Krystal

Medicines for Rare Diseases –
An HSV-1 Based Gene Therapy Company

CORPORATE PRESENTATION
September 2020

Forward-Looking Statements

This presentation contains forward-looking statements that involve substantial risks and uncertainties. Any statements in this presentation about future expectations, plans and prospects for Krystal Biotech, Inc. (the "Company"), including but not limited to statements about the development of the Company's product candidates, such as the future development or commercialization of beremagene geperpavec ("B-VEC"), KB105, KB104, KB301 and KB407 and the Company's other product candidates; conduct and timelines of clinical trials, the clinical utility of B-VEC, KB105, KB104, KB301 and KB407 and the Company's other product candidates; plans for and timing of the review of regulatory filings, efforts to bring B-VEC, KB105, KB104, KB301 and KB407 and the Company's other product candidates to market; the market opportunity for and the potential market acceptance of B-VEC", KB105, KB104, KB301 and KB407 and the Company's other product candidates, the development of B-VEC, KB105, KB104, KB301 and KB407 and the Company's other product candidates for additional indications; the development of additional formulations of B-VEC, KB105, KB104, KB301 and KB407 and the Company's other product candidates; plans to pursue research and development of other product candidates, the sufficiency of the Company's existing cash resources; and other statements containing the words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "target," "potential," "likely," "will," "would," "could," "should," "continue," and similar expressions, constitute forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, including: the content and timing of decisions made by the U.S. Food and Drug Administration, European Medicines Agency and other regulatory authorities; the uncertainties inherent in the initiation and conduct of clinical trials, availability and timing of data from clinical trials; whether results of early clinical trials or studies in different disease indications will be indicative of the results of ongoing or future trials; uncertainties associated with regulatory review of clinical trials and applications for marketing approvals; the availability or commercial potential of product candidates; the ability to retain and hire key personnel; the sufficiency of cash resources and need for additional financing; and such other important factors as are set forth in the Company's annual and quarterly reports and other filings on file with the U.S. Securities and Exchange Commission. In addition, the forward-looking statements included in this presentation represent the Company's views as of the date of this presentation. The Company anticipates that subsequent events and developments will cause its views to change. However, while the Company may elect to update these forward-looking statements at some point in the future, it specifically disclaims any obligation to do so. These forward-looking statements should not be relied upon as representing the Company's views as of any date subsequent to the date of this presentation.

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Krystal Overview

A fully integrated, clinical stage gene therapy company powered by proprietary HSV-1 vector technology

Differentiated viral vector platform enables *in vivo,* repeat dose gene therapies

- Platform powered by proprietary, engineered *replication incompetent* HSV-1 vector
- Pre-clinical and clinical data shows maintenance of safety and transgene expression with repeat dosing; additional external clinical and regulatory precedent with *in vivo* HSV-1 based therapy

Initial focus on rare, dermatologic indications led to rapid clinical proof of concept and pipeline

- Lead program, B-VEC (formerly KB103) went from IND to Phase 3 in less than 3 years; pivotal data anticipated in 2021
- Two lead dermatologic pipeline programs, KB104 and KB105, leverage the same vector

In house GMP manufacturing to support both clinical and commercial needs

- Current ~7,500 sqft GMP facility near company headquarters in Pittsburgh is producing pivotal material at commercial scale, and BLA readiness is underway
- Investing in additional capacity via construction of an ~150,000 sqft facility which is expected to be operational in 2022

Leveraging platform to address larger indications, new tissues while investing in next gen tech

- Recently initiated a Phase 1 trial in acne scars and wrinkles with KB301, under our wholly owned subsidiary Jeune, Inc.
- Positive pre-clinical data from KB407 for cystic fibrosis demonstrates HSV-1 potential to target lung tissue; pre-IND studies underway
- Continue to drive innovation by investing in next-gen platform capabilities

Differentiated HSV-1 Based Platform Enables *In Vivo*, Repeat Dose Gene Therapies

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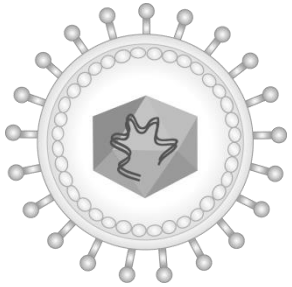
HSV-1 is Positively Differentiated vs. Other Gene Therapy Technologies

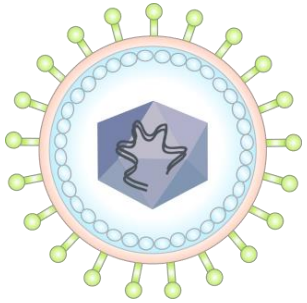
Wild-type HSV-1



HSV-1 has natural affinity for epithelial cells (in addition to several other cell types) with favorable immune-evasion mechanisms

Targeted mutagenesis resulting in platform that is both safe and effective for localized, repeat delivery of genetic payload

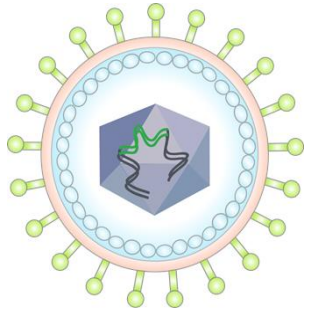
Krystal's Platform



Replication incompetent HSV-1 vector that can be administered repeatedly for chronic dosing

Insertion of one or more therapeutic effectors of interest into the vector

Therapeutic Vector



Large carrying capacity allows insertion of large transgenes, multiple copies of the transgene of interest and/or multiple different transgenes

Current Status of Viral and Non-Viral Gene Therapy Vectors

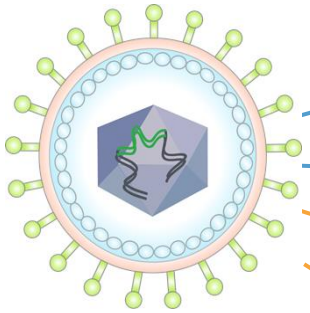
	LV	AAV	HSV-1	LNP
In Vivo Dosing?	No	Yes	**Yes**	Yes
Baseline antibody exclusion criteria?	No *(ex-vivo)*	Yes	**No**	No
Repeat-dose capabilities?	No	No	**Yes**	Yes
Carrying Capacity?	8 kb[1]	<4 kb[1]	**>30 kb**	~12 kb[2]
Integrates payload into host cell DNA?	Yes	No	**No**	No
Regulatory Precedent?	Yes	Yes	**Yes**	Yes

1. Lundstrom, K. Viral Vectors in Gene Therapy. *Diseases* 2018, 6, 42.
2. Generation Bio (GBIO) Prospectus. (2020, June 11). Retrieved September 4, 202020, https://www.sec.gov/Archives/edgar/data/1733294/000119312520167812/d924849d424b4.htm

LV = lentivirus
AAV = adeno-associated virus
LNP = lipid nanoparticle

Clinically Validated Platform Targeting Skin; Ongoing Investment in Next-Gen Technology to Broaden Platform Applicability

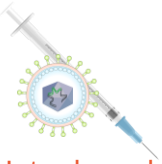
Therapeutic Vector



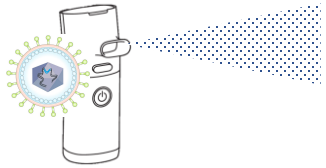
The current vector has been clinically validated to target skin cells. Preclinical exploration of its ability to target additional epithelial cell types is under way

