a debilitating rare skin disease characterized by excessive, thick scaling of the skin, causing multiple chronic health conditions. There are approximately 23,000 cases of TGM1-deficient ARCI worldwide and about 400 new cases per year globally. Krystal's approach is to use a non-replicating, non-integrating engineered HSV-1 vector to deliver the TGM1 gene to keratinocyte skin cells, potentially allowing them to produce the TGM1 protein that is lacking in this patient population. KB105 is designed to be an off-the-shelf treatment for TGM1-deficient ARCI that can be applied topically, directly to a patient's skin.

About KB105

KB105 is Krystal's second product candidate, currently in preclinical development, and 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. 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 (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's **S**kin **TAR**geted **D**elivery platform, or STAR-D platform, is a proprietary gene therapy platform consisting of an engineered viral vector and skin-optimized gene transfer technology that Krystal is employing to develop off-the-shelf treatments for dermatological diseases for which there are no known effective treatments. The company believes that the STAR-D platform provides an optimal approach for treating dermatological conditions due to the nature of the HSV-1 viral vector it has created. 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 topical and intradermal "off-the-shelf" novel treatments for patients suffering from rare 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 the potential of KB103 to treat the underlying causes of DEB, the timetable for bringing GMP manufacturing in-house and the potential for rapid development of the company's clinical programs. 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 registration statement on Form S-1 and Form 10-K, as amended from time to time, under the caption "Risk Factors."

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