Krystal

The Leader in **Redosable Gene Therapy** for Rare Disease

January 2022

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 VYJUVEK™ (beremagene geperpavec) and KB408 and the Company's other product candidates; conduct and timelines of preclinical and clinical trials, the clinical utility of VYJUVEK™ and the Company's other product candidates; plans for and timing of regulatory filings and, efforts to bring VYJUVEK™ and the Company's other product candidates to market; the market opportunity for and the potential market acceptance of VYJUVEK™ 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 redosable gene delivery platform

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

- Proprietary, engineered *replication incompetent* HSV-1 based platform
- Clinical data shows maintenance of safety and transgene expression after repeat dosing
- Positive 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, VYJUVEK™ (beremagene geperpavec) went from IND to Phase 3 in less than 3 years; BLA filling anticipated in 1H22, MAA shortly after
- Two lead dermatologic pipeline programs, KB104 and KB105, leverage the same vector

Broadening focus to address larger indications and new tissue types

- Ongoing Phase 1 trial in aesthetic skin indications with KB301, under our wholly owned subsidiary Jeune Aesthetics, Inc.
- Positive pre-clinical data from KB407 for cystic fibrosis demonstrates potential to target lung tissue
- Continue to drive innovation by investing in next-gen platform capabilities

In-house GMP manufacturing to support clinical and commercial needs

- Stable producer cell line developed and leveraged across pipeline has cost, scale, and regulatory benefits
- Current GMP facility near company headquarters in Pittsburgh is producing pivotal material at commercial scale; BLA readiness is underway
- Investing in additional capacity via construction of an ~150,000 sqft facility which is expected to be operational in 2022

Upcoming Milestones

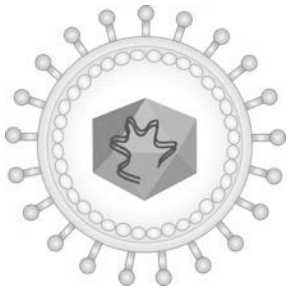
Timing	Program	Event
1Q22	KB301 for aesthetic indications	Initial POC efficacy data from Phase 1 study
1H22	VYJUVEK™ for Dystrophic EB	File BLA with US FDA
1H22	VYJUVEK™ for Dystrophic EB	Present more detailed GEM-3 results at medical congress
1H22	KB407 for cystic fibrosis	Initiate Phase 1 clinical trial in Australia
2H22	VYJUVEK™ for Dystrophic EB	Fila MAA with EMA
2H22	KB407 for cystic fibrosis	File IND / Initiate clinical trial in US
2022	KB105 for TGM1-ARCI	Initiate dosing in next Phase 1/2 cohort
2022	KB104 for Netherton	File IND and initiate clinical trial

All pipeline compounds are investigational, being evaluated in clinical or pre-clinical studies.

Our technology platform enables noninvasive, redosable gene therapy

HSV-1 is positively differentiated vs. other gene therapy technologies

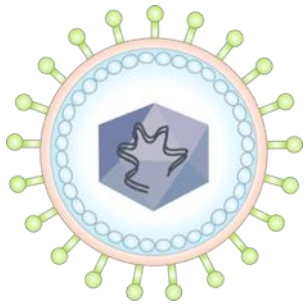
Wild-type HSV-1



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

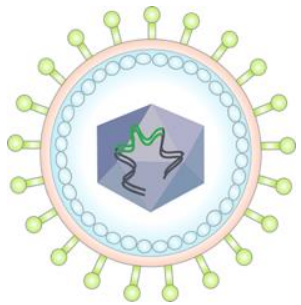
Krystal's Modified HSV-1 Platform



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

Vector Platform Comparison

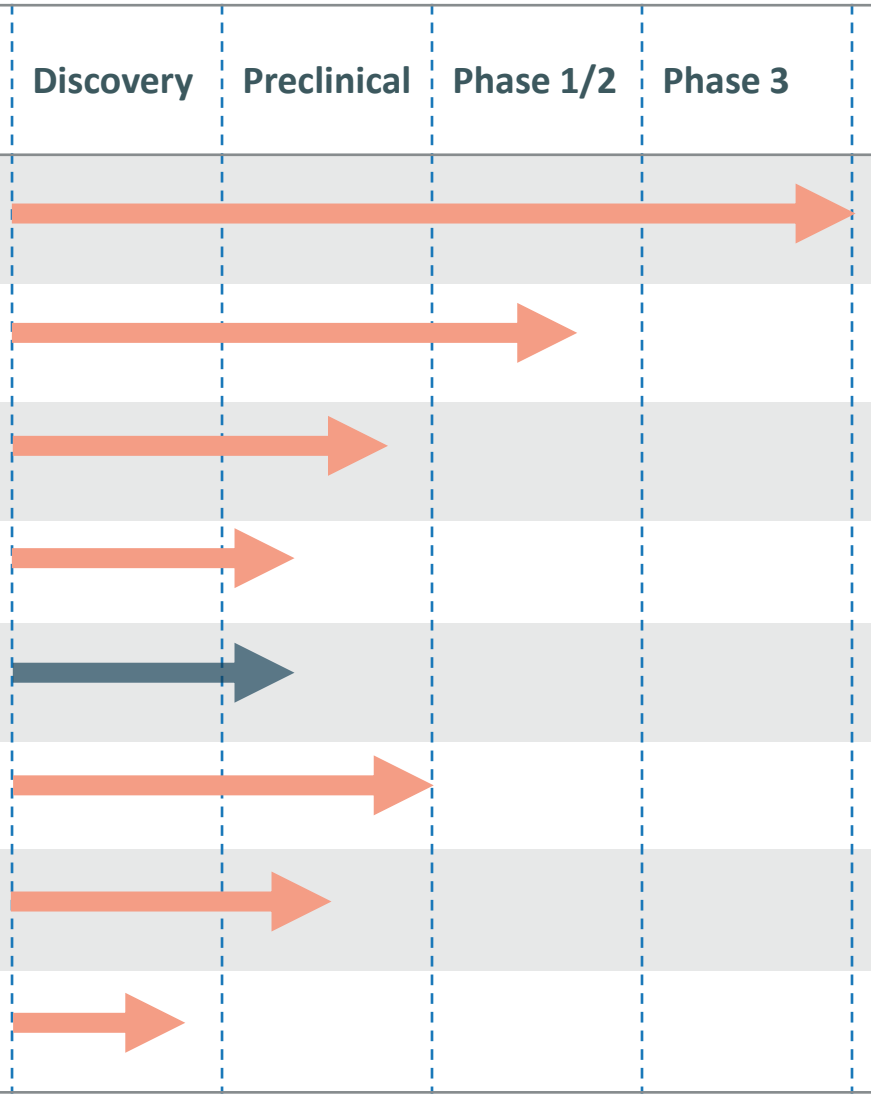
	LV	AAV	HSV-1	LNP
In vivo dosing	No	Yes	**Yes**	Yes
Baseline antibody exclusion criteria	No (if *ex vivo*)	Yes	**No**	No
Repeat-dose capabilities	Yes (if *ex vivo*)	No	**Yes**	Yes
Carrying capacity	8 kb[1]	<4 kb[1]	**>30 kb**	~12 kb[2]
Integrates payload into host cell DNA	Yes	No	**No**	No
Regulatory 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, 2020, https://www.sec.gov/Archives/edgar/data/1733294/000119312520167812/d924849d424b4.htm

LV = lentivirus
AAV = adeno-associated virus
HSV-1 = Herpes Simplex Virus type 1
LNP = lipid nanoparticle

Therapeutic pipeline

	Product	Protein	Indication	Key Upcoming Milestone	Ownership
Dermatology	**VYJUVEK™** †¤•Δ‡§	Type VII collagen (COL7)	Dystrophic EB	File BLA in 1H22; MAA in 2H22	Wholly owned
Dermatology	**KB105**†¤•‡	Transglutaminase 1 (TGM1)	TGM1-deficient ARCI	Initiate next Phase 2 cohort in 2022	Wholly owned
Dermatology	**KB104**¤	Serine Peptidase Inhibitor Kazal Type 5 (SPINK5)	Netherton Syndrome	File IND in 2022	Wholly owned
Dermatology	**KB1XX**	Undisclosed programs			Wholly owned
Dermatology	**KB5XX**	Vectorized antibodies	Chronic conditions		Wholly owned
Respiratory	**KB407**†¤‡	Cystic fibrosis transmembrane conductance regulator (CFTR)	Cystic fibrosis	Initiating Phase 1 study in 1H22	Wholly owned
Respiratory	**KB408**	Alpha-1 antitrypsin (AAT)	Alpha-1 antitrypsin deficiency		Wholly owned
Respiratory	**KB4XX**	Undisclosed programs			Wholly owned



All pipeline compounds are investigational, being evaluated in clinical or pre-clinical studies.