Formulation / Route of Admin

Topical



Intradermal



Inhaled

Additional potential routes of administration under investigation

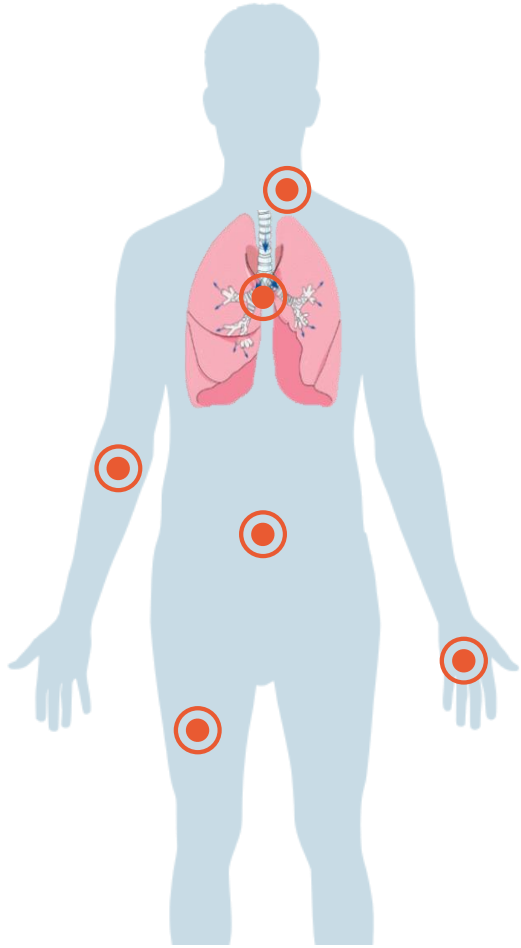
Drug Product

- ✓ B-VEC
- ✓ KB105
- • KB104
- • *Early stage research projects*

- ✓ KB105
- ✓ KB301
- • KB104
- • *Early stage research projects*

- • KB407
- • *Early stage research projects*

- • *Early stage research projects*



Current Pipeline

Product	Indication	Discovery	Preclinical	Phase I/II	Phase III	Milestones	WW Rights
B-VEC†¤•Δ‡§	Dystrophic EB					Topline Pivotal Data in 2021	Krystal
KB105†¤•‡	TGM1-deficient ARCI					Initiated Ph 2 in 2H 2020	Krystal
KB301	Aesthetic Skin Conditions					Initiated clinical trial in 2H 2020	Krystal
KB104	Netherton Syndrome					File IND in 2021	Krystal
KB407†	Cystic Fibrosis					File IND in 2021	Krystal
KB5XX	Chronic Skin Diseases						Krystal

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

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Initial Focus on Rare Skin Diseases Led to Rapid Clinical POC and Pipeline

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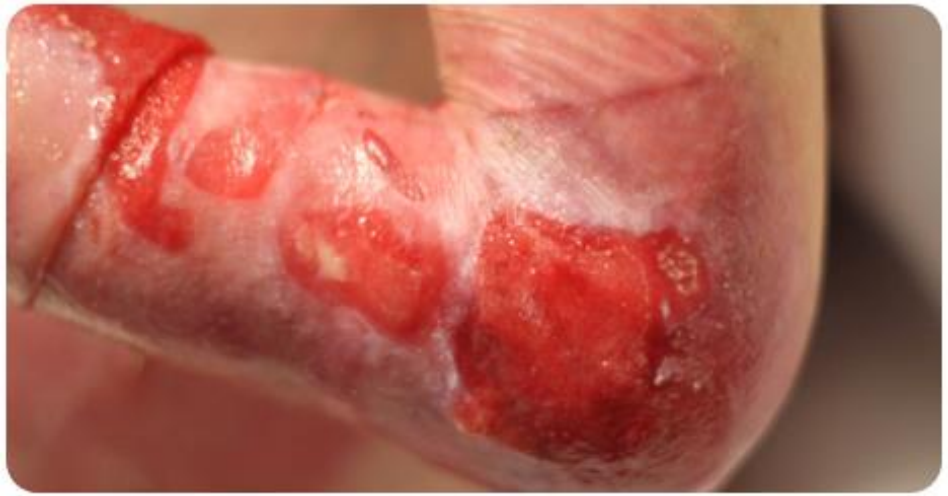
Dystrophic Epidermolysis Bullosa (DEB)

"Butterfly Children" is used to describe young DEB patients because their skin is as fragile as a butterfly's wings

Dystrophic Epidermolysis Bullosa

- A rare, genetic connective tissue disease that causes skin to tear or blister from minor contact
- Mutations in the *COL7A1* gene lead to absent or dysfunctional COL7 protein, without which the epidermis does not anchor to the dermis
- The recessive form (RDEB) is the classic, most severe form of the condition. Dominant DEB (DDEB) tends to be milder with blistering often limited to the hands, feet, knees, and elbows.



Epidemiology

- **Prevalence:** Up to 125,000 people are affected by DEB worldwide[1]
- **Incidence:** The incidence of DEB is 6.5 per million births in the US[2]

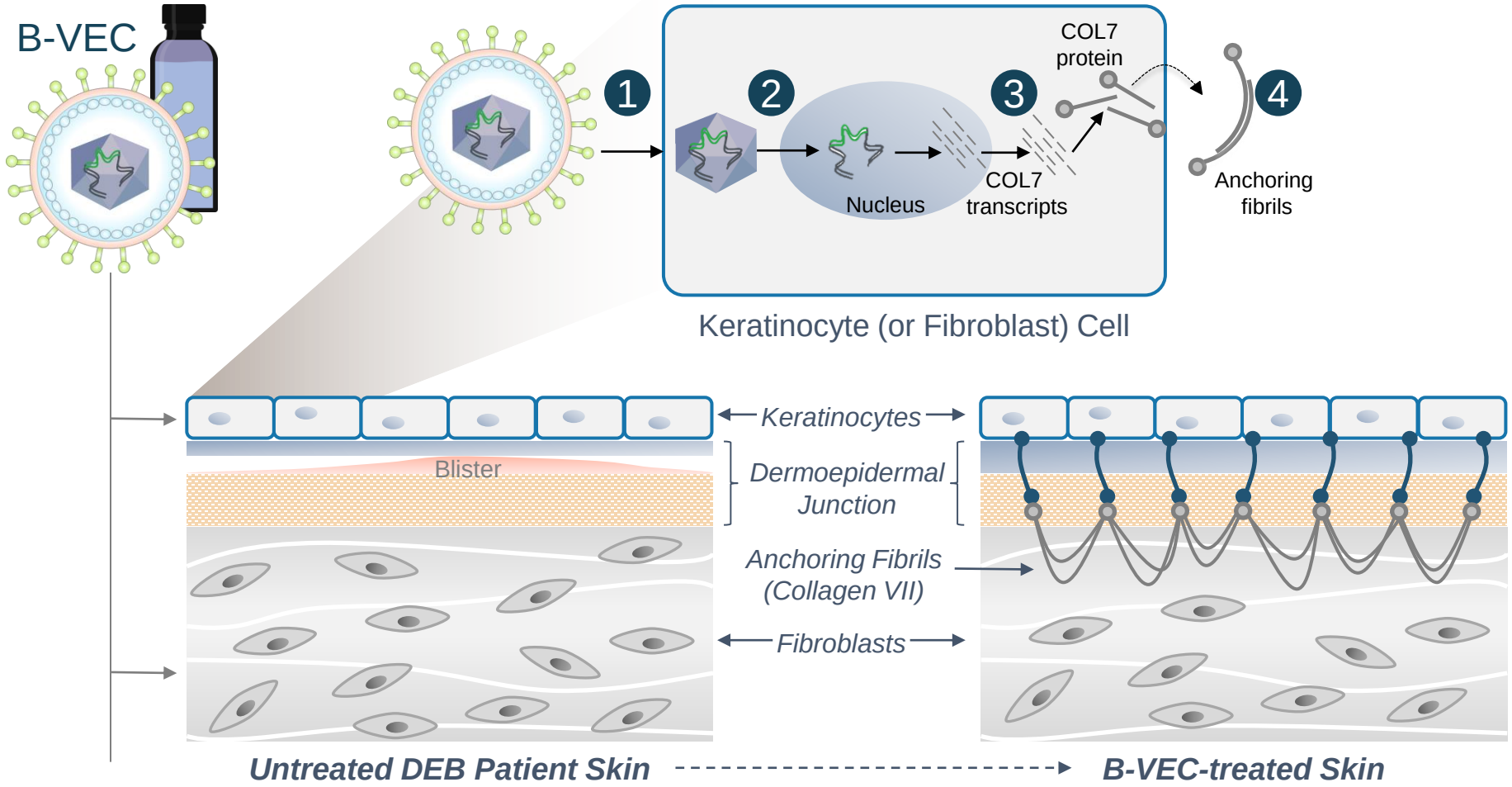
Current Standard of Care

- There are no approved treatments for DEB
- Existing therapies limited to expensive and time-consuming palliative treatments
- Palliative treatments cost $200k – $400k annually[3,4]

