Krystal Biotech

Investor Factsheet | Q2 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 with recent interim data in ongoing GEM-Phase II clinical trial in adult and pediatric patients with DEB
  - > Top-line data from KB103 GEM-Phase II clinical trial in H1 2019
- 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): 34% of fully diluted shares outstanding (as of 3/31/19)

Fast Facts

Ticker (Exchange)	KRYS (NASDAQ)
Stock Price	$32.90
Market Cap	$475 MM
Outstanding Shares	14.43 MM
52-Week Range	$8.91 – $33.12
Avg. Daily Volume (90-Day Window)	~66,00
Headquarters	Pittsburgh, PA
Fiscal Year End	December 31
Cash, Cash Equivalents, and Short-Term Investments Position	$106.6 MM
Insider ownership (management, employees, directors)	34% of fully diluted shares outstanding

All figures as of 3/31/2019

Management Team

Suma Krishnan
Founder and COO

Krish Krishnan
Chairman and CEO

Tony Riley
CFO

Krystal Biotech

Investor Factsheet | Q2 2019

Current Pipeline Opportunities & Development Status

Product	Indication	Discovery	Preclinical	Phase I/II	Phase III	Marketed
KB103	Dystrophic EB					
KB104	Netherton Syndrome					
KB105	TGM1-deficient ARCI					
KB107	Junctional EB					
KB301/KB302	Aesthetic Skin Conditions				Two Undisclosed Targets	
KB5xx	Chronic Skin Diseases				Two Undisclosed Targets	

FDA Orphan Drug Designation;
EMA Orphan Drug Designation;
Fast-track Designation;
FDA Rare Pediatric Disease 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.

- Interim data from two adult patients treated with KB103 showed:
  - > Clearly detectable robust functional COL7 expression by immunofluorescence in biopsy samples as early as Day 2 of treatment
  - > Functional anchoring fibril formation as early as day 14 of treatment and persisting up to the last biopsy in both patients
  - > Topically administered KB103 wounds closed in 2 weeks (as opposed to 10 weeks or not-at-all for control treated wounds) with durable wound-closure lasting greater than 3.5 months in both patients
  - > Safety data from both patients show that KB103 was well tolerated, even after repeat administration. No serious adverse events, and no product-related adverse events, were reported.
- Top-line data from full study enrollment, including pediatric DEB patients is expected in H1 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

March 29, 2019

The European Medicines Agency (EMA) has granted access to its PRIME (PRIority MEdicines) scheme for KB103. The PRIME designation is awarded by the EMA to promising medicines that target an unmet medical need. Through PRIME, the EMA offers enhanced support to medicine developers including early interaction and dialogue, and a pathway for accelerated evaluation by the agency. The program is intended to optimize development plans and expedite the review and approval process so that these medicines may reach patients as early as possible. To be eligible and accepted for PRIME, a medicine has to show its potential to benefit patients with unmet medical needs based on early clinical data coupled with non-clinical data.

March 5, 2019

Official opening and inaugural ribbon cutting ceremony held in Pittsburgh for "Ancoris", the new cGMP facility, following successful completion of a trial run for KB103 manufacturing. The ribbon cutting ceremony was attended by City of Pittsburgh Mayor William Peduto, representatives from the Office of U.S. Senator Patrick J. Toomey and U. S. Senator Robert P. Casey, and Brett Kopelan, executive director of Debra of America, a nonprofit organization providing all-inclusive support to the epidermolysis bullosa (EB) community.