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

Aesthetics pipeline

Program	Indication	Gene	Discovery	IND Enabling	Clinical Development	Next Milestone
KB301	Skin quality (face)	type III collagen (COL3)	→	→	→	Phase I efficacy data in 1Q22
KB302	TBA	type I collagen (COL1)	→			
KB303	TBA	elastin (ELN)	→	→		
KB304	TBA	COL3 + ELN	→			

All pipeline compounds are investigational, being evaluated in clinical or pre-clinical studies.

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

Upstream process using stable producer cell lines has cost and regulatory benefits

- Stable complementary cell lines developed in-house are used in established methods for production of consistent batches
- Eliminates the need for multiple cGMP qualifications of plasmids and variability in transfection efficiency from batch to batch
- Scalable from clinical phase to commercial

We have successfully developed a robust and reproducible downstream process

- Work conducted in an aseptic closed system process
- The same process is leveraged across pipeline with minimal redevelopment effort between product candidates
- Compliant with global regulatory requirements

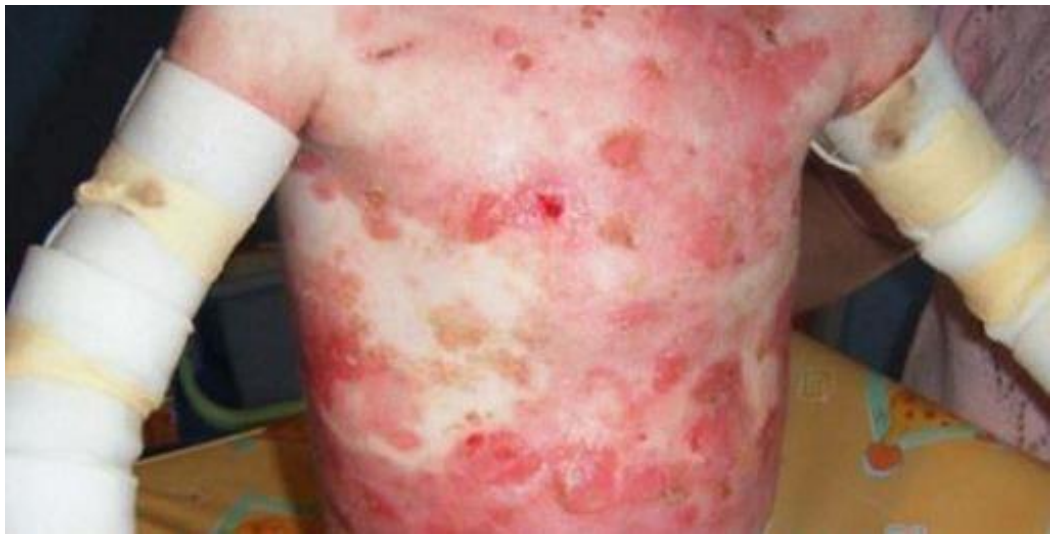
Initial focus on rare skin diseases led to rapid clinical POC and pipeline

Dystrophic epidermolysis bullosa (DEB)

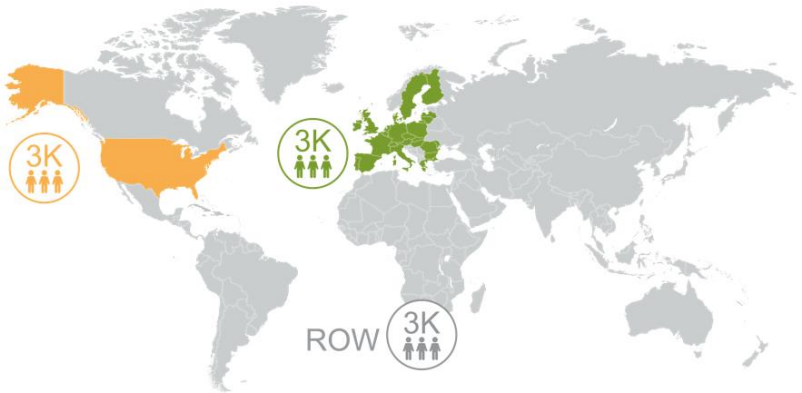
"Butterfly Children" is often used to describe young DEB patients because their skin is as fragile as a butterfly's wings

Dystrophic Epidermolysis Bullosa

- A rare, genetic skin 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) has a broader range in severity and is often characterized by blistering on the hands, feet, knees, and elbows



Epidemiology:



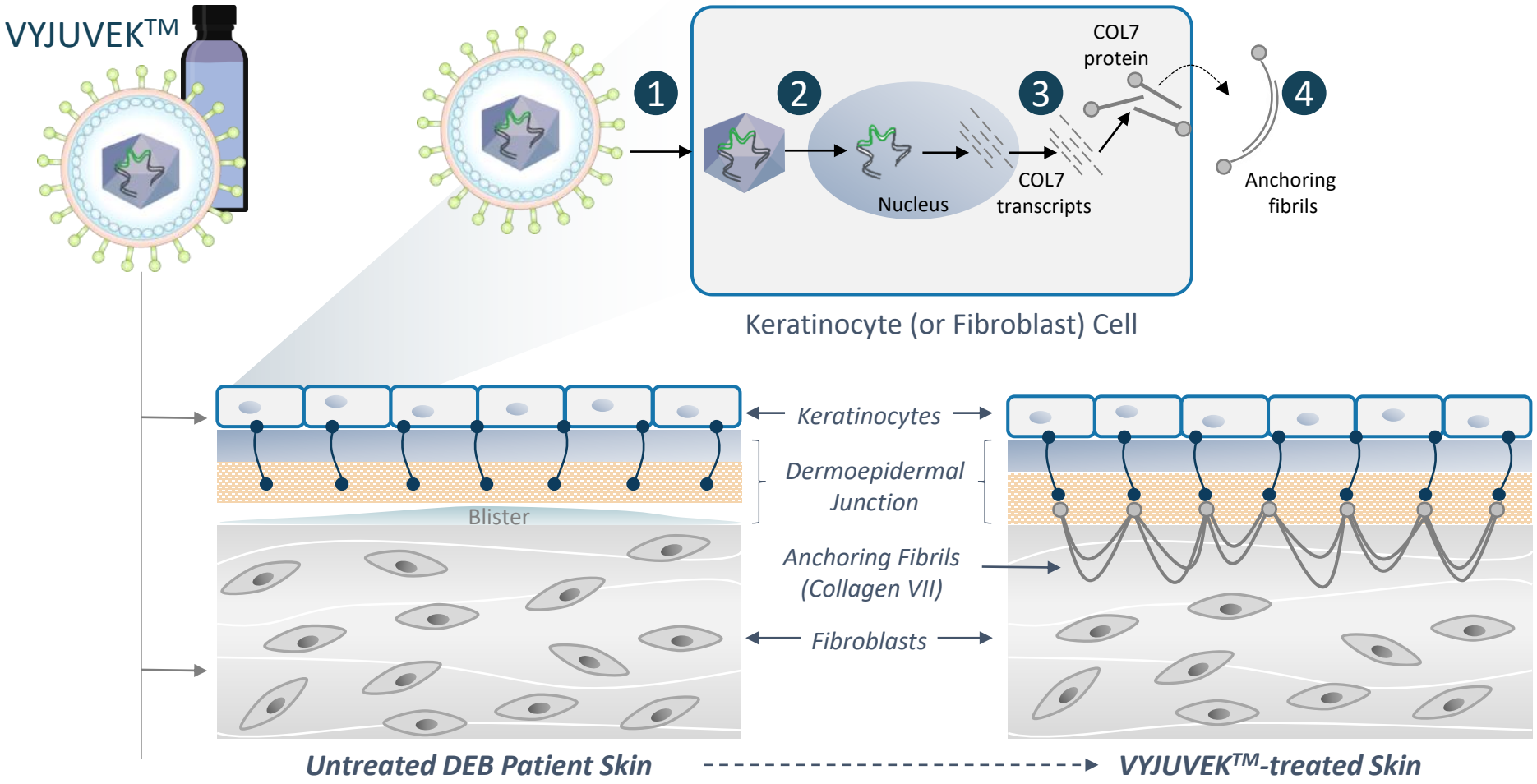
Current Standard of Care

- There are no approved treatments for DEB
- Existing therapies limited to expensive and time-consuming palliative treatments
- Palliative treatments cost $200k – $400k annually[1,2]

1. 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
2. GENEGRAFT Report Summary. (2015, February 16). Retrieved December 13, 2016, from http://cordis.europa.eu/result/rcn/156078_en.html