1. DEBRA International, http://www.debra-international.org/epidermolysis-bullosa/causes-and-subtypes.html; http://www.debra-international.org/what-is-eb/causes-and-subtypes/deb.html
2. Pfendner EG, Lucky AW. Dystrophic Epidermolysis Bullosa. 2006 Aug 21 [Updated 2015 Feb 26]. In: Adam MP, Ardinger HH, Pagon RA, et al., editors. GeneReviews® [Internet].
3. Rashidghamat E., Mellerio J.E., Management of chronic wounds in patients with dystrophic epidermolysis bullosa: challenges and solutions, Chronic Wound Care Management and Research Volume 2017:4, 45-54
4. GENEGRAFT Report Summary. (2015, February 16). Retrieved December 13, 2016, from http://cordis.europa.eu/result/rcn/156078_en.html

Beremagene Geperpavec (B-VEC) for DEB

B-VEC is a topically administered, replication-deficient HSV-1 vector containing two functional COL7A1 genes applied directly to DEB patient wounds in an outpatient setting.



1. B-VEC enters the compromised skin of DEB patients and transduces both keratinocytes and fibroblasts
2. Once in the nucleus of transduced cells the vector genome is deposited (episomally)
3. As a result, *COL7A1* transcripts are generated, allowing the cell to produce and secrete functional COL7 protein
4. The secreted COL7 protein assembles into anchoring fibrils which hold the epidermis and dermis together

Topical B-VEC Was Evaluated in a Phase 1/2 Study at Stanford

Design

- GEM1/2 (NCT03536143) was an intra-patient comparison of wounds randomized to receive either topical B-VEC or placebo.
- Each patient on-study for ~6 months; 3 months of on-site visits followed by 3-month at-home imaging period
- *Study PI: Dr. Peter Marinkovich*

Enrollment

- A total of 9 RDEB subjects (adult and pediatric) were enrolled in the study; 3 subjects enrolled early and completed the study were subsequently re-enrolled and new wounds were randomized

Dosing

- In the Ph1 portion (n=2) one wound was administered B-VEC and one wound was administered placebo at a dose of 1e8 PFU/wound with varying frequency throughout the study period
- In Phase 2 portion (n=10) 2 wounds were administered B-VEC and one wound was administered placebo (except 1 patient who was 1:1) at doses of either 2e8, 3e8, 6e8 or 8e8 PFE/wound with varying frequency throughout the study period

Key Endpoints

Safety Measures

- AEs, including clinically significant changes in laboratory results, vitals, and physical exam findings
- Viral shedding was analyzed through the collection of blood, urine, and skin swabs, and antibodies to HSV and COL7 were analyzed through collection of serum

Efficacy measures

- Level of collagen VII (COL7) in B-VEC-administered skin as measured by immunofluorescence; presence of anchoring fibrils as measured by immunoelectron microscopy
- Wound closure (change in wound surface area relative to baseline), time to wound closure, and duration of wound closure, all relative to placebo

Topical B-VEC was Safe; COL7 expression and molecular correction established

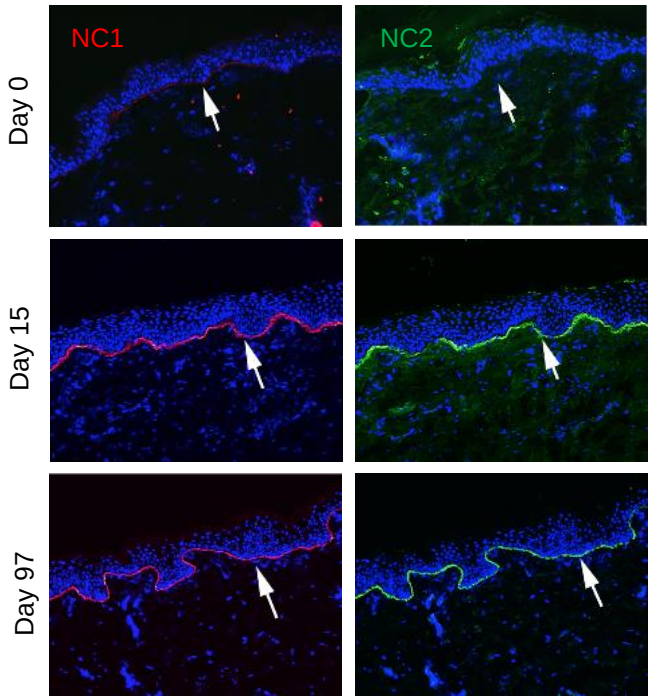
B-VEC Was Well Tolerated Following First and Repeat Dosing

- **No treatment-related serious AEs were reported; AEs deemed possibly related were mild (n=7) or moderate (n=1) and self limiting**
- No immune response or blistering observed around the sites of administration following first and repeat doses
- Blood and urine samples collected throughout the study revealed:
  - No systemic viral shedding
  - No adverse events associated with routine labs (chemistry and hematology)
- **Some patients had baseline COL7 and HSV-1 antibodies which did not impair efficacy or tolerance of therapy**

Molecular correction established and correlates with wound healing

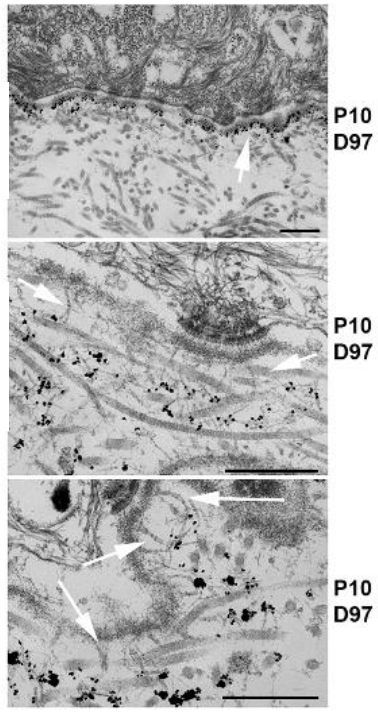
- Expression and correct localization of full-length COL7 following B-VEC therapy, which *promoted the formation of mature anchoring fibrils in all biopsy samples*

Baseline, Days 15 and 97 collagen VII expression using NC1 and NC2 specific antibodies (patient 10)



Arrows indicate basement membrane zone

Immunoelectron microscopy shows mature anchoring fibrils at day 97 (patient 10)

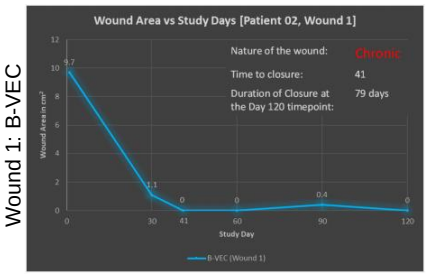


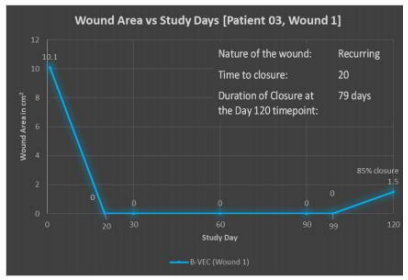
Statistically Significant Reduction in Wound Area achieved in Weeks 8,10 and 12

