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https://www.krystalbio.com/focus/our-focus/
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Krystal

Our goal is to make a meaningful difference in the lives of underserved patient p
skin diseases. We work to accomplish this goal through scientific innovation and
"nature operates in the shortest way possible" (Aristotle).

Krystal Biotech, Inc. is a clinical-stage gene therapy company using its proprietary
platform to develop effective and innovative treatments for skin diseases. While
intradermal "off-the-shelf" novel therapies for rare and orphan dermatological in
our pioneering STAR-D technology to target and treat other skin conditions as we

Our Approach

Platform

Viral gene therapy platform for dermatology

We are leveraging the advantageous properties of type 1 herpes simplex virus (H
affinity for skin cells, to develop a safe viral gene therapy platform adapted for de
based system is suited for a topical route of administration, allowing for non-inva
treating the skin.

Programs

Initial focus on orphan diseases

Our initial products are directed to the treatment of monogenic and congenital s
epidermolysis bullosa (DEB) and autosomal recessive congenital ichthyosis (ARCI

DEB is an incurable, often fatal skin blistering condition caused by mutations in t
COL7, a protein that provides critical structural adhesion between skin layers in a
geperpavec ("B-VEC", previously "KB103"), a replication-defective, non-integrating
viral gene therapy platform, is Krystal's lead product candidate currently being ev
a planned phase III clinical trial. B-VEC has been engineered to deliver wild-type h
thus, functional COL7 protein) directly to a DEB patient's dividing and non-dividin
correction of the underlying genetic deficiency to stabilize the patient's otherwise

ARCI is a life-long, severe genetic skin disease that often results in marked scaling

epidermal barrier, leading to pronounced dehydration, transepidermal exposure microorganisms, and a greatly increased risk of infection. While a number of gen with the development of ARCI, the most common cause of ARCI is an inactivating transglutaminase-1, a protein that is essential for the proper formation of the ski product candidate that is being tested in an ongoing phase I/II clinical trial. This p type human TGM1 genes into the skin of ARCI patients, allowing for the treatmer this patient population.

Expansion

Expanding beyond rare and orphan diseases

In addition to our work developing novel treatments for rare, orphan skin diseas expertise and viral gene therapy platform to explore new therapeutic approache indications, including non-monogenic diseases and diseases not caused by inher

Manufacturing

Bringing all stages of the manufacturing process in-house affords us a number o ensuring robust virus production and the highest quality of purified drug produc virus batches; being able to rapidly institute process improvements identified by Chemistry, Manufacturing, and Controls (CMC) teams; optimizing our internal pro requirements; and avoiding the high demand for gene therapy services from thir Organizations (CMOs), safeguarding against delays.

Construction of Ancoris, a new state-of-the-art Good Manufacturing Practice (GM headquarters in Pittsburgh, is complete. The 4,500 square foot facility has been c manufacturing requirements for commercial development of B-VEC and the high commercial production for biopharmaceutical use. The Ancoris facility will be the projected commercial demand for B-VEC.

A ground breaking ceremony for our second commercial gene therapy facility, na 2020 in Findley-Township, Pennsylvania. The Findley-based facility is being desig Manufacturing Practice (cGMP) manufacturing facility that, beyond expanding Kr allow the in-house incorporation of raw material preparation, excipient manufac distribution, fully-integrating all components of the supply chain from starting m ASTRA facility will initially be used as a commercial back up facility for B-VEC, whi of dystrophic epidermolysis bullosa, a rare and devastating skin disorder, and ex commercial material for our pipeline products.

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