

### Gene therapy company focused on Rare Diseases

Krystal Biotech, Inc. is a clinical-stage gene therapy company dedicated to developing and commercializing novel “ready-for-use” treatments for patients suffering from rare diseases. Krystal’s patented technology includes a fully-integrated gene therapy platform consisting of an engineered HSV-1 vector and optimized gene transfer technology for repeated localized delivery to develop treatments for diseases for which there are no currently effective therapies. Krystal is also expanding the use of its pioneering technology beyond severe monogenic diseases to target and treat other skin conditions.

### Investment Highlights

- Pipeline of first-in-class gene therapy candidates for skin and lung conditions, including:
  - Beremagene geperpavec (B-VEC, previously KB103), soon to initiate pivotal trial for dystrophic epidermolysis bullosa (DEB)
  - KB105 entering phase II for treatment of TGM1-deficient ARCI
  - KB407 with recently announced preclinical data in cystic fibrosis
- Established a patented, fully-integrated HSV-1-based gene therapy platform and a pipeline of clinical and non-clinical effectors.
  - Zero royalty burden
- Use of platform has several distinct advantages over other viral gene therapy vectors:
  - HSV-1 has a significant payload capacity allowing for delivery of multiple genes and/or large genes
  - HSV-1 efficiently transduces dividing and non-dividing cells, increasing efficacy of therapeutic gene transfer
  - Focused and localized delivery of protein(s) to area of interest/intent-to-treat site
  - Platform-based products are non-replicating and are diluted by cell divisions, allowing for transient transgene expression
  - HSV-1 is an episomal virus that does not insert itself into, or otherwise disrupt, the human genome. This allows for a ready-to-use chronic application of treatment unlike other customized gene therapy products
  - Clinically proven ability to be safely re-administered, even in the presence of pre-existing anti-HSV antibodies
- Construction of “Ancoris”, the first in-house Good Manufacturing Practice (GMP) facility in Pittsburgh PA is complete. Broke ground on a second GMP facility, ASTRA, in January 2020.
  - In-house manufacturing allows for robust and flexible drug production, control over productions schedules
  - Manufacturing process is scalable from clinical phase to commercial, and accommodates ever-expanding pipeline with minimal redevelopment efforts between product candidates
- Strong IP portfolio protecting platform and products

### Fast Facts

<b>Ticker (Exchange)</b>	KRYS (NASDAQ)
<b>Market Cap</b>	~\$692 MM
<b>Outstanding Shares</b>	17.3 MM
<b>52-Week Range</b>	\$22.02 – \$66.85
<b>Avg. Daily Volume (90-Day Window)</b>	~158,000
<b>Headquarters</b>	Pittsburgh, PA
<b>Fiscal Year End</b>	December 31
<b>Cash, Cash Equivalents, and Short-Term Investments Position</b>	\$186.7 MM
<b>Insider ownership (management, employees, directors)</b>	32% of fully diluted shares outstanding