Efficacy was Observed in both Recurrent and Chronic Wounds

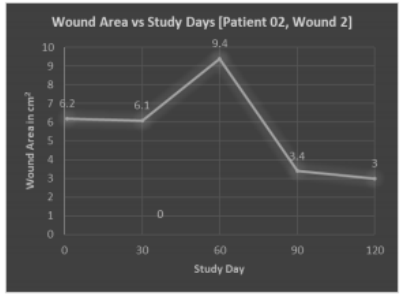
Patient 2 | Patient 3

Wound 1: B-VEC

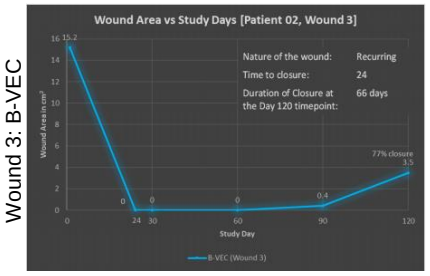


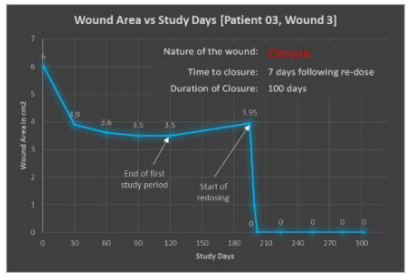


Wound 2: Placebo

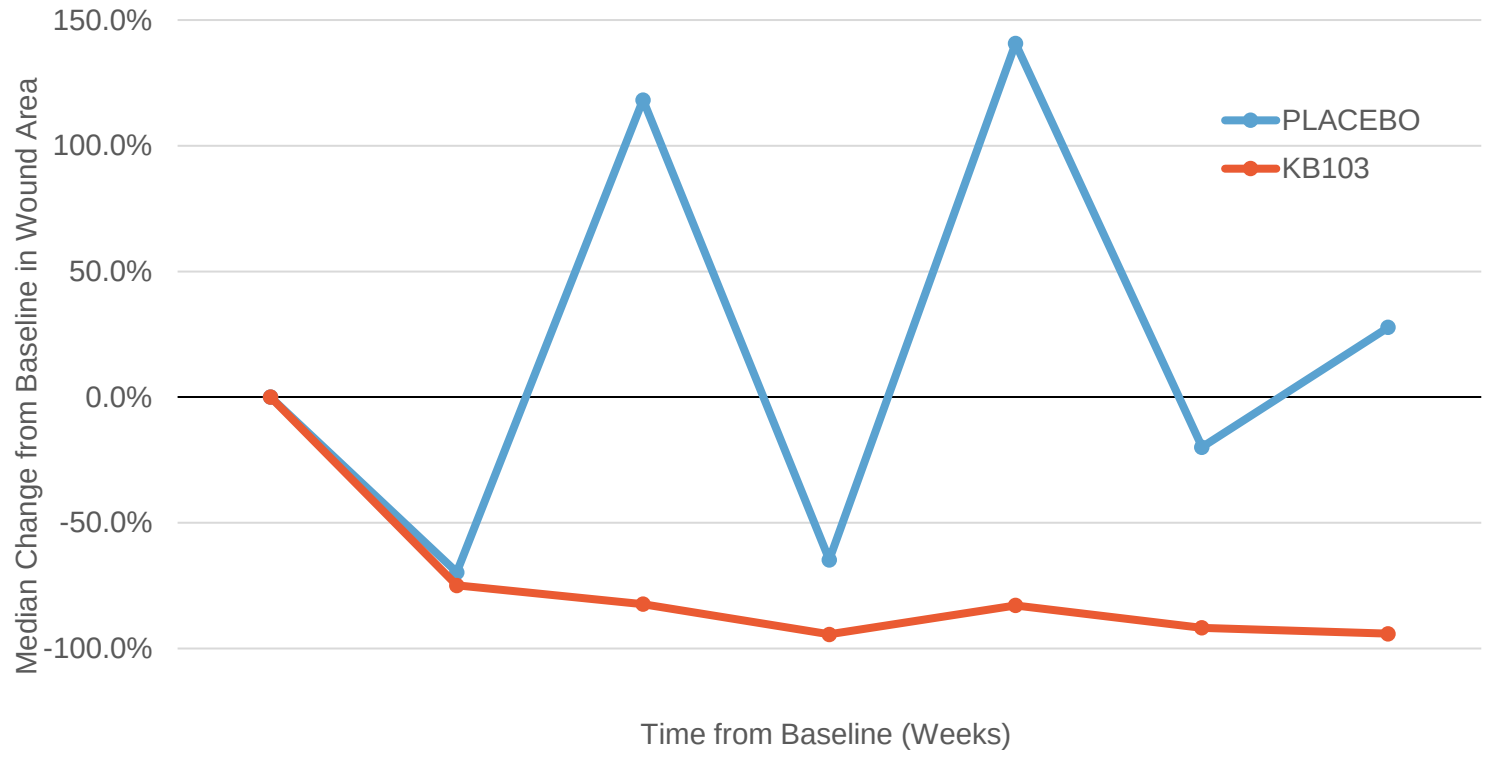


Wound 3: B-VEC





Median Change in Wound Area Across Phase 1/2 Study



	0	2	4	6	8	10	12
PLACEBO	0.0%	-69.7%	118.3%	-64.7%	140.7%	-19.9%	27.8%
KB103	0.0%	-74.9%	-82.3%	-94.4%	-82.8%	-91.8%	-94.1%
p-value **		0.298	0.004	0.071	0.002	0.015	0.020

** Based on Wilcoxon Rank-Sum Test

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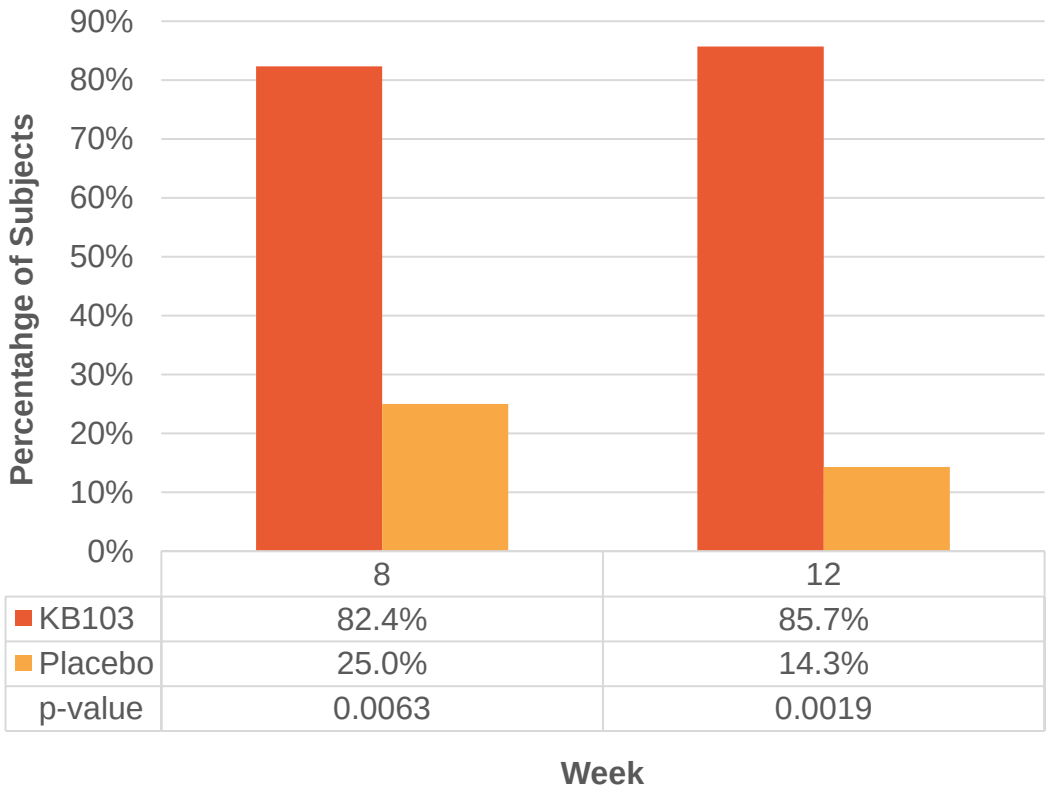
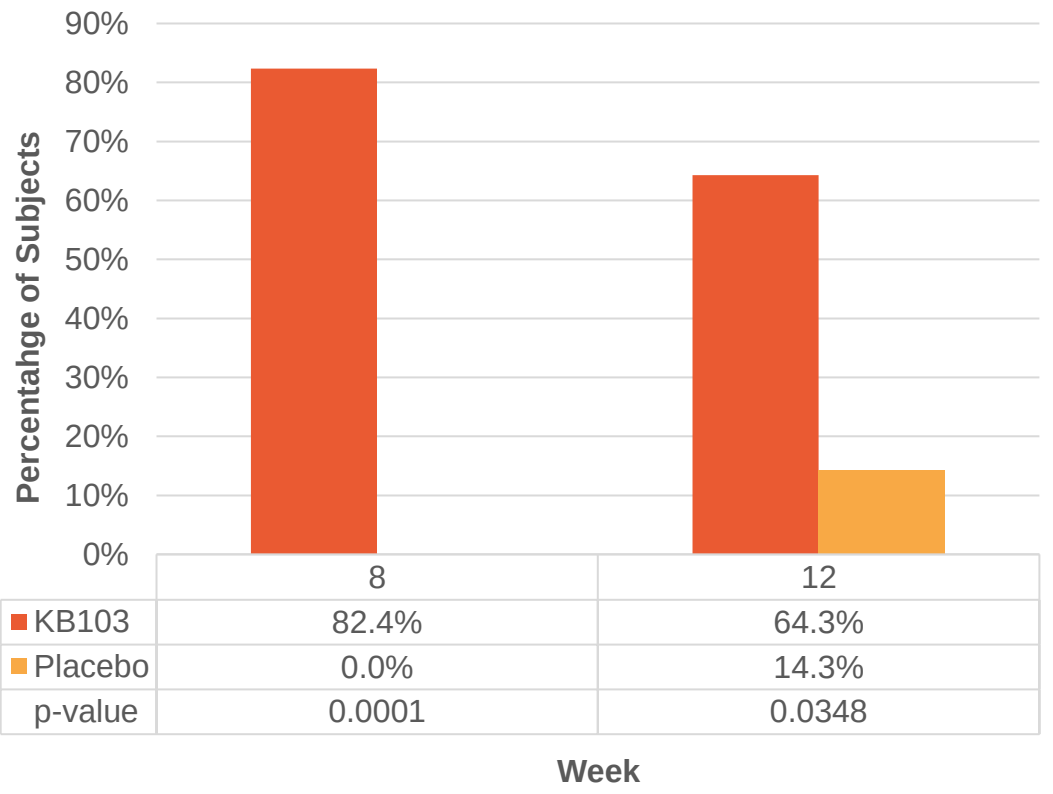
Wound Closure (>75% and 100%) for B-VEC vs. Placebo at Weeks 8 and 12