VYJUVEK™ (beremagene geperpavec) for DEB

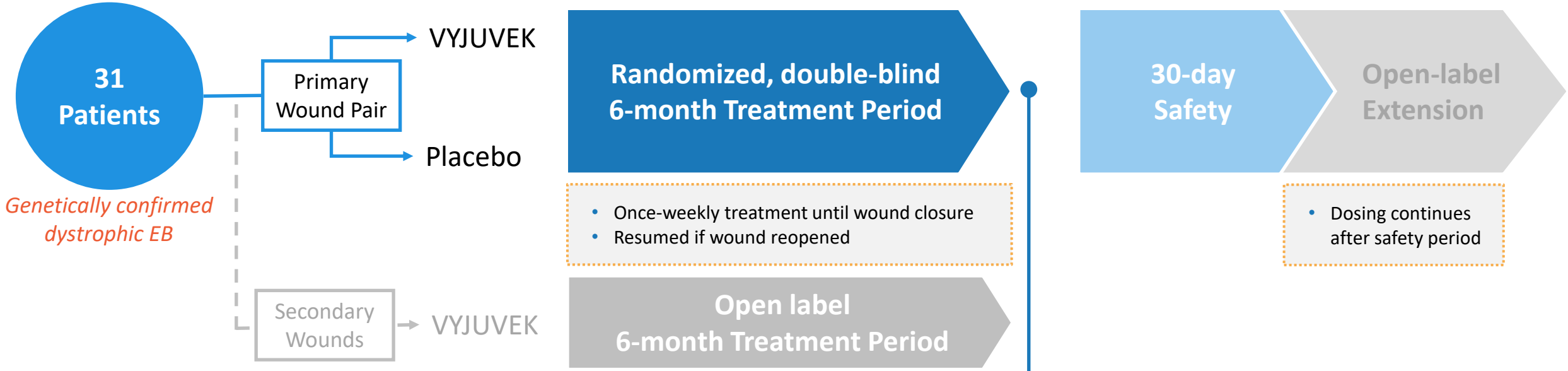
Topically applied VYJUVEK gel is designed to induce local COL7 expression and molecular correction



1. VYJUVEK 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

VYJUVEK is an investigational therapy being studied in clinical trials

GEM-3 evaluated weekly VYJUVEK™ or Placebo in dystrophic EB patients



Primary Efficacy Endpoints

- Complete wound healing at Week 22 and Week 24; or at Week 24 and Week 26 (six-month timepoints)

Secondary Efficacy Endpoints

- Complete wound healing at weeks 8 and 10, or 10 and 12 (three-month timepoints)
- Mean change in pain severity (VAS or FLACC-R Scale) associated with wound dressing changes

Demographics

- 31 patients, each with one primary wound pair were enrolled and included in the intent-to-treat (ITT) analysis
- Enrolled patients ranged from 1 year old to 44 years old at baseline; 61% of the patients enrolled were pediatric (≤18 years old)
- Less than ten percent 10% of enrolled patients had the dominant form of dystrophic epidermolysis bullosa (DDEB)

VYJUVEK is an investigational therapy being studied in clinical trials

Topline Ph3 safety data summary

1 **Topical VYJUVEK™ was well tolerated with a safety profile consistent with prior studies**

2 **No drug-related serious AEs or discontinuations due to treatment were reported**

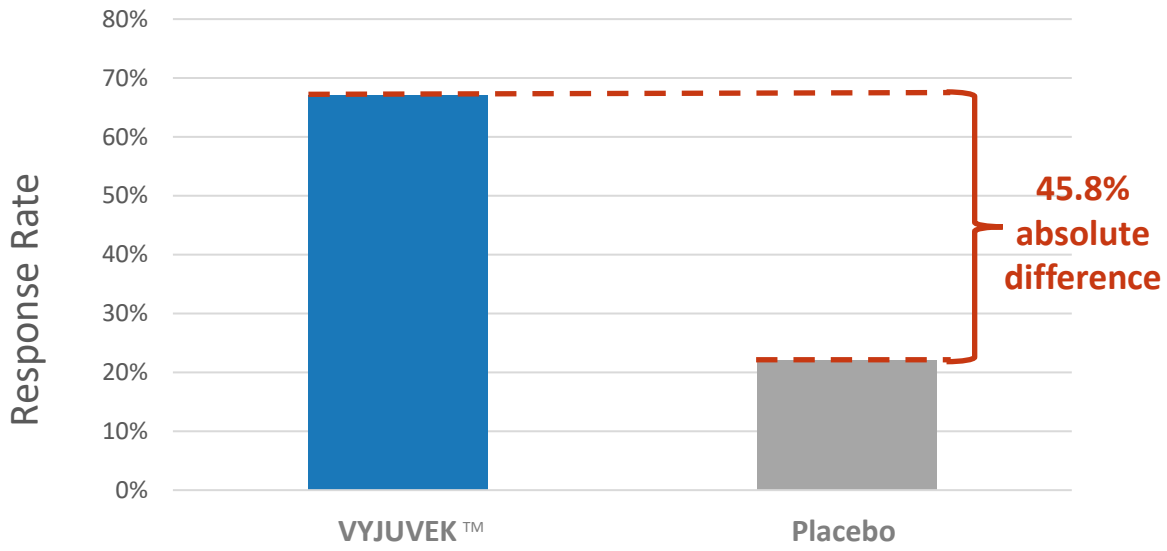
- One mild drug-related AE was reported during the trial

3 **Immunogenicity profile (as measured by anti-HSV-1 and anti-COL7 antibodies) was consistent with prior studies**

VYJUVEK is an investigational therapy being studied in clinical trials

Topline Ph3 efficacy data summary

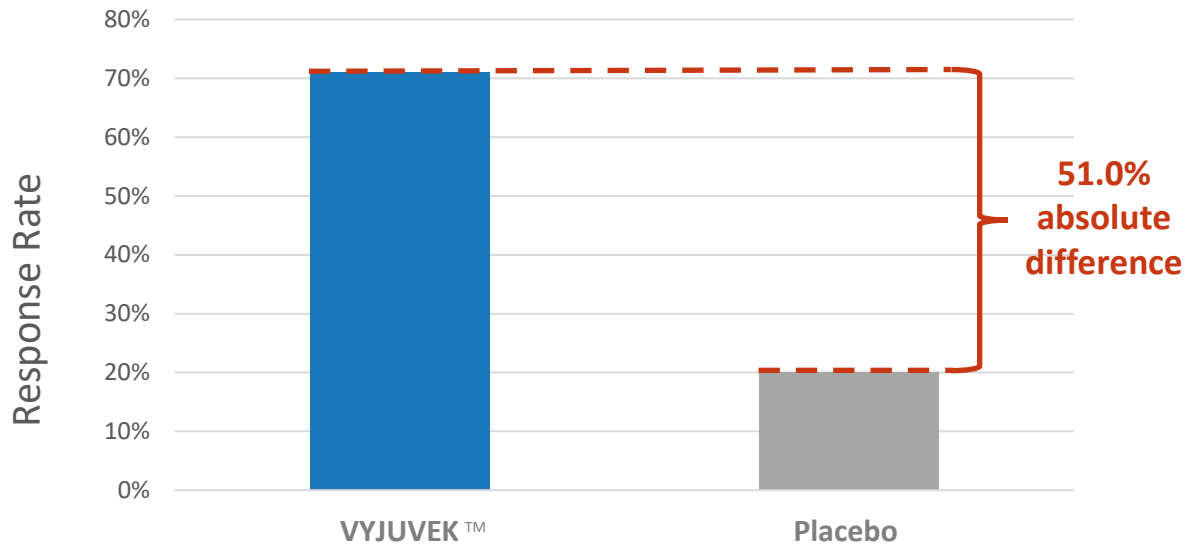
Met **primary endpoint** of complete wound healing at 6-month timepoints (ITT; N=31 primary wound pairs)



Response Rate*	67%	22%
Absolute Difference	45.8%	
95% Confidence Interval	23.6%-68.0%	
p-value**	**<0.005**	

*Response rate numbers rounded to whole values
**Based on McNemar test

Met **secondary endpoint** of complete wound healing at 3-month timepoints (ITT; N=31 primary wound pairs)



