Krystal Biotech and GeneDx Announce Collaboration to Provide No-charge Genetic Testing for Patients with Suspected Dystrophic Epidermolysis Bullosa (DEB)

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The Krystal Decode DEB™ program is designed to shorten time to accurate diagnosis and facilitate the delivery of optimal care

PITTSBURGH and GAITHERSBURG, Md., Oct. 12, 2021 (GLOBE NEWSWIRE) -- Krystal Biotech, Inc., ("Krystal") (NASDAQ: KRYS), the leader in redosable gene therapies for rare diseases, and GeneDx, Inc., a leader in genomic analysis and a wholly-owned subsidiary of BioReference Laboratories, Inc., an OPKO Health company (NASDAQ:OPK), today announced a collaboration offering no-charge genetic testing for all types of Epidermolysis Bullosa (EB). The goal of the program, called Krystal Decode DEB, is to help patients with the dystrophic form of this genetic condition, also known as DEB, get a definitive diagnosis sooner, with highly accurate results obtained with a blood or cheek swab sample.

"In the US, EB is too often diagnosed by clinical features alone without molecular confirmation. Since clinical features in different EB subtypes and other blistering disorders can show many similarities, a diagnosis based solely on clinical features can be inaccurate or delayed, which ultimately hurts the patients by preventing them from receiving proper care. The Krystal Decode DEB program will help accelerate and/or confirm an accurate diagnosis of DEB and empower health care providers, patients and families to make informed health decisions to facilitate optimal care delivery," said Dr. Peter Marinkovich, M.D., Blistering Disease Clinic Director and Associate Professor of Dermatology at Stanford University, and Primary Investigator for Krystal's ongoing Phase 3 trial.

EB is a rare genetic connective tissue disorder that causes extremely fragile skin that blisters and tears from minor friction or trauma. There are 4 types of EB, each caused by mutations in different genes. Accurately diagnosing dystrophic EB (caused by mutations in the *COL7A1* gene) is critical due to the higher risk of squamous cell cancer, internal complications and mortality.

"Epidermolysis Bullosa is a group of disorders with varied presentations, prognoses and long-term sequelae resulting from distinct gene mutations. However, many suspected EB patients do not receive diagnostic testing due to lack of insurance coverage, cost of assessment and unfamiliarity with the impact of genetic information on health care delivery," said Dr. Jeanett Segal M.D., United States Medical Director of Krystal Biotech, Inc. "Fortunately, genetic tests are available and we are thrilled to work with GeneDx to offer this at no-charge to patients with EB symptoms."

The Krystal Decode DEB program utilizes a comprehensive panel of genes to identify DEB or other genetic conditions with similar phenotype to DEB, including other EB types and some other non EB skin blistering conditions, to aid in diagnosis.

"Diagnosis of rare diseases like DEB can take years. Access to genetic testing as early as possible is essential to ensure people suspected of having DEB receive an accurate diagnosis and optimal treatment as soon as possible," said Dr. Paul Kruszka, M.D., F.A.C.M.G., Chief Medical Officer of GeneDx. "We're pleased to be working with Krystal Biotech to make it easier for patients suspected of having DEB to receive genetic testing."

The Krystal Decode DEB program is open to all US residents, including residents of Puerto Rico, who have clinical symptoms consistent with EB and have not previously received genetic testing. More information on the Decode DEB program can be obtained by emailing krystal@genedx.com or calling 1-888-729-1206. Krystal reserves the right to amend, suspend or terminate this program without notice.

About Dystrophic EB

DEB is a rare and severe monogenic disease that affects the skin and mucosal tissues. It is caused by one or more mutations in a gene called *COL7A1*, which is responsible for the formation of the protein type VII collagen protein (COL7) that forms anchoring fibrils that bind the dermis (inner layer of the skin) to the epidermis (outer layer of the skin). The lack of functional anchoring fibrils leads to extremely fragile skin that blisters and tears from minor friction or trauma. DEB patients suffer from open wounds, which leads to skin infections, fibrosis which can cause fusion of fingers and toes, and ultimately a significantly increased risk of developing squamous cell carcinoma of the skin, which is often fatal.

About Krystal Biotech

Krystal Biotech, Inc. (NASDAQ:KRYS) is a pivotal-stage gene therapy company leveraging its novel, redosable gene therapy platform and in-house manufacturing capabilities to develop therapies to treat serious rare diseases. For more information, please visit http://www.krystalbio.com.

About GeneDx

GeneDx, Inc. is a global leader in genomics, providing testing to patients and their families worldwide. Originally founded by scientists from the National Institutes of Health, GeneDx offers a world-renowned clinical genomics program with particular expertise in rare and ultra-rare genetic disorders. In addition to its market-leading exome sequencing service, GeneDx offers a suite of additional genetic testing services, including diagnostic testing for hereditary cancers, cardiac, mitochondrial, neurological disorders, prenatal diagnostics, and targeted variant testing. GeneDx is a subsidiary of BioReference Laboratories, Inc., a wholly-owned subsidiary of OPKO Health, Inc. To learn more, please visit http://www.genedx.com.

About OPKO Health

OPKO is a multinational biopharmaceutical and diagnostics company that seeks to establish industry-leading positions in large, rapidly growing markets by leveraging its discovery, development, and commercialization expertise and novel and proprietary technologies. For more information, visit www.opko.com.

Forward-Looking Statements

This press release contains forward-looking statements, including with respect to the Krystal Decode DEB program, such as its ability to help accelerate and/or confirm an accurate diagnosis of DEB and empower health care providers, patients and families to make informed health decisions to facilitate optimal care delivery. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors as are set forth under the caption "Risk Factors" in Krystal's annual and quarterly reports on file with the U.S. Securities and

Exchange Commission. Krystal undertakes no duty or obligation to update any forward-looking statements contained herein as a result of new information, future events or changes in its expectations or circumstances.

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