Change from Baseline Wound Area ≥75%



	8	12
KB103	82.4%	85.7%
Placebo	25.0%	14.3%
p-value	0.0063	0.0019

Change from Baseline Wound Area = 100%



	8	12
KB103	82.4%	64.3%
Placebo	0.0%	14.3%
p-value	0.0001	0.0348

**p-values are based on Cochran-Mantel Haenszel (CMH) Test Without Adjusting for Week-to-Week Placebo Variability*

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The Pivotal GEM-3 Study is Currently Enrolling

Design

- GEM-3 (NCT04491604) is a randomized, double-blind, intra-patient comparison of wounds randomized to receive either topical B-VEC or placebo.
- Each patient on-study for approximately 7 months: the 6-month dosing period followed by a 30-day safety follow up

Enrollment

- Approximately 30 DEB subjects (adult and pediatric) will be enrolled across 6 trial sites in the US
- Each subject provides at least 1 pair (up to 3) of primary target wounds, 1 randomized to B-VEC and the other to placebo
- In addition to the primary target wound pair(s), additional wounds (secondary wounds) may be selected to be treated with B-VEC in an open-label manner

Key Efficacy Endpoints

Primary

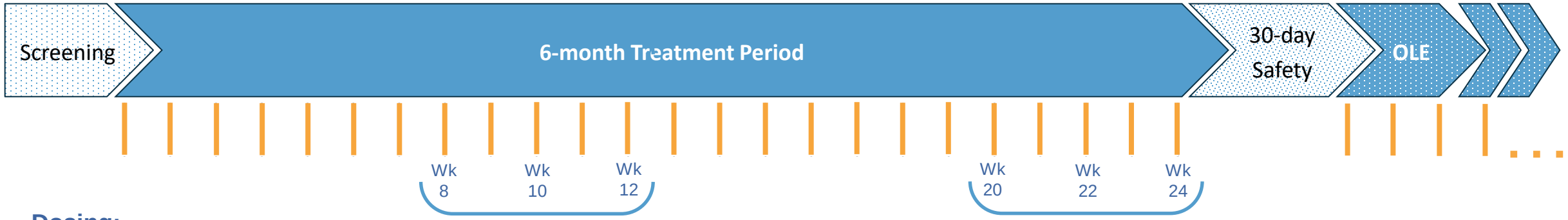
- Complete wound healing, determined by the Investigator, as compared to baseline in B-VEC treated wounds versus placebo treated at weeks 20, 22 and 24

Secondary

- Complete wound healing, determined by the Investigator, as compared to baseline in B-VEC treated wounds versus placebo at weeks 8, 10 and 12
- Mean change in pain severity (using either a VAS or FLACC-R Scale) per primary wound site associated with wound dressing changes
- The proportion of primary wound sites with ≥75% would healing as compared to baseline at Week 24 using Canfield photography quantitation

Phase 3 Design Optimized for Clinical and Commercial Success

The trial is designed to maximize chances of success while maintaining potential for a broad label, inclusive of chronic and recurring wounds of any size in RDEB or DDEB patients



Dosing:

- Primary wounds will be treated once weekly with a fixed dose until wound closure; should a wound re-open, weekly dosing will resume at the assigned dose until wound closure
- The fixed dose per wound is dependent on the size of the wound at baseline and ranges from $4x10^8$ to $1.2x10^9$ PFU per wound
- Each patient is allowed a maximum weekly dose of B-VEC; if that maximum is not reached in dosing primary wounds, additional secondary wounds may be chosen and treated with B-VEC in an open label manner
- The maximum weekly dose, administered once weekly per patient, is defined by patient age (right)

Maximum Weekly Dose Per Subject:	
Age	**Max Weekly Dose**
≥ 6 months to < 3 years	1.6x10^9 PFU/week
≥ 3 years to < 6 years	2.4x10^9 PFU/week
≥ 6 years	3.2x10^9 PFU/week

Key Design Elements:

- ✓ No restriction on chronic or recurring wounds
- ✓ Maximum weekly dose allows for flexibility to treat multiple and / or larger wounds
- ✓ Inclusive of RDEB *and* DDEB patients

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Autosomal Recessive Congenital Icthyosis Associated With TGM1 mutations

Autosomal Recessive Congenital Ichthyosis (ARCI) associated with TGM1

- The most common form of ARCI is caused by an inactivating mutation in the TGM1 gene encoding the enzyme transglutaminase-1, a protein that is essential for the proper formation of the skin barrier
- The condition is characterized by thick dry scaly skin, increased trans-epidermal water loss (TEWL), risk for dehydration, sepsis, skin malignancies, etc.