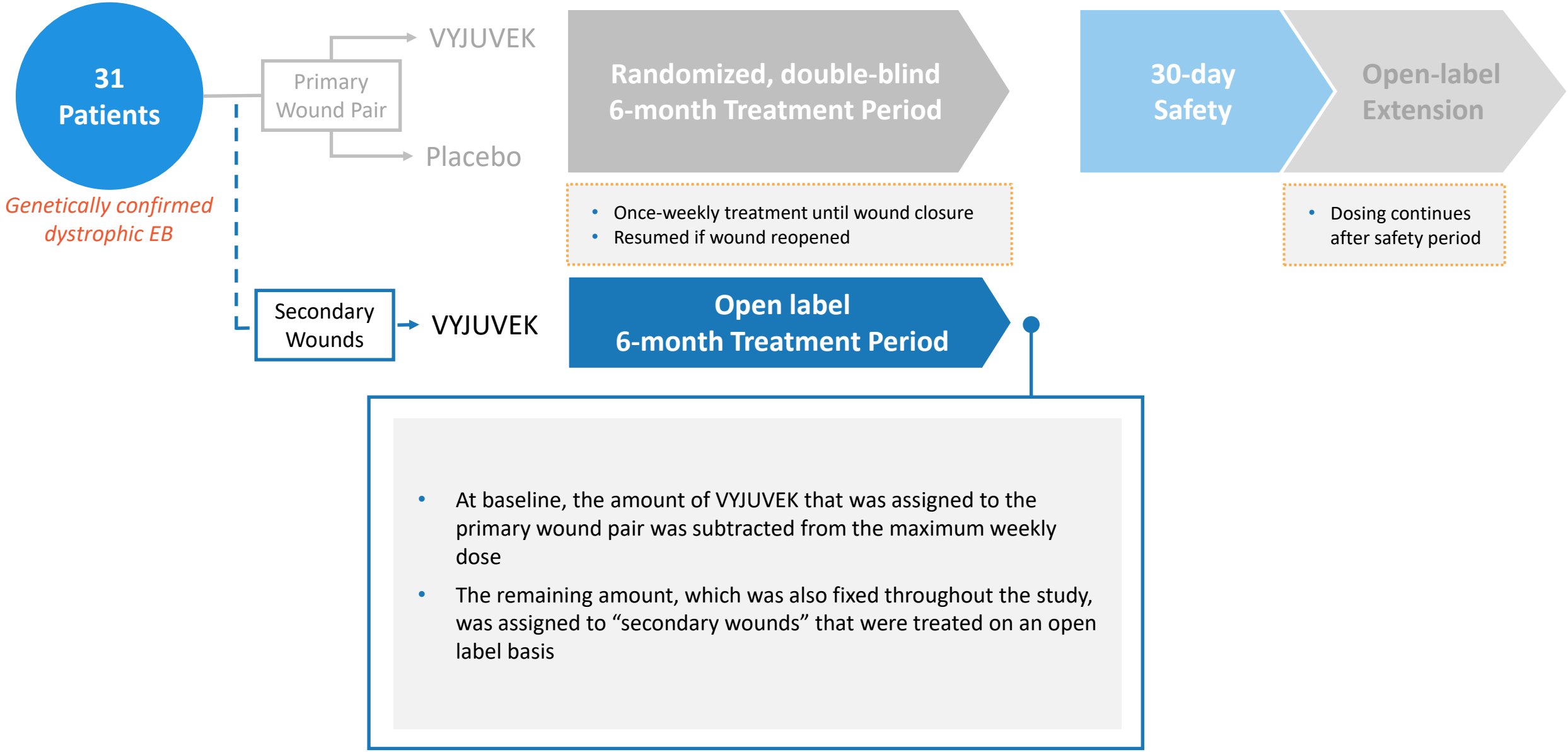
Response Rate*	71%	20%
Absolute Difference	51.0%	
95% Confidence Interval	29.3%-72.6%	
p-value**	**<0.005**	

*Response rate numbers rounded to whole values
**Based on McNemar test

- In an ad-hoc analysis, the trial also demonstrated a statistical difference between the active and placebo groups for wounds that demonstrated complete wound healing at both the three and six-month timepoints (p<0.005)

VYJUVEK is an investigational therapy being studied in clinical trials

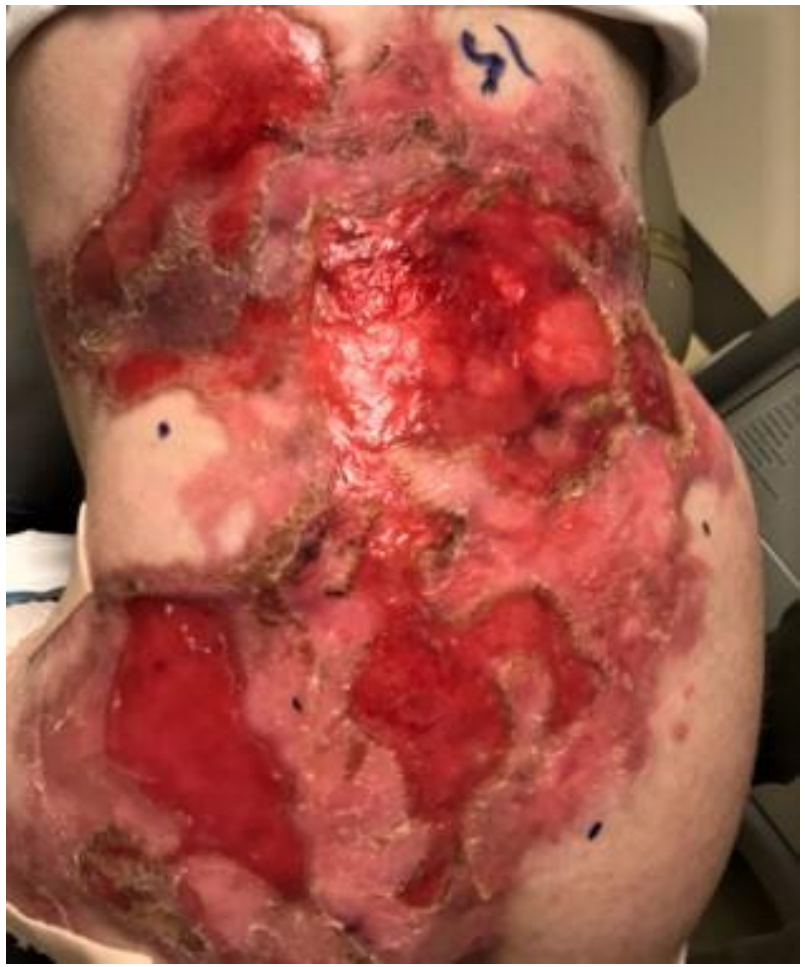
Secondary wounds received open-label VYJUVEK™ throughout the study



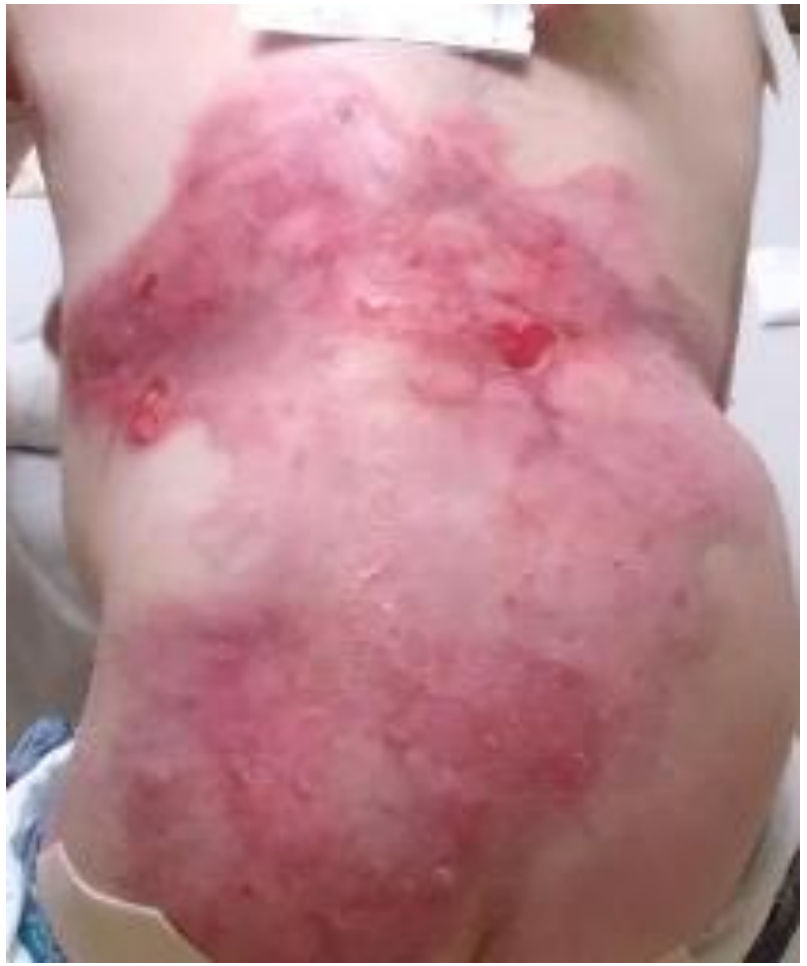
VYJUVEK is an investigational therapy being studied in clinical trials

Secondary wound (Illustrative)

