UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 8-K

CURRENT REPORT
Pursuant to Section 13 or 15(d)
of the Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): November 29, 2021

KRYSTAL BIOTECH, INC.
(Exact name of registrant as specified in its charter)

Delaware	**001-38210**	**82-1080209**
(State or other jurisdiction of incorporation)	**(Commission File Number)**	**(IRS Employer Identification Number)**

2100 Wharton Street, Suite 701
Pittsburgh, Pennsylvania 15203
(Address of principal executive offices, including Zip Code)

Registrant's telephone number, including area code: (412) 586-5830

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock	**KRYS**	**Nasdaq Capital Market**

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company ☒

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☒

Item 7.01 Regulation FD Disclosure.

On November 29, 2021, Krystal Biotech, Inc. (the "Company") issued a press release announcing positive topline results of the Company's Phase 3 randomized, double-blind, intra-patient placebo-controlled study designed to evaluate the efficacy and safety of VYJUVEK™ for the treatment of dystrophic epidermolysis bullosa. In addition, the press release indicated that the Company would host an investor conference call at 8:00 a.m. ET on November 29, 2021 to discuss topline results from the pivotal GEM-3 trial and the VYJUVEK™ program. For purposes of the call, the Company provided an investor slide presentation (the "Investor Slide Presentation"), which is available on the "Investors" section of the Company's website at www.krystalbio.com. Copies of the press release and the Investor Slide Presentation are attached hereto as Exhibit 99.1 and Exhibit 99.2, respectively, and are incorporated by reference herein.

This information in this Item 7.01 of this Current Report on Form 8-K shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, or otherwise subject to the liabilities of that Section, or incorporated by reference in any filing.

8.01 Other Events.

The results described under Item 7.01 above summarize data from 31 enrolled patients in a randomized, double-blind, placebo-controlled study designed to evaluate the efficacy and safety of VYJUVEK™ for the treatment of dystrophic Epidermolysis Bullosa.

The primary endpoint of the trial evaluated complete wound healing and was met with statistical significance compared to placebo, as well as complete wound healing at the secondary endpoint.

The trial produced the following updates:

- 67% of wounds treated with VYJUVEK™ achieved the primary endpoint of investigator assessed complete wound healing at the six-month timepoints as compared to 22% of wounds treated with placebo (absolute difference (95% CI): 45.8% (23.6%-68.0%); p<0.005).
- 71% of wounds treated with VYJUVEK™ achieved the secondary endpoint of investigator assessed complete wound healing at the three-month timepoints as compared to 20% of wounds treated with placebo (absolute difference (95% CI): 51.0% (29.3%-72.6%); p<0.005).
- 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™ was well tolerated. No drug-related serious adverse events or discontinuations due to treatment were reported. One mild drug-related AE was reported during the trial.
- The study also examined the treatment of secondary wounds with VYJUVEK™, in which we saw impressive closure of wounds treated with VYJUVEK™.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits.

Exhibit No.	Description
99.1	Press Release, dated November 29, 2021
99.2	Investor Slide Presentation, dated November 29, 2021
104	Cover page Interactive data file (embedded with in the inline XBRL document)

Forward-Looking Statements

Any statements in this current report about future expectations, plans and prospects for Krystal Biotech, Inc., including but not limited to statements about the development of Krystal's product candidates, such as plans for the design, conduct and timelines of clinical trials of VYJUVEK™; the clinical utility of VYJUVEK™, and Krystal's plans for filing of regulatory approvals and efforts to bring VYJUVEK™ to market; plans to pursue research and development of other product candidates;; 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 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 trials 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 including VYJUVEK™, the sufficiency of cash resources and need for additional financing and such other important factors as are set forth under the caption "Risk Factors" in Krystal's annual and

quarterly reports on file with the U.S. Securities and Exchange Commission. In addition, the forward-looking statements included in this current report represent Krystal's views as of the date of this current report. Krystal anticipates that subsequent events and developments will cause its views to change. However, while Krystal 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 Krystal's views as of any date subsequent to the date of this current report.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, as amended, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

Date: November 29, 2021

KRYSTAL BIOTECH, INC.

By: /s/ Krish S. Krishnan
Name: Krish S. Krishnan
Title: Chairman and Chief Executive Officer

Exhibit 99.1

Krystal Biotech Announces Positive Topline Results from GEM-3 Pivotal Trial of VYJUVEK™ in Patients with Dystrophic Epidermolysis Bullosa

- Pivotal GEM-3 trial met its primary endpoint of complete wound