Epidemiology[1-8]

- **Prevalence:** There are approximately 20,000 people affected by TGMI related icthyosis worldwide (~1,800 US; 3,000 EU; 18,000 ROW)
- **Incidence:** It is estimated that around 350-400 babies are born with the condition each year, worldwide

Current Standard of Care

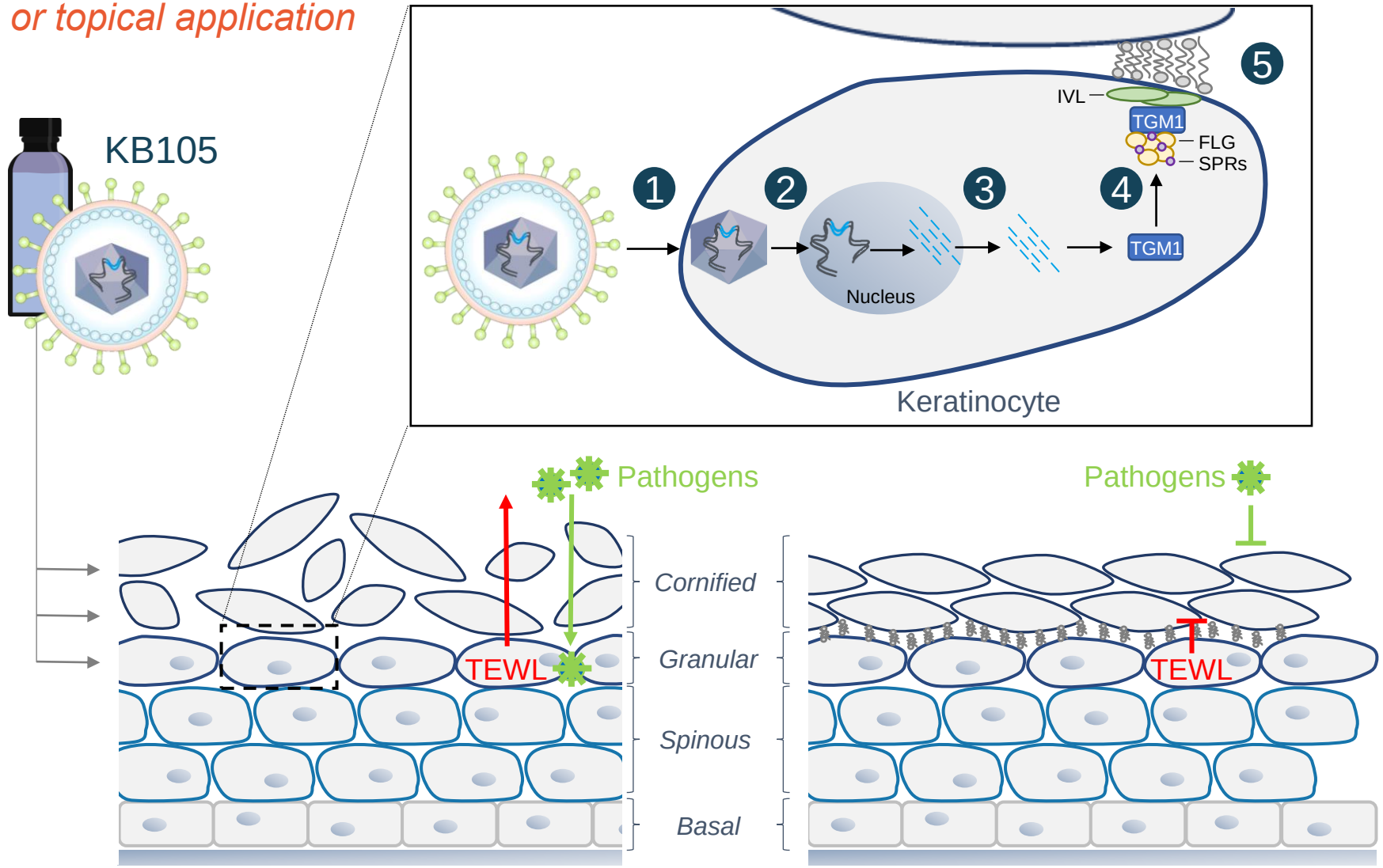
- There are no approved treatments for ARCI associated with TGM1
- Topical and systemic retinoids and time-consuming supportive treatments (up to 4 hours a day of skin care) are most often used

1. Rodriguez-Pazos et al. *Actas Dermosifiliogr.* 2013 May;104(4):270–284;
2. Dreyfus et al. *Orphanet J Rare Dis.* 2014 Jan 6;9:1;
3. Hernandez-Martin et al. *J Am Acad Dermatol.* 2012 Aug;67(2):240–244;
4. Pigg et al. *Eur J Hum Genet.* 1998 Nov-Dec;6(6):589–596.
5. Pigg et al. *Acta Derm Venereol.* 2016 Nov 2;96(7):932–937;
6. Orphanet;
7. Foundation for Ichthyosis & Related Skin Types (FIRST);
8. National Organization for Rare Disorders (NORD).

KB105 for TGM1 associated ARCI

KB105 is a replication-deficient HSV-1 vector that delivers multiple copies of human transglutaminase 1 ("TGM1") via intradermal injection or topical application

1. KB105 enters permeabilized skin and transduces keratinocytes (native TGM1-producing cells)
2. KB105 is transported into the nucleus of transduced cells and the vector genome is deposited (episomally)
3. *TGM1* transcripts are generated, which allows the cell to produce functional TGM1 protein that localizes to the cell membrane
4. TGM1 crosslinks target proteins (*e.g.*, filaggrin (FLG), involucrin (IVL), small proline-rich proteins (SPRs)) to aid in the formation of the cornified cell envelope
5. This layer, known as the stratum corneum, acts as a mechanical barrier to protect against transepidermal water loss (TEWL) and entry of infectious agents



KB105 is Being Evaluated in a Phase 1/2 Study

Design

- The Ph1/2 trial (NCT04047732) is an open label, intra-patient comparison of KB105 and placebo
- Each patient on-study for approximately six months; 3 months of on-site visits followed by 3-month at-home imaging period
- *Study PI: Dr. Amy Paller*

Enrollment

- ~6 TGM1-ARCI subjects will be enrolled across 2 sites; three Ph1 patients were enrolled at Paddington Testing Company (Philadelphia); Ph2 subjects will be enrolled at Northwestern University (Chicago)

Dosing

- In the Ph1 portion (n=3) one or two ~20cm^2 target areas were administered placebo, and 3 target areas were administered 2x10^9 PFU with varying frequency over ~60-90 days
- In Ph1, topical and microneedle administration was evaluated; in Ph2 topical administration will be utilized

Key Endpoints

Safety Measures

- AEs, including clinically significant changes in laboratory results, vitals, and physical exam findings
- Viral shedding analyzed through the collection of blood, urine, and skin swabs; antibodies to HSV and TGM1 analyzed through collection of serum

Efficacy measures

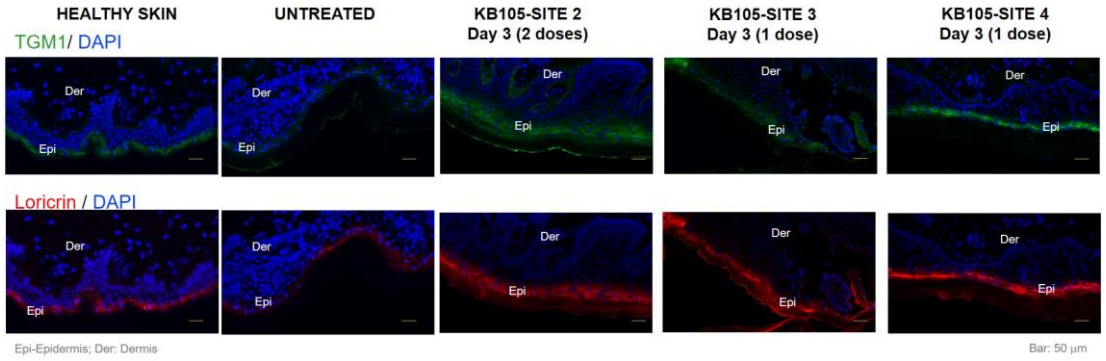
- Level of transglutaminase 1 in KB105-administered skin as measured by immunofluorescence microscopy
- Improvement of disease severity in the treatment area assessment through Investigator's Global Assessment (IGA)
- Improvement of disease severity in the treatment area through use of the Visual Index for Ichthyosis Severity scale, lamellar (VIIS-L) standard assessment