Large, chronic back wound in 21 year old RDEB patient



Baseline

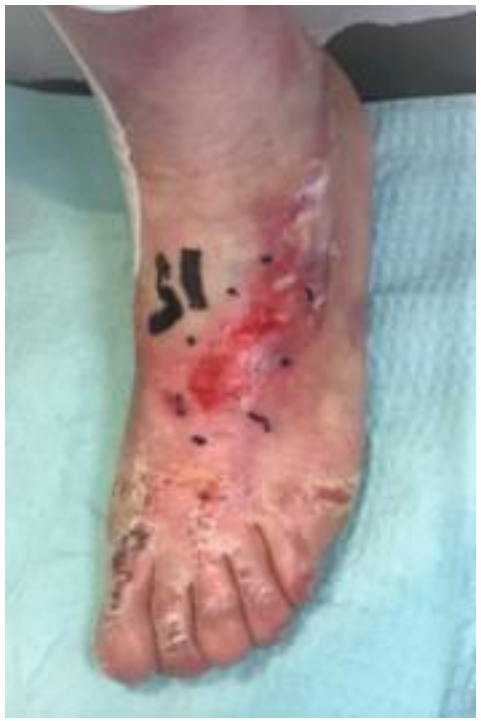


End of Study

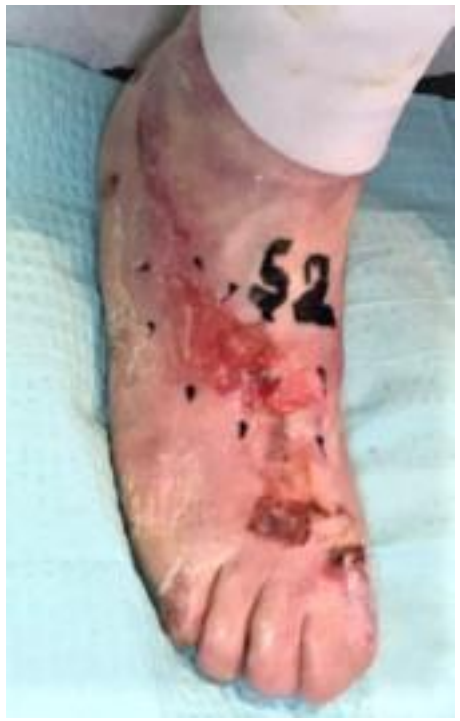
VYJUVEK is an investigational therapy being studied in clinical trials

Secondary wound (Illustrative)

Recurring foot wound in 34 year old RDEB patient

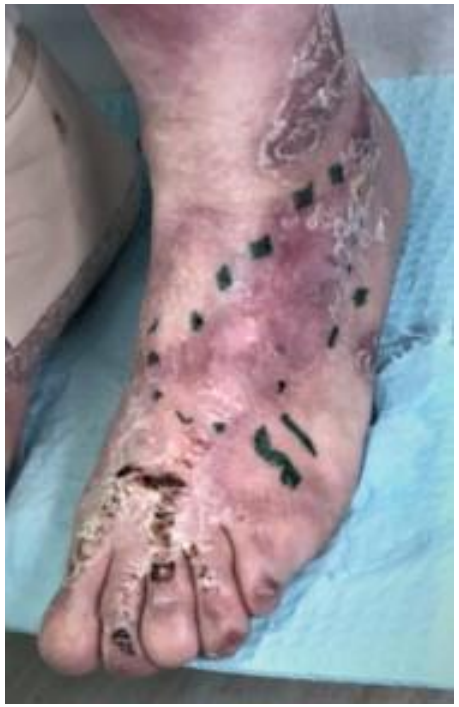


left foot



Right foot

Baseline



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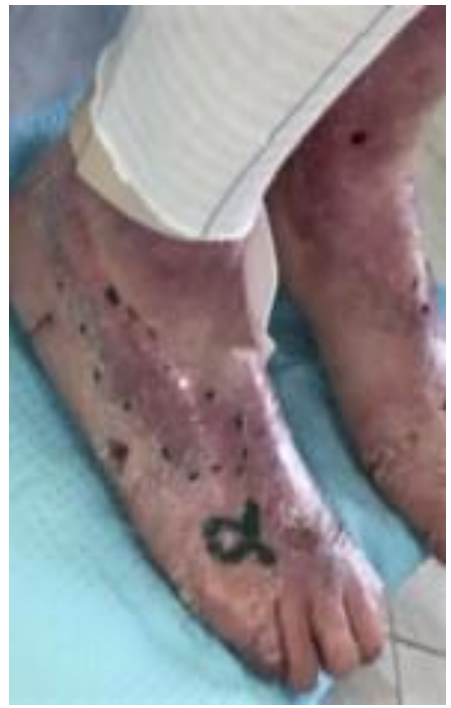
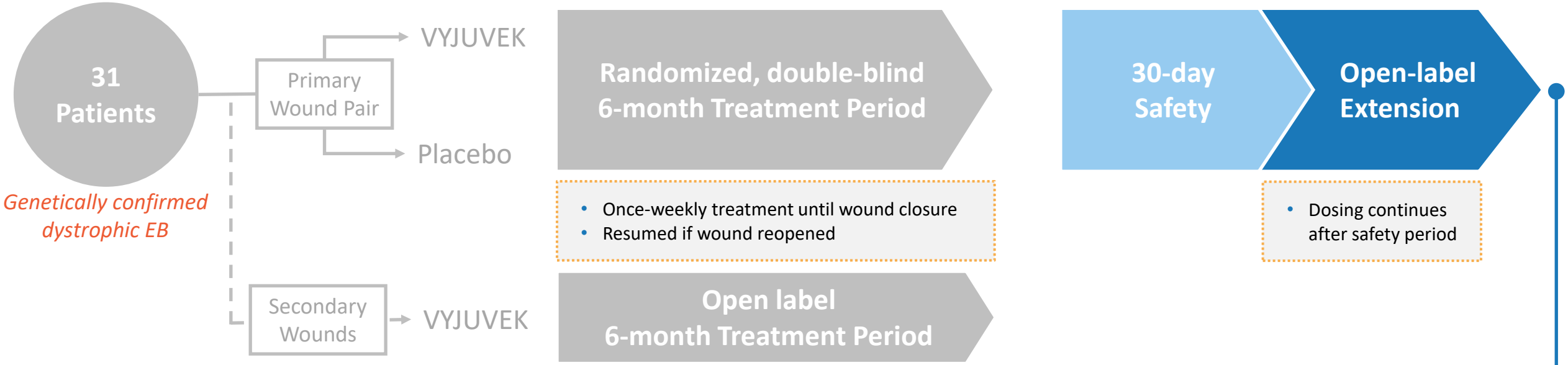


Right foot

End of Study

VYJUVEK is an investigational therapy being studied in clinical trials

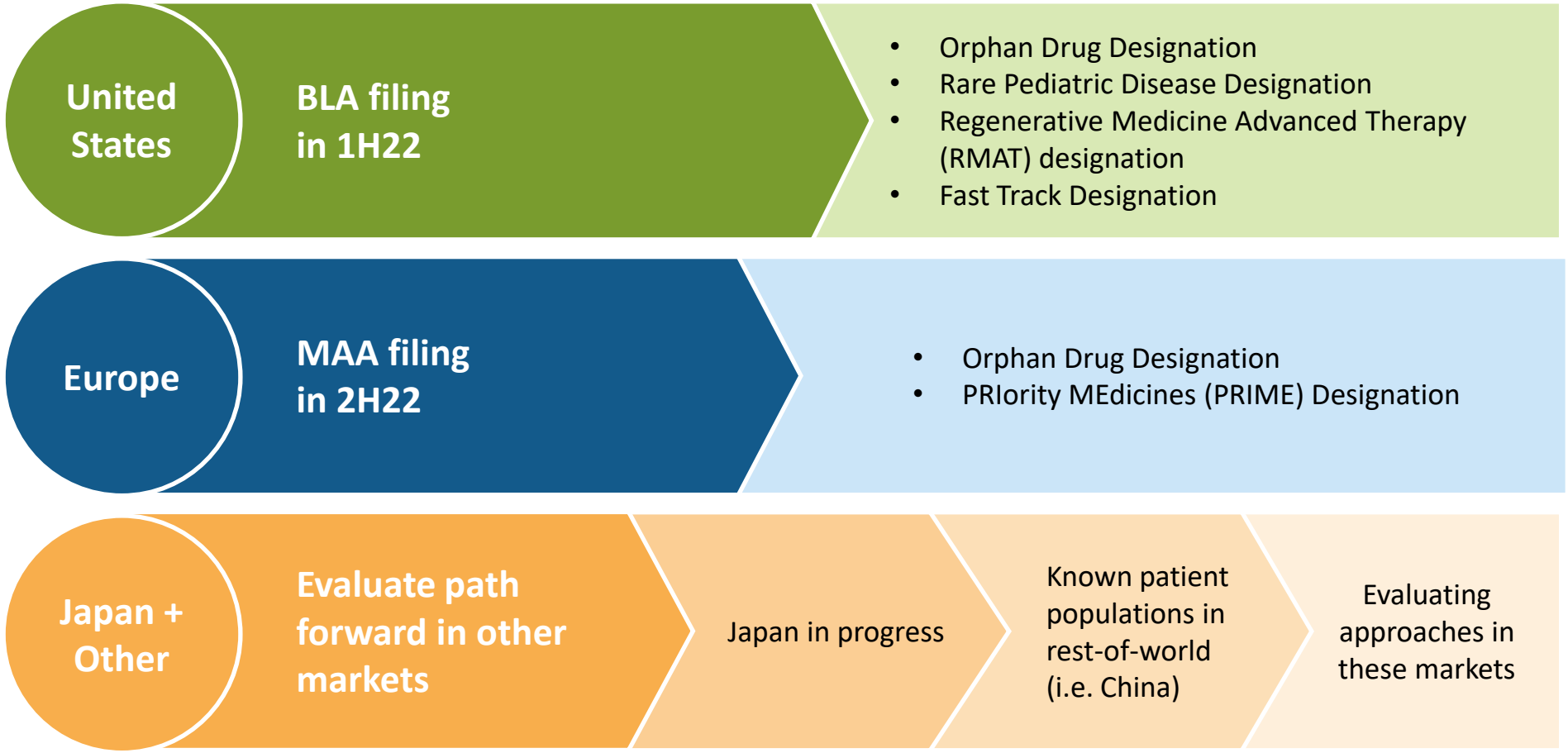
Open-label extension ongoing



- Patients who completed the Phase 3 trial have option to enroll
- Also opening three new sites where new patients who meet Ph3 entry criteria - but did not complete the Phase 3 trial – are eligible to enroll

