Krystal Biotech

Investor Factsheet | Q3 2020

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), which initiated pivotal trial for dystrophic epidermolysis bullosa (DEB) on July 28th, 2020
  - KB105 entered 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

Ticker (Exchange)	KRYS (NASDAQ)
Market Cap	~$812 MM
Outstanding Shares	~19.7 MM
52-Week Range	$31.89 – $66.85
Avg. Daily Volume (90-Day Window)	~202,000
Headquarters	Pittsburgh, PA
Fiscal Year End	December 31
Cash, Cash Equivalents, and Short-Term Investments Position	$297.2 MM
Insider ownership (management, employees, directors)	27% of fully diluted shares outstanding

All figures as of 06/30/2020

Contacts

Krystal Biotech, Inc.
2100 Wharton St. #701
Pittsburgh, PA 15203
412-586-5830
inquiries@krystalbio.com

Investors, News & Media
Ashley Robinson
LifeSci Advisors, LLC
arr@lifesciadvisors.com

Clinical Trials
patientadvocacy@krystalbio.com

Expanded Access
expandedaccess@krystalbio.com

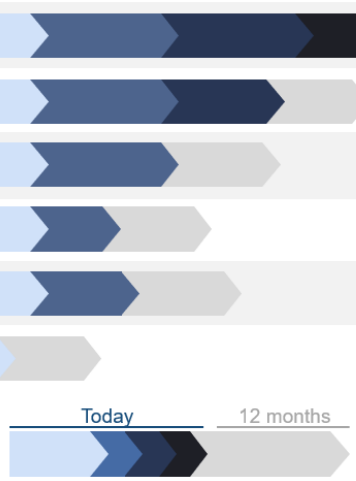
Partnering
inquiries@krystalbio.com

Krystal Biotech

Investor Factsheet | Q3 2020

Current Pipeline Opportunities & Development Status

Product	Indication	Key Upcoming Milestones
B-VEC†¤•Δ‡§	Dystrophic EB	Topline Pivotal Data in 2021
KB105†¤•‡	TGM1-deficient ARCI	Initiated Ph 2 in 2H 2020
KB301	Aesthetic Skin Conditions	Initiate clinical trial in 2H 2020
KB104	Netherton Syndrome	File IND in 2021
KB407	Cystic Fibrosis	File IND in 2021
KB5XX	Chronic Skin Diseases	



†: FDA Orphan Drug Designation;
¤: FDA Rare Pediatric Disease Designation;
•: Fast-track Designation;
Δ: FDA RMAT designation;
‡: EMA Orphan Drug Designation;
§: EMA PRIME Designation.

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 initiated on July 28th, 2020.

- 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

July 28, 2020
Initiation of GEM-3 study, a multi-center, placebo-controlled, double-blinded, Phase III clinical study of B-VEC for the treatment of dystrophic epidermolysis bullosa patients.

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 ~150,000 ft^2 state-of-the-art current Good Manufacturing Practice (cGMP) facility.