Initial Data Shows KB105 safe; Molecular and Phenotypic Improvement Evident

KB105 Was Well Tolerated and Generated Functional TGM1 protein

- Repeat dosing with KB105 was well-tolerated with **no drug related AEs and no immune response** to HSV or TGM1
- **No vector shedding** detected in swabs, blood or urine in all three patients
- KB105 treatment **restored functional TGM1 protein expression** and activity in all treated sites
- **KB105-expressed TGM1 was correctly localized** in the epidermis, colocalizing with Loricrin, and was functionally active
- qPCR, IF, and in situ analyses demonstrated **similar delivery efficacy of TGM1 DNA from single and repeat administration**

Subject 1: Treatment Restored TGM1 Expression to Normal Levels

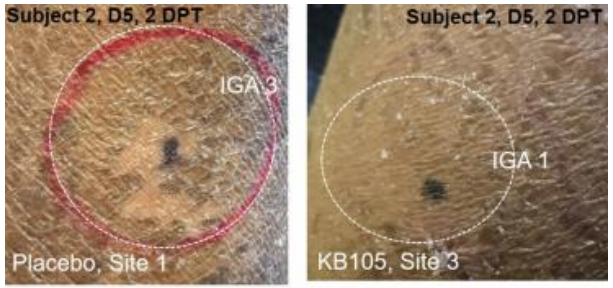


Phenotypic Improvement Evident After Topical and Microneedle Application

- Similar delivery efficacy with and without microneedling; no microneedling required in future studies
- Phenotypic evaluation limited by small treatment areas, but KB105 treated areas showed reduced reversion to ichthyotic scaling phenotype

DPT: Days Post Treatment

DPRT: Days Post Re-Treatment



In-House GMP Manufacturing to Support both Clinical and Commercial Needs

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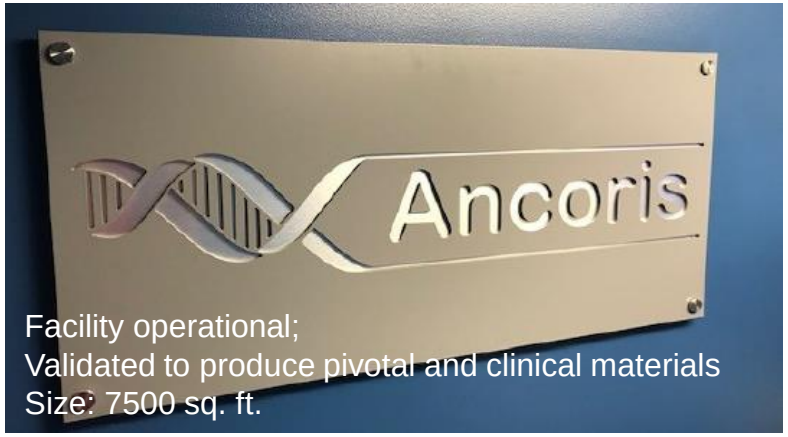
Platform Supported by In House Manufacturing Capacity and Expertise

Established process conducted at Krystal's end-to-end GMP facility (Ancoris)

- ✓ Maintains control of IP/trade secrets relating to manufacturing process
- ✓ Adheres to internal process and production schedules, avoiding use of high demand gene therapy CMOs



Facility operational;
Validated to produce pivotal and clinical materials
Size: 7500 sq. ft.

Upstream Production Process

- Proprietary engineered vectors and complementary/supporting cell lines developed in-house are used in established methods for production of consistent batches
- Scalable from clinical phase to commercial

Downstream Purification Process

- Work conducted in an aseptic closed system process
- Process accommodates ever-expanding vector pipeline with minimal redevelopment effort between product candidates
- Compliant to global regulatory requirements


ASTRA (rendering)
Operational in 2022
Size: 150,000 sq. ft.

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Leveraging Platform to Target New Tissues and Larger Indications

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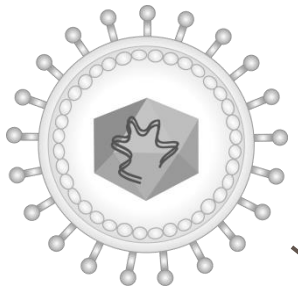
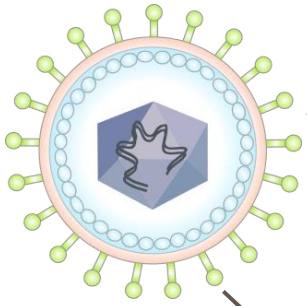
HSV-1 Has Potential Beyond Rare Skin

Wild-type HSV-1

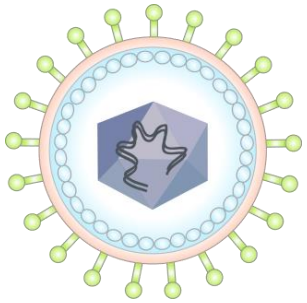


HSV-1 has natural affinity for **epithelial cells,** in addition to several other cell types, with favorable immune-evasion mechanisms

Krystal's Current Platform



Investing in Next Gen Technology to Maintain Leadership in HSV-1 Space



Skin

- Rare Skin Disease
  - ✓ B-VEC (COL7A1) for DEB
  - ✓ KB105 (TGM1) for TGM1-ARCI
  - • KB104 (SPINK5) for Netherton's Disease
  - • Additional preclinical candidates
- Aesthetics (Jeune Inc.)
  - ✓ KB301 (COL3) for Acne Scars and Wrinkles
  - • Additional preclinical candidates
- Different Diseases
- Different Effectors (e.g. antibodies, RNAi)

Lung

- Rare Lung Disease
  - • KB407 (CFTR) for Cystic Fibrosis
  - • Additional preclinical candidates
- Different Diseases
- Different Effectors (e.g. antibodies, RNAi)

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KB407 for Cystic Fibrosis

We are developing KB407 as an inhalable, repeat dose gene therapy that delivers the full human CFTR gene

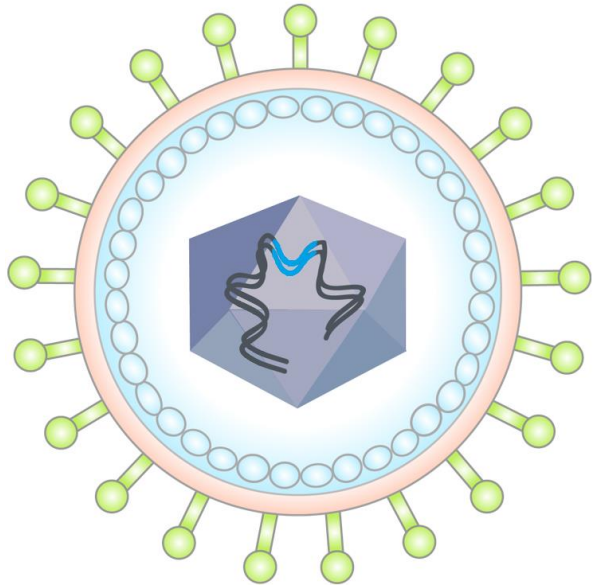
Gene therapy is an attractive modality for cystic fibrosis, though prior attempts have been unsuccessful

- Viral (adenovirus and AAV) and non-viral (DNA plasmids and stabilized mRNA) approaches have been tested in more than 25 clinical trials enrolling >470 patients
- Past approaches suffer from some combination of physical limitations for large cargo, low efficiency of gene transfer, toxicity, immune intolerance, product instability, and burdensome delivery

Our HSV-1 vector has potential to overcome prior limitations

- Accommodates large genes and necessary regulatory elements
- Is amenable to rapid, non-invasive inhaled administration
- Natural tropism to epithelial cells, e.g., those lining the airways
- Non-cytotoxic
- Non-immune stimulating
- Safe and effective for repeated administration in highly inflammatory environments