VYJUVEK is an investigational therapy being studied in clinical trials

VYJUVEK™ regulatory next steps



VYJUVEK is an investigational therapy being studied in clinical trials

Autosomal Recessive Congenital Icthyosis associated with TGM1 mutations

Transglutaminase-1 deficiency is associated with increased mortality in the neonatal period and has a dramatic impact on quality of life

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

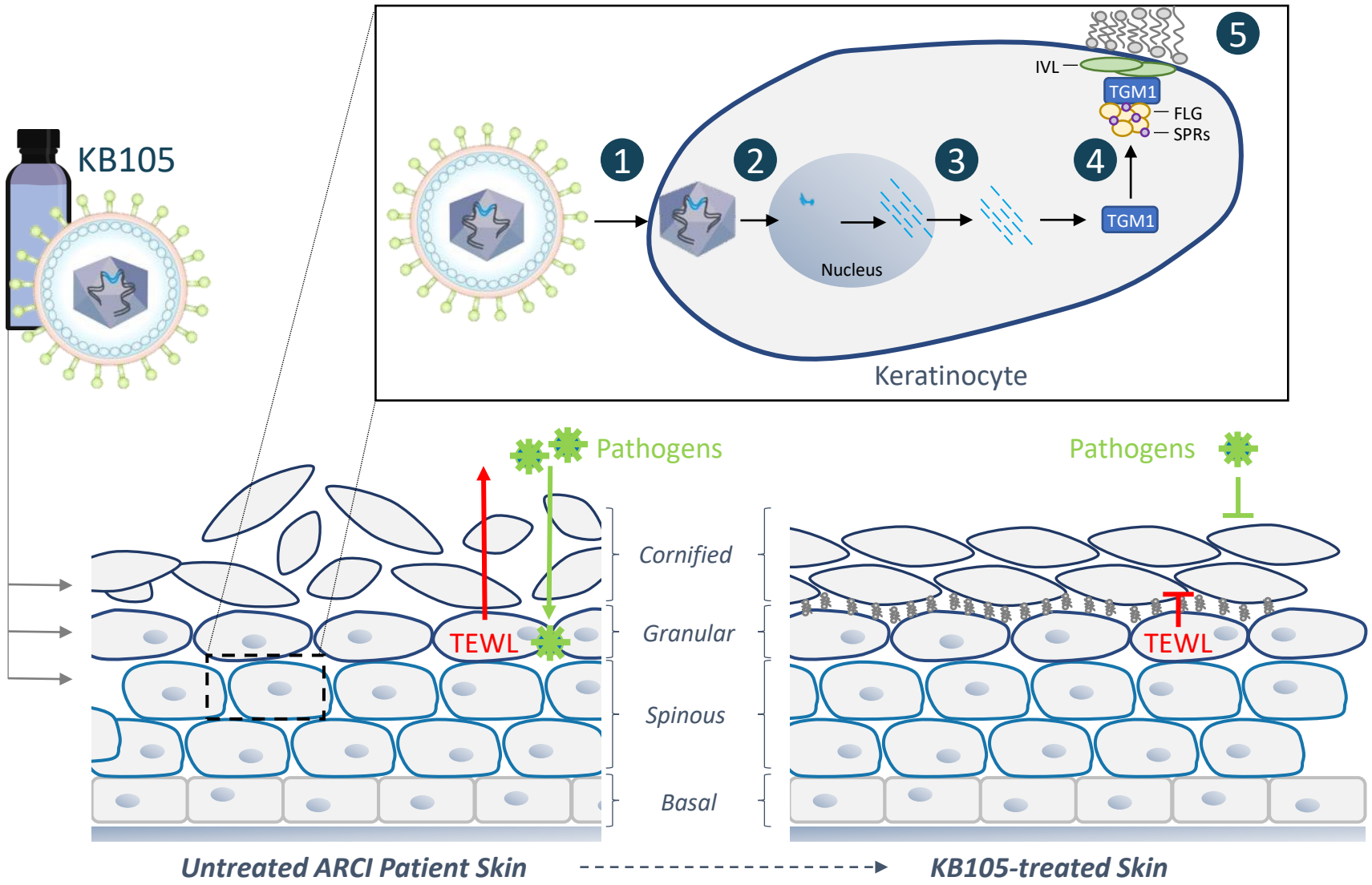
- There are no approved treatments for ARCI associated with TGM1
- Topical and systemic retinoids and time-consuming supportive treatments (up to 4 hours a day of skin care) are most often used

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

Topically applied KB105 delivers multiple copies of the human transglutaminase 1 ("TGM1") gene

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 an investigational therapy being studied in clinical trials

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 four to six months
- *Study PI: Dr. Amy Paller (Northwestern University)*

Enrollment

- 4 TGM1-ARCI subjects were enrolled across 2 sites; three Ph1 patients were enrolled at Paddington Testing Company (Philadelphia); one Ph2 subject was enrolled at Northwestern (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 days
- In Ph1, topical and microneedle administration was evaluated; in Ph2 topical administration will be utilized
- In the Phase 2 portion (n=1) four ~100cm^2 treatment areas were administered KB105, either 4x10^9 PFU or 1x10^{10} at either a high or low dosing frequency

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 (Ph1)
- Improvement of disease severity in the treatment area assessment through Investigator's Global Assessment (IGA)

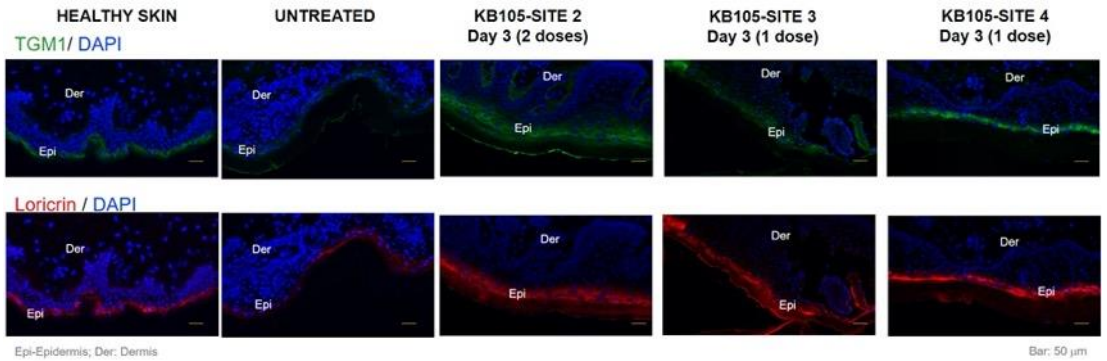
KB105 is an investigational therapy being studied in clinical trials

Phase 1/2 data shows repeat dosing of KB105 to be well tolerated; 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
- Biopsies assessed in the Phase 1 portion of the study show:
  - 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

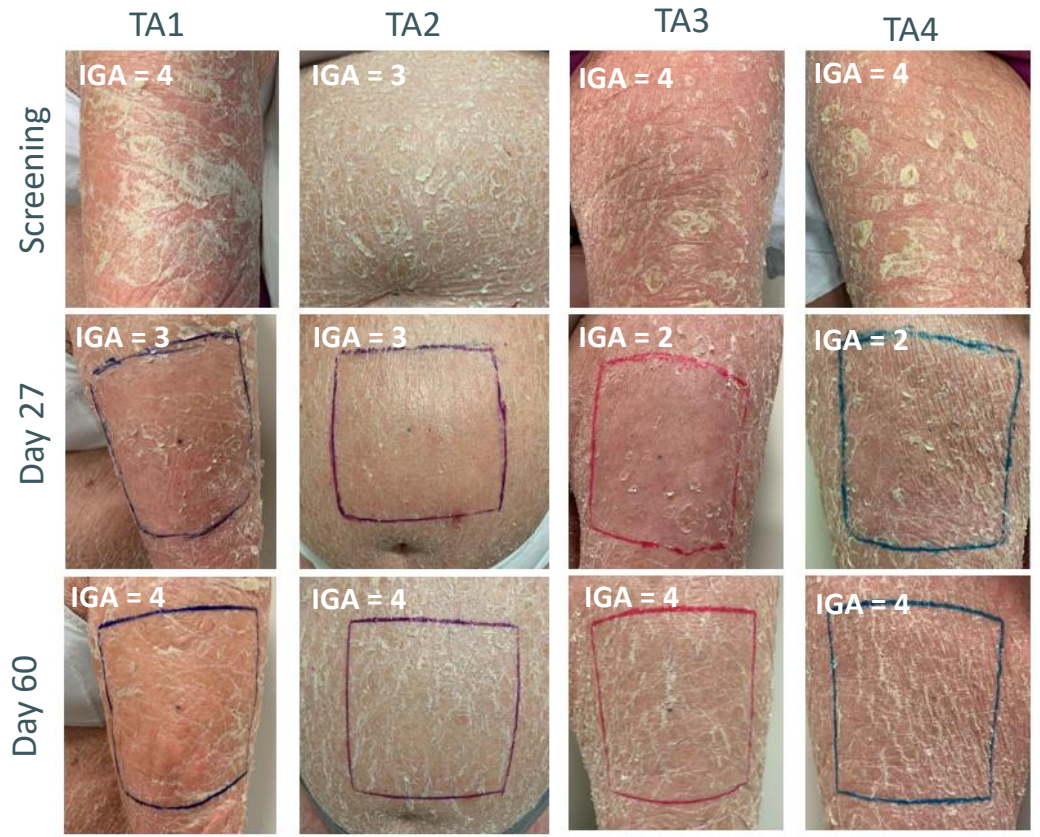
Subject 1: Treatment Restored TGM1 Expression to Normal Levels



- TGM-1 turnover was observed to be variable and rapid, and pharmacokinetic data suggested that the optimal dosing frequency may be more frequent

Maximum 2-point Improvement in IGA Scale as Compared to Screening Observed at Day 27 at High Dose

- In the Phase 2 portion (n=1) four ~100cm2 treatment areas (TAs) were administered KB105, either $4x10^9$ PFU or $1x10^{10}$ at either a high or low dosing frequency

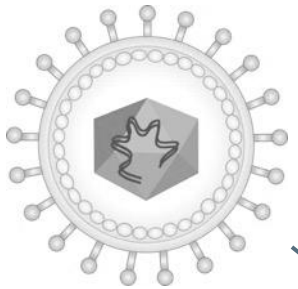


KB105 is an investigational therapy being studied in clinical trials

Leveraging platform to target new tissues and larger indications

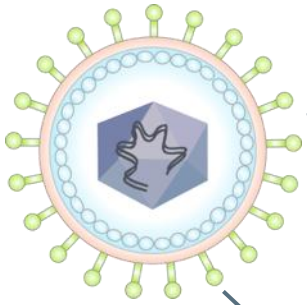
HSV-1 has potential beyond rare skin diseases

Wild-type HSV-1



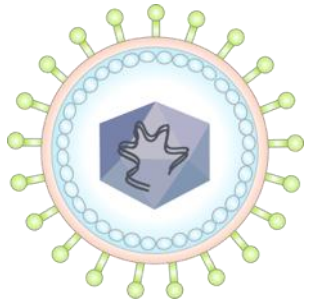
HSV-1 has natural affinity for **epithelial cells,** in addition to several other cell types, with favorable immune-evasion mechanisms

Krystal's Current Platform



- Skin
  - Rare Skin Disease
    - ✓ VYJUVEK (COL7) for DEB
    - ✓ KB105 (TGM1) for TGM1-ARCI
    - • KB104 (SPINK5) for Netherton's Disease
    - • Additional preclinical candidates
  - Aesthetics (Jeune Aesthetics)
    - ✓ 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
    - • KB408 (AAT) for alpha-1 antitrypsin deficiency
    - • Additional preclinical candidates
  - Different Diseases
  - Different Effectors (e.g. antibodies, RNAi)

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



KB407 for cystic fibrosis

Gene therapy approaches have been tried and failed in their attempts to replace CFTR protein

- 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

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

- ✓ Replication incompetent HSV-1
- ✓ Delivers two copies of full length, human CFTR protein (mutation agnostic approach)
- ✓ Duration of nebulization expected to be under 30 minutes, using a commercially available nebulizer
- ✓ Episomal delivery of CFTR gene does not disrupt cell DNA
- ✓ Ability to re-dose and/or adjust dose over time as lung cells turnover

Our vector can be formulated and delivered via nebulizer with no significant change in activity

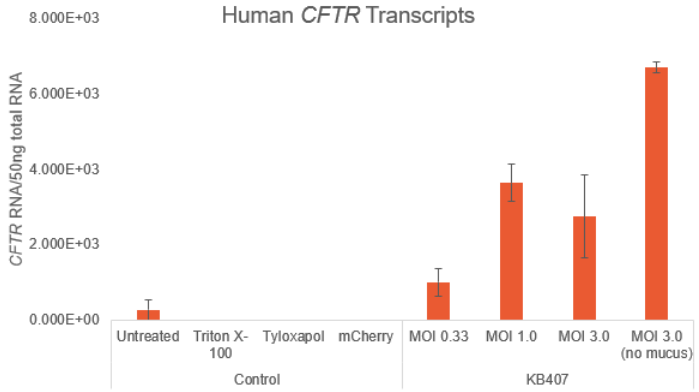
- In vitro data shows KB407 can be nebulized, successfully transduce target lung cells and induce expression of fully functional and properly localized CFTR

KB407 is an investigational therapy being studied in preclinical trials

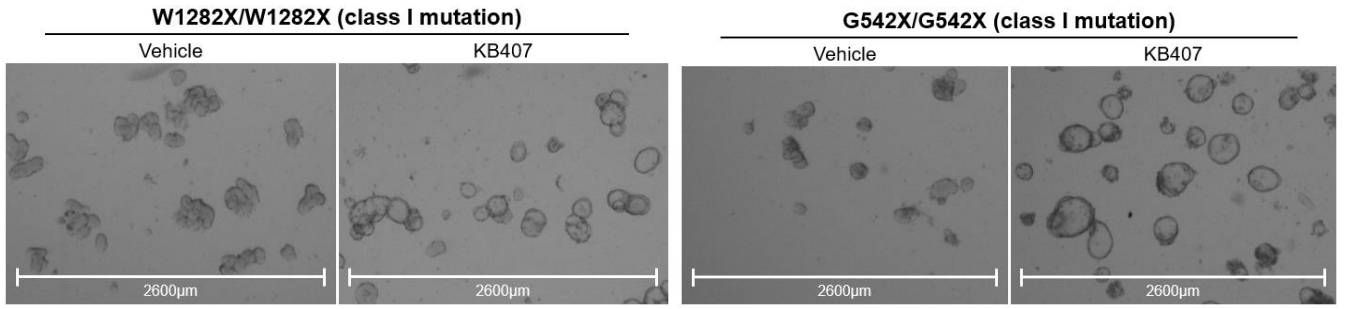
In vitro and in vivo data supportive of initial clinical testing

In Vitro
CF-patient derived cell culture)

KB407 transduction and CFTR expression is similar with or without presence of mucus in ALI culture

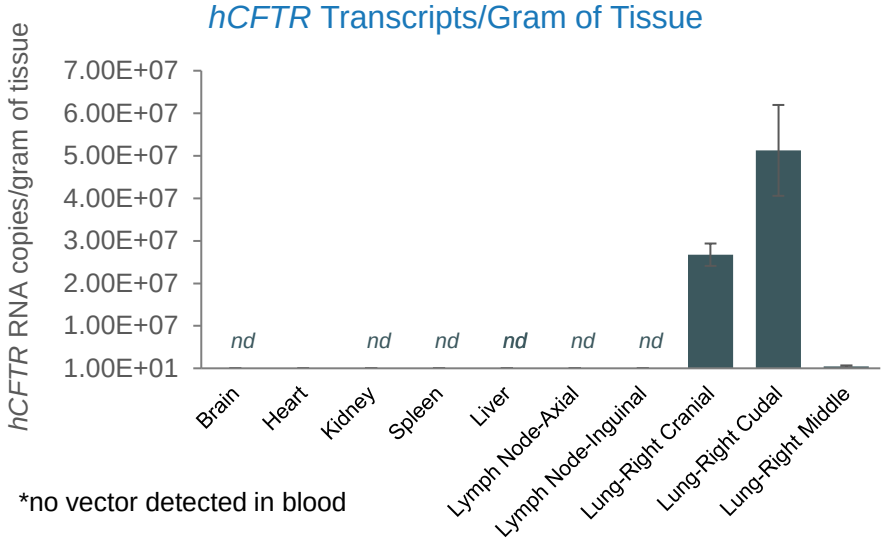


Treatment with KB407 rescued CF-patient derived organoids, restored to wild-type phenotype



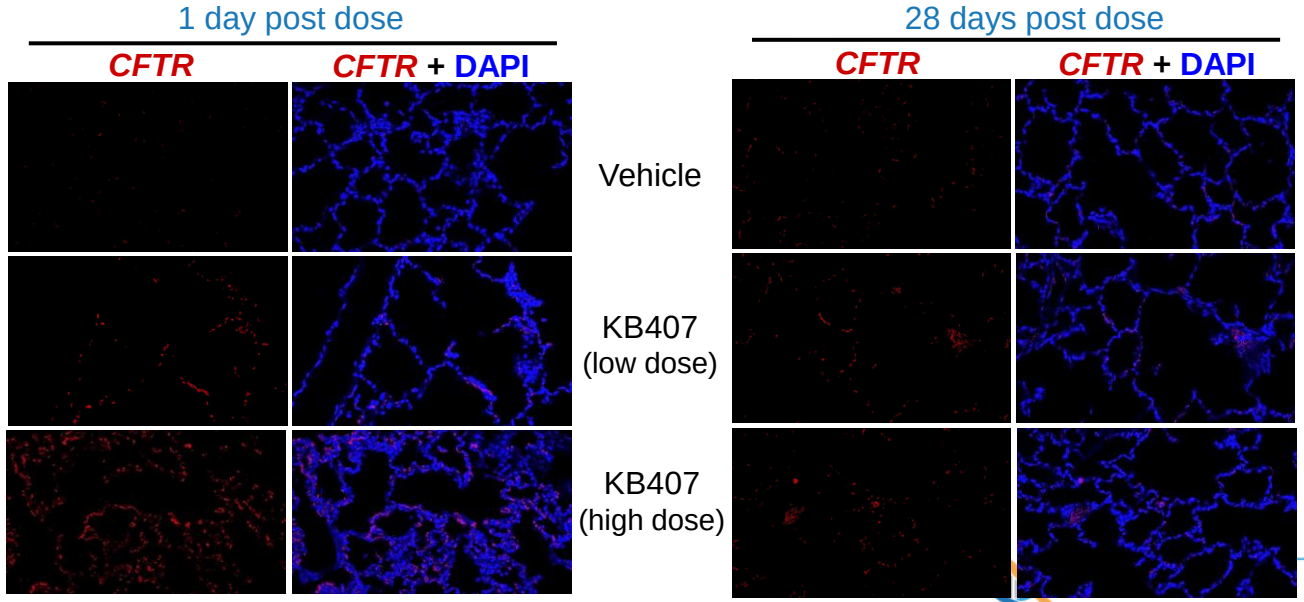
In Vivo
nonhuman primates

Nebulized KB407 was distributed throughout the lung, with no evidence of systemic distribution



*no vector detected in blood

Dose dependent *CFTR* expression via FISH was observed and durable for at least 28 days



KB301 for aesthetic indications

KB301 and other discovery programs in Aesthetics, are housed in our wholly owned subsidiary, Jeune Aesthetics, Inc.

KB301 aims to increase neocollagenesis, thereby correcting the underlying molecular defect of the aged phenotype

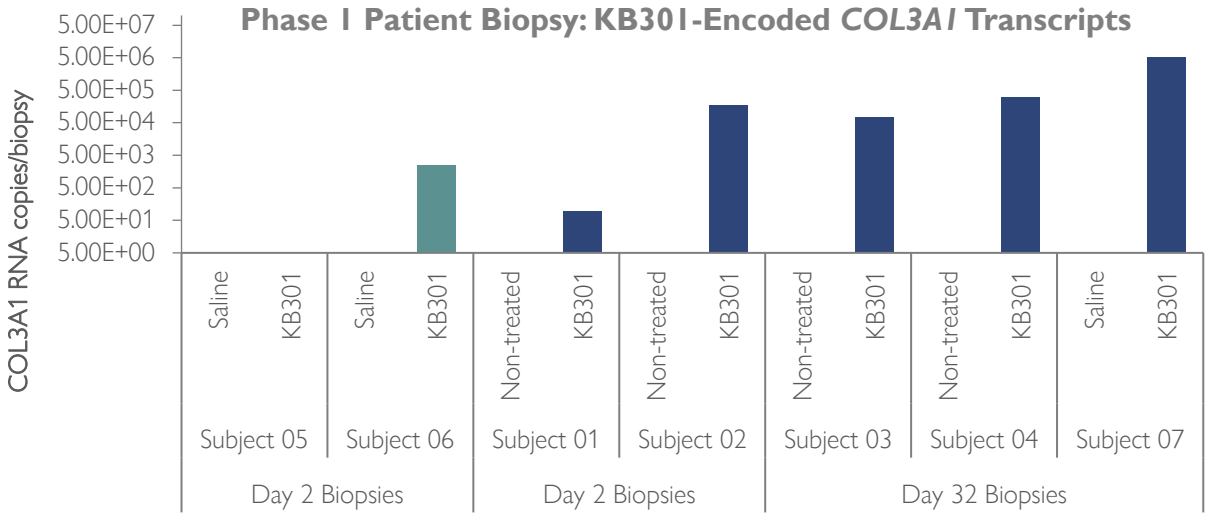
- Dermal collagen, composed primarily of types 1 and 3 collagen fibrils, represents >90% (dry weight) of human skin
- Declining levels of collagen are caused by reduced collagen biosynthesis and increased collagen fibril fragmentation resulting from both intrinsic and extrinsic pressures
- KB301 is designed to deliver the gene for full-length type III collagen (COL3), to enable increased endogenous COL3 production

KB301 is currently being evaluated in a Phase 1 trial (NCT04540900)

- The open label, dose ranging study will evaluate repeat dosing of KB301 injections
- Safety data from Cohort 1 (repeat KB301 injections into the buttock region) was reported at SID 2021
- Cohort 2 is currently enrolling and will evaluate efficacy of repeat doses of KB301 on the face and knees
  - Change in skin quality from baseline will be assessed via the Skin Roughness Score (SRS)[1], Fine Lines Score (FLS)[2], and the Subject Satisfaction Score (SSS). Skin calipers will be used to measure the change in skin thickness over the knee

Initial data from Cohort 1 of the Phase 1 study shows safety and tolerability of repeat KB301 injections

- Repeated intradermal injections of KB301 were well tolerated.
- Recorded adverse events were transient and limited to expected mild or moderate injection site or biopsy site reactions (e.g. erythema, site pain, purpura, ecchymosis)
- For all subjects who have completed follow up through day 90 (subjects 1-6; subject 7 follow up ongoing) no clinically significant changes in anti-drug antibodies were observed



- KB301-encoded COL3A1 expression measurable at the mid and high dose, with no detectable expression in control samples
- Expression was evident by day 2 following the first dose; expression levels were similar following the first and second dose

1. *Donofrio L, Carruthers A, Hardas B, et al. Development and validation of a photonumeric scale for evaluation of facial skin texture. Dermatol Surg. 2016;42(suppl 1):S219–S226.*
2. *Carruthers J, Donofrio L, Hardas B et al. Development and validation of a photonumeric scale for evaluation of facial fine lines. Dermatol Surg. 2016;42:S227–S234. 101*

KB301 is an investigational therapy being studied in clinical trials

Financials and Milestones

Krystal summary

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

Current Status and Milestones

Rare Skin

- **VYJUVEK™:** BLA filing in US in 1H22, MAA filing in EU in 2H22
- **KB105:** Phase 2 study ongoing; Initiation of dosing in next Phase 2 cohort in 2022
- **KB104:** IND filing in 2022

Jeune Aesthetics, Inc

- **KB301:** Phase 1 trial in aesthetic skin indications ongoing; initial Phase 1 efficacy data anticipated in 1Q22

Rare Lung

- **KB407:** Phase 1 trial being initiated in Australia in 1H22; US IND filing anticipated in 2H22

Platform

- **Manufacturing:** Ancoris facility currently supplying all clinical material and will supply initial phase of VYJUVEK launch; Astra facility (150,000 sqft) construction underway, completion anticipated in 2022
- **Next Gen Tech:** Evaluation of novel effectors, routes of administration, and tissue tropism underway

September 30, 2021 cash balance of $362.3M

- Completed secondary offering in December 2021 with proceeds net of underwriting fees of $202M, which includes net proceeds from the underwriter's option to purchase additional shares of $14M
- VYJUVEK, KB105, KB104 and KB407 are PRV eligible

Krystal

The Leader in **Redosable Gene Therapy** for Rare Disease

January 2022