

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 Skin TARgeted Delivery platform, or STAR-D platform, is a proprietary 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 STAR-D 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 (STAR-D) and a pipeline of clinical and non-clinical effectors to target skin diseases and conditions.
 - > Zero royalty burden
- Use of STAR-D 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
 - > STAR-D-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 STAR-D platforms 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): 33% of fully diluted shares outstanding (as of 12/31/18)

Fast Facts

Ticker (Exchange)	KRYS (NASDAQ)
Stock Price	\$20.78
Market Cap	\$300 MM
Outstanding Shares	14.43 MM
52-Week Range	\$8.61 – \$28.75
Avg. Daily Volume	45,965
Headquarters	Pittsburgh, PA
Fiscal Year End	December 31
Cash, Cash Equivalents, and Short-Term Investments Position	\$111.8 MM
Insider ownership (management, employees, directors)	33% of fully diluted shares outstanding

All figures as of 12/31/2018

Management Team

Suma Krishnan
Founder and COO

Krish Krishnan
Chairman and CEO

Tony Riley
CFO

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 STAR-D 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

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.

October 23, 2018

Krystal closes previously announced underwritten public offering of 3,000,000 shares of its common stock, at a public offering price of \$20.00 per share. In addition, the underwriters fully exercised their option to purchase an additional 450,000 shares of the Company's common stock at the public offering price, less underwriting discounts. The gross proceeds to the Company from this offering were \$69.0 million, before deducting the underwriting discounts and commissions and other estimated offering expenses payable by the Company

October 15, 2018

Positive interim results from Phase I/II-GEM study of KB103 in DEB patients: <http://ir.krystalbio.com/sec-filings/sec-filing/8-k/0001193125-18-298897> <http://ir.krystalbio.com/node/6751/html>

August 23, 2018

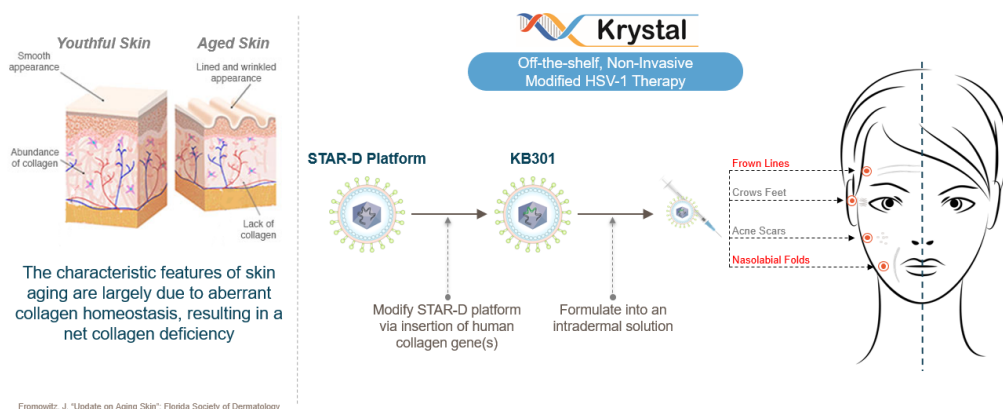
KB105 receives orphan drug and rare pediatric disease designation from FDA for treatment of patients with TGM-1 deficient autosomal recessive congenital ichthyosis. Eligible for priority review voucher (PRV).

Krystal Biotech

Investor Factsheet | Q1 2019

Future Opportunities

Application of STAR-D platform to treat aesthetic defects



Contacts

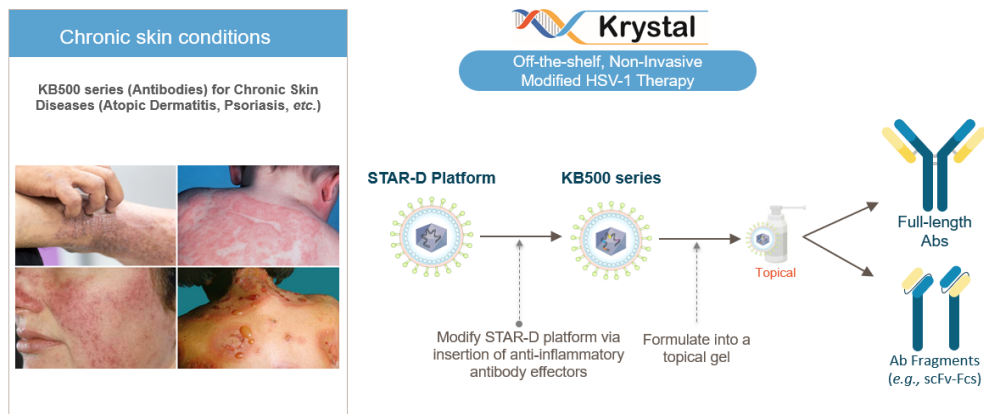
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Application of STAR-D platform to treat complex, chronic skin conditions



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-associated autosomal recessive congenital ichthyosis; H1 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