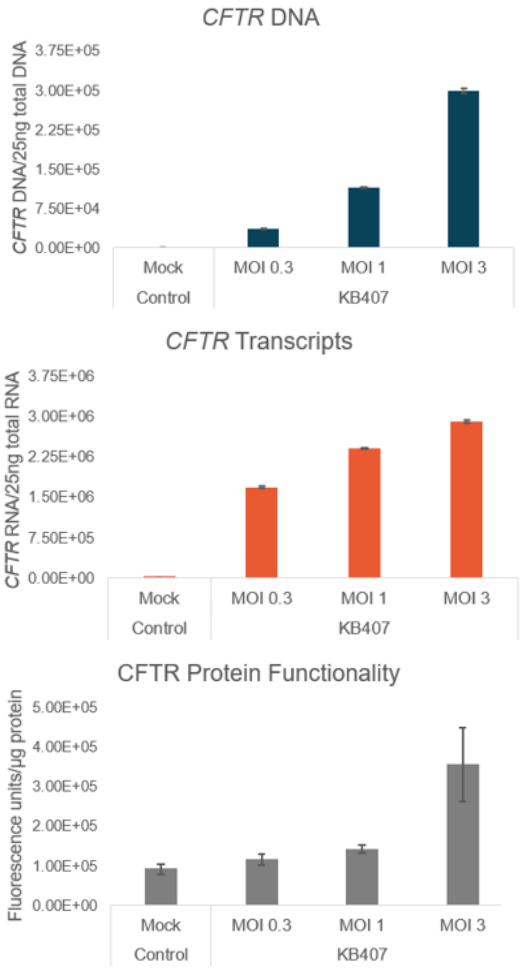
KB407



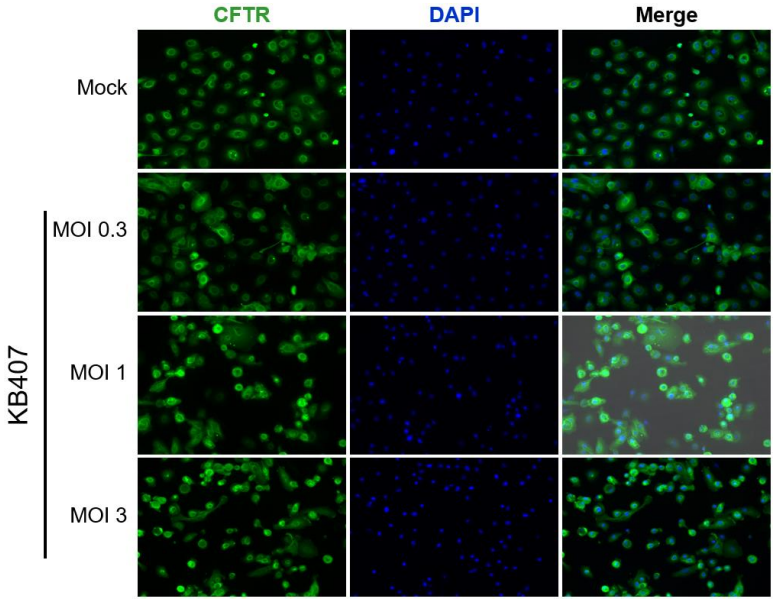
A replication-incompetent HSV-1 vector expressing full-length human CFTR

Efficient Cell Targeting, CFTR Expression and Function In Vitro

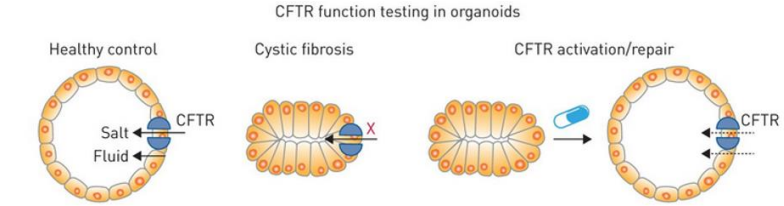
Robust, dose-dependent CFTR expression and functional correction in 2D Airway Epithelial Cell Culture



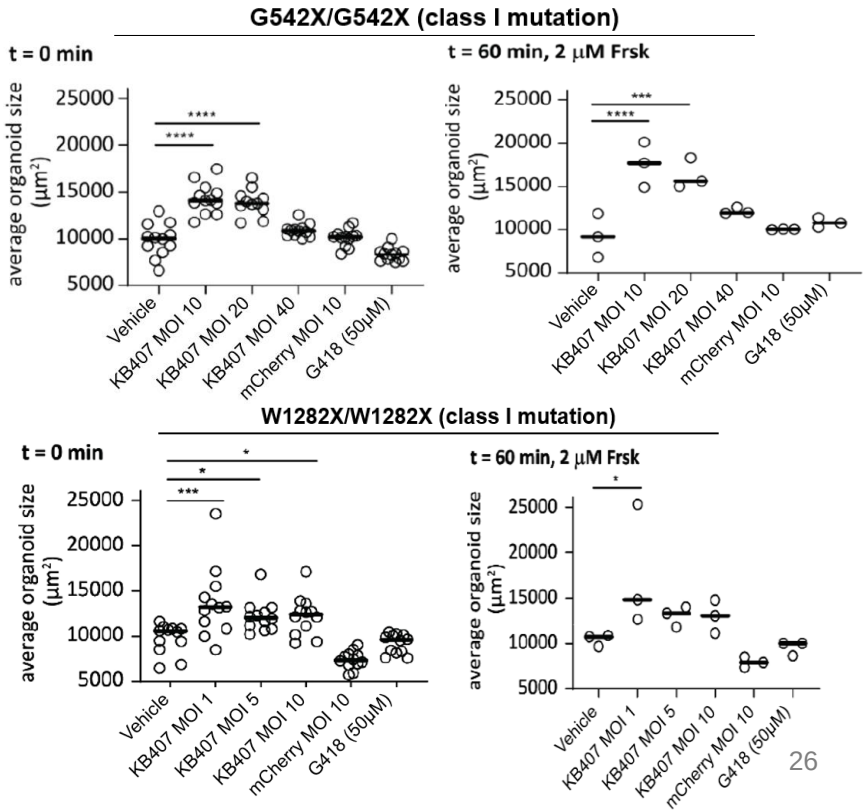
KB407-expressed human CFTR properly localizes to the plasma membrane of transduced cells



KB407-mediated functional correction of CF phenotype in clinically relevant 3D organotypic system (HUB)



*Van Mourik *et al.* (2019). "Intestinal organoids to model cystic fibrosis".



Financials and Milestones

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Krystal Summary

A fully integrated, clinical stage gene therapy company powered by proprietary HSV-1 vector technology

Current Status and Milestones

Rare Skin

- **B-VEC:** Pivotal GEM-3 Trial currently enrolling; topline data expected 2021. Commercial planning in US and EU underway.
- **KB105:** 4th patient (first Ph2 patient) recently enrolled to evaluate higher dose and larger treatment area. Data from this patient will guide next steps in 2021
- **KB104:** preclinical work ongoing; IND anticipated in 2021

Aesthetics (Jeune Inc.)

- **KB301:** Phase 1 trial in acne scars and wrinkles recently initiated; Pre-clinical data to be presented at the American Society for Dermatologic Surgery in October; initial Ph1 safety data anticipated YE20/early 2021
- Update on Jeune Inc. / aesthetics strategy in 2021

Lung

- **KB407:** pre-IND work ongoing; in vivo data will be presented in 4Q20; IND anticipated in 2021

Platform

- **Manufacturing:** Astra facility construction underway, completion anticipated in 2022
- **Next Gen Tech:** Evaluation of novel effectors, routes of administration, and tissue tropism underway

Cash balance at end of 2Q20: ~$297M

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Medicines for Rare Diseases –
An HSV-1 Based Gene Therapy Company

CORPORATE PRESENTATION
September 2020

Appendix

Links to Scientific Presentations

B-VEC

- May 2020: Complete Phase 1/2 at SID 2020 – Link
- October 2019: Phase 1/2 Data Update – Link
- June 2019: Phase 1/2 Data Update – Link
- May 2018: Preclinical Poster at IID 2018 – Link
- September 2017: In Vitro preclinical data at EB2017 - Link

KB105

- May 2020: Initial Phase 1/2 at SID 2020 - Link
- May 2019: In Vivo preclinical data at SID 2019 – Link
- May 2019: In Vitro preclinical data at SID 2019 – Link

KB104

- May 2019: In Vitro preclinical data at SID 2019 - Link

KB407

- May 2020: In Vitro preclinical data at ASGCT 2020 - Link