January 15, 2019

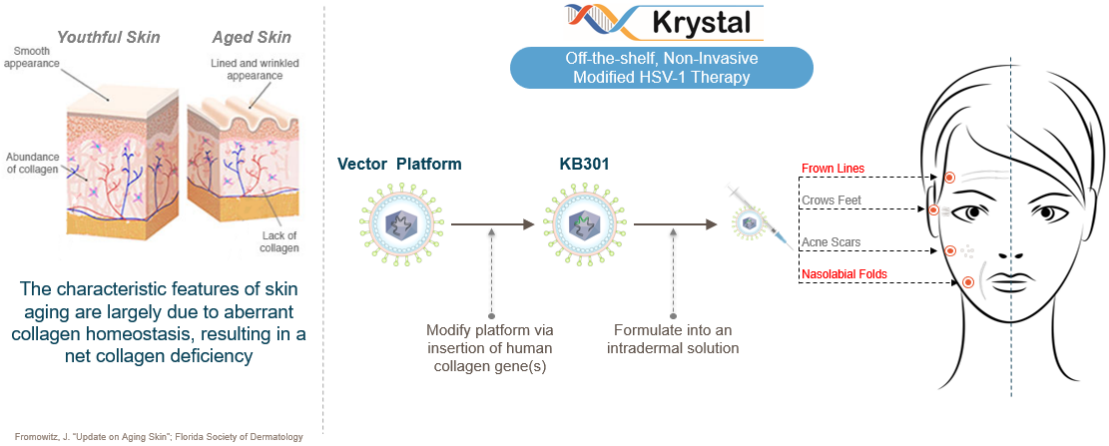
Construction of "Ancoris", a new state-of-the-art Good Manufacturing Practice (GMP) facility, is complete. The 4,500 square foot facility has been designed to satisfy the necessary manufacturing requirements for commercial development of KB103 and the highest current GMP standards governing commercial production for biopharmaceutical use.

Krystal Biotech

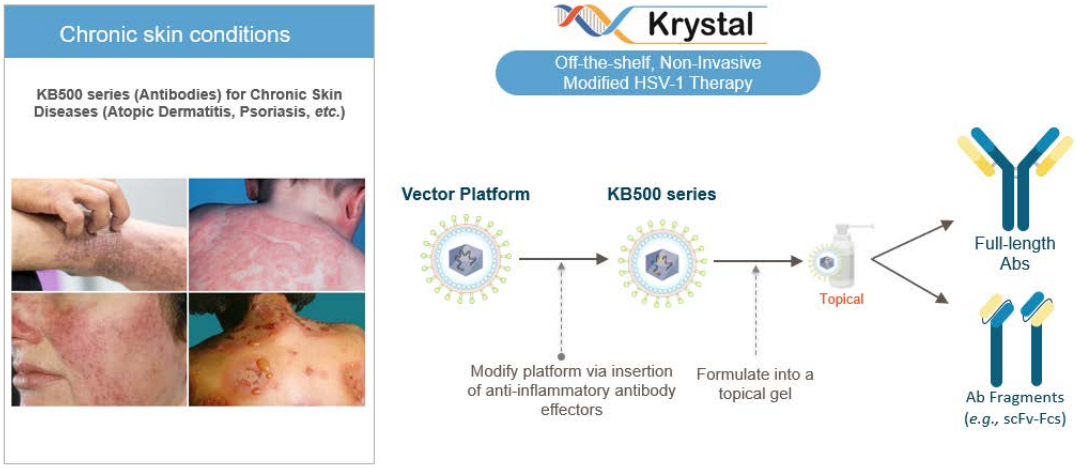
Investor Factsheet | Q2 2019

Future Opportunities

Application of fully-integrated vector platform to treat aesthetic defects



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



Key Value Drivers, 2019

- Announce final results for KB103 phase 1/2 trial; H1 2019
- Commence pivotal phase III trial for KB103; H2 2019
- Initiate phase 1 clinical trials for two pipeline candidates
  - > KB105 for TGM1-asssociated autosomal recessive congenital ichthyosis; Q3 2019
  - > KB301 for aesthetic conditions; H2 2019
- First GMP facility inauguration and operational in Q1 2019. Break ground on second GMP facility in H2 2019
- Preclinical work in chronic skin diseases; anticipate clinical program in 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

Partnering
inquiries@krystalbio.com