All figures as of 03/31/2020

### Contacts

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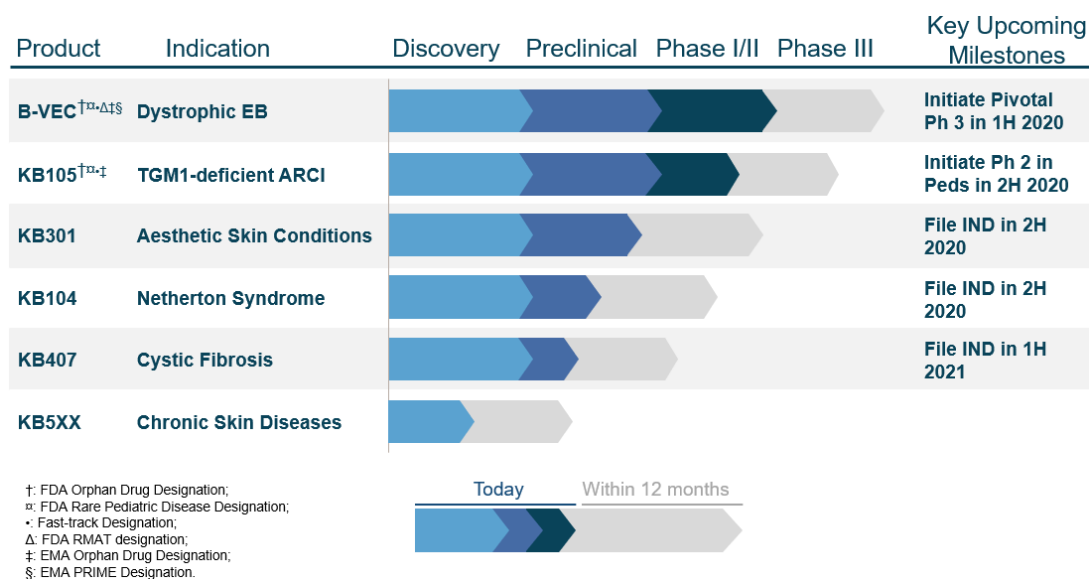
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### Current Pipeline Opportunities & Development Status



### Lead Therapeutic Candidate: B-VEC

B-VEC is a replication-defective, non-integrating viral vector engineered to deliver functional human *COL7A1* genes directly to DEB patients' dividing and non-dividing skin cells. The pivotal Phase III clinical trial of B-VEC is expected to initiate in 1H2020.

- Data from final update of combined phase I/II study in adult and pediatric patients treated with B-VEC showed:
  - > 9 out of 10 wounds treated with B-VEC closed completely (100%) following initial administrations of B-VEC
  - > The average time to 100% wound closure on the 9 B-VEC treated wounds was 17.4 days (median 14 days)
  - > The average duration of wound closure on the 9 B-VEC treated wounds at last measured timepoint was 113 days (median 110 days)
  - > The wound that did not close was re-administered B-VEC and closed completely within 7 days following re-administration
    - > The wound was originally reported to be open for over 4 years
    - > The wound remained closed for over 100 days (and ongoing)
  - > Safety data showed that B-VEC was well tolerated following initial and repeated dosing. No treatment-related adverse events (serious or otherwise) were reported. No immune response or blistering was observed around the sites of administration following first and repeat doses. Blood and urine samples collected throughout the study revealed no viral shedding, no adverse events associated with routine labs, and no antibodies to COL7 were detected

### Epidermolysis Bullosa Market Opportunity

- There are no approved treatments for DEB
- Current treatment for DEB is limited to palliative care estimated to cost between \$200k – 400k annually

### Recent Developments

#### May 18, 2020

Pricing of \$125M public offering of common stock.

#### May 14, 2020

Positive interim results from Phase I/II clinical trial of KB105 in patients with TGM1-related Autosomal Recessive Congenital Ichthyosis (ARCI). KB105 was well tolerated, able to be re-dosed safely, and mechanistically able to localize TGM1 correctly in the epidermis with functional activity.

#### May 12, 2020

Krystal presents positive proof-of-concept data at the 23rd annual meeting of the American Society of Gene & Cell Therapy on KB407, an HSV-based gene therapy vector encoding full-length human CFTR, for its cystic fibrosis (CF) program. *In vitro* pharmacology of KB407 indicated that the vector possessed a robust safety and efficacy profile in multiple CF models, including restoration of CFTR function in clinically relevant 3D patient-derived intestinal organoid cultures. This data represents critical early work supporting the application of KB407 as a novel, broadly applicable gene therapy for the treatment of CF.

#### January 24, 2020

Breaking ground on a second commercial gene therapy facility named ASTRA in Findlay Township, Pennsylvania. This will be a ~100,000 ft<sup>2</sup> state-of-the-art current Good Manufacturing Practice (cGMP) facility.