Krystal Biotech

Investor Factsheet | Q3 2019

Medicines for skin diseases and conditions – a gene therapy company

Krystal Biotech, Inc. is a clinical-stage gene therapy company dedicated to developing and commercializing topical and intradermal "off-the-shelf" novel treatments for patients suffering from rare dermatological diseases. Krystal's platform is a proprietary, fully-integrated gene therapy platform consisting of an engineered viral vector and skin-optimized gene transfer technology to develop off-the-shelf treatments for monogenic dermatological diseases with no current effective treatments. 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 a variety of skin conditions
- Established a proprietary, fully-integrated HSV-1-based gene therapy platform and a pipeline of clinical and non-clinical effectors to target skin diseases and conditions.
  - > Zero royalty burden
- Use of platform has a number of distinct advantages over other viral gene therapy vectors:
  - > Products may be applied topically in a gel-formulation or injected intradermally directly to the affected skin
  - > HSV-1 has a significant payload capacity allowing for delivery of multiple genes and/or large genes
  - > HSV-1 transduces dividing and non-dividing cells, increasing efficiency of therapeutic gene transfer
  - > HSV-1 is an episomal virus that does not insert itself into, or otherwise disrupt, the human genome. This allows for an "off-the-shelf" chronic application of treatment unlike other customized gene therapy products.
  - > HSV-1 stability significantly minimizes supply chain and logistics issue when compared to other gene therapy treatments
  - > Platform based products are non-replicating and are diluted by cell divisions, allowing for transient transgene expression
  - > HSV-1 has natural tropism for skin cells leading to high transduction efficiency
  - > Engineering of vector potentially allows for safe repeated administration (re-dosing) of products
  - > Strong patent and IP portfolio protecting STAR-D-based products and processes
- KB103, the most advanced product candidate, is in development for dystrophic epidermolysis bullosa (DEB), an incurable skin blistering condition caused by a lack of collagen 7 in the skin
  - > Clinical proof-of-concept for STAR-D platform and for KB103 demonstrated by completed GEM-Phase I/II clinical trial in adult and pediatric patients with DEB
  - > Top-line data from KB103 GEM-Phase II clinical trial announced 6/24/19
- Construction of "Ancoris", the first in-house Good Manufacturing Practice (GMP) facility in Pittsburgh PA is complete. Plans to build a second GMP facility in 2H 2019 in motion.
- Insider ownership (management, employees, directors): Approximately 30% of fully diluted shares outstanding (as of 6/30/19)

Fast Facts

Ticker (Exchange)	KRYS (NASDAQ)
Stock Price	$30.27
Market Cap	~$683 MM
Outstanding Shares	16.95 MM
52-Week Range	$14.28 – $46.90
Avg. Daily Volume (90-Day Window)	~183,000
Headquarters	Pittsburgh, PA
Fiscal Year End	December 31
Cash, Cash Equivalents, and Short-Term Investments Position	$195.5 MM
Insider ownership (management, employees, directors)	~30% of fully diluted shares outstanding

All figures as of 6/30/2019

Management Team

Suma Krishnan
Founder and COO

Krish Krishnan
Chairman and CEO

Tony Riley
CFO

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Current Pipeline Opportunities & Development Status

Product	Indication	Discovery	Preclinical	Phase I/II	Phase III	Marketed
KB103†¤·Δ‡§	Dystrophic EB					
KB105†¤	TGM1-deficient ARCI			IND filed June 13th, 2019		
KB301 / KB302	Aesthetic Skin Conditions			IND to be filed 2H 2019		
KB104	Netherton Syndrome			IND to be filed 1H 2020		
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: KB103

KB103 is a replication-defective, non-integrating viral vector that has been engineered employing Krystal's fully-integrated gene therapy platform to deliver functional human COL7A1 genes directly to DEB patients' dividing and non-dividing skin cells. KB103 is currently being tested in an ongoing Phase II clinical trial.

- Data from combined phase I/II study in adult and pediatric patients treated with KB103 showed:
  - > 7 out of 8 wounds treated with KB103 closed completely (100%)
  - > The average time to 100% wound closure on all KB103 treated wounds was 20.14 days (median 20 days)
  - > In phase I study, the duration of wound closure on two patients following 100% wound closure as of the last follow up was 184 days (6.6 months) and 174 days (6.2 months)
  - > In phase II study, preliminary results indicate that duration of wound closure at 120-day timepoint was 101 days
  - > Safety data from patients in phase I and phase II study show that KB103 was well tolerated, even after repeat administration. No serious adverse events, and no product-related adverse events, were reported. No immune response or blistering was observed around the sites of administration following first and repeat dose. Blood and urine samples collected throughout the study revealed: no viral shedding; no adverse events associated with routine labs (chemistry and hematology); and no antibodies to COL7 were detected
- Commencement of pivotal phase III clinical trials for KB103 anticipated in Q4 2019

Epidermolysis Bullosa Market Opportunity

- DEB affects ~7 people per 1 million worldwide, 52,000+ cases total (Kho et al. Arch Dermatol. 2010 146(6):635-40; Orphanet Report Series Rare Diseases Collection 2018)
- 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 1, 2019

Krystal has been added to the Russell 2000® index, effective after the stock market closed on June 28, 2019 following Russell's annual reconstitution of its comprehensive set of U.S. and global equity indexes. The Russell 2000® index measures the performance of the small-cap segment of the U.S. equity market, and is a subset of the Russell 3000® index, representing approximately 10% of the total market capitalization of that index.

June 24, 2019

Krystal announces positive results from phase II placebo-controlled clinical trial of KB103 (GEM-2) study and an update from the phase I (Gem-1) study.

June 24, 2019

KB103 receives Regenerative Medicine Advanced Therapy (RMAT) designation from the U.S. Food and Drug Administration (FDA) based on the positive interim data from the GEM-1 and GEM-2 studies.

June 24, 2019

Krystal announces that it has priced the previously announced underwritten public offering of 2,500,000 shares of its common stock at a public offering price of $40.00 per share.

June 14, 2019

Krystal announces that it has submitted an Investigational New Drug (IND) application with the U.S. Food and Drug Administration (FDA) to initiate a phase I/II, first in human trial of KB105, an HSV-1-based gene therapy engineered to deliver a human transglutaminase-1 (TGM1) gene to patients with TGM1-deficient autosomal recessive congenital ichthyosis (ARCI).

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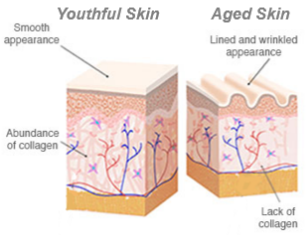
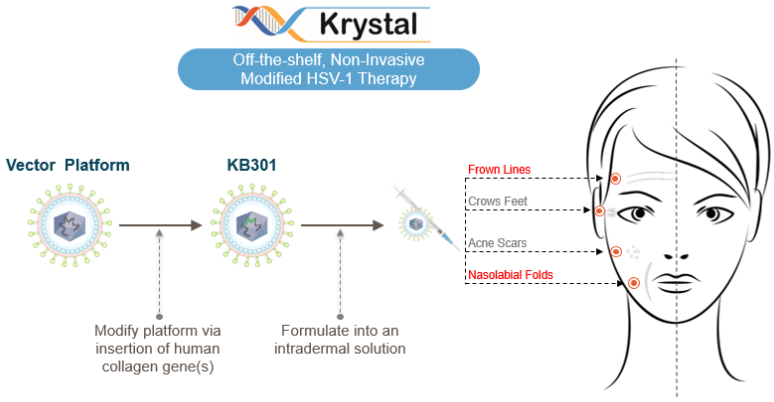
Future Opportunities

Application of fully-integrated vector platform to treat aesthetic defects

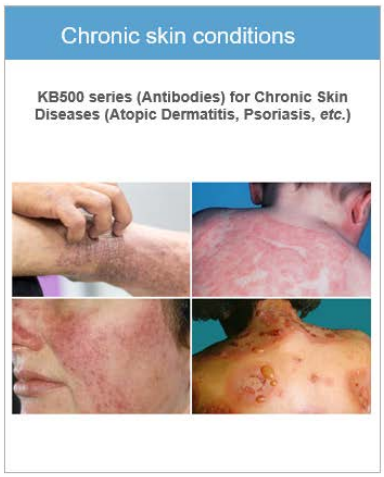


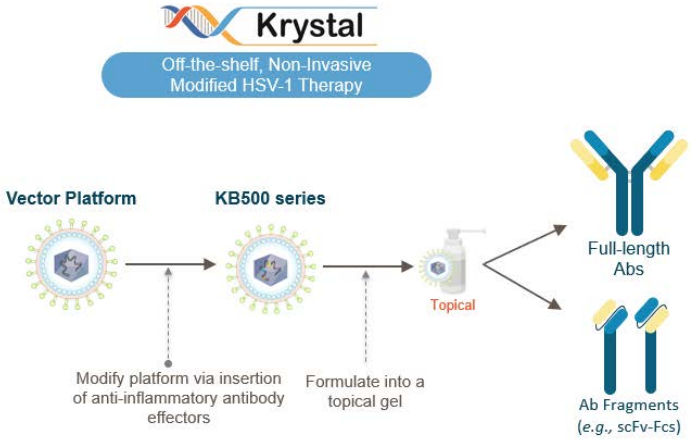
The characteristic features of skin aging are largely due to aberrant collagen homeostasis, resulting in a net collagen deficiency

Fromowitz, J. "Update on Aging Skin"; Florida Society of Dermatology



Application of fully-integrated vector platform to treat complex, chronic skin conditions





Upcoming Milestones

- Commence pivotal phase III trial for KB103 (DEB) in Q4 2019
- Commence pivotal phase I/II trial for KB105 (ARCI) in 2H 2019
- File IND for KB301 (aesthetics) in 2H 2019
- Filed IND for KB104 (Netherton Syndrome) in 1H 2020
- Commence clinical trial for KB103 in EU in 1H 2020
- Break ground on second GMP manufacturing facility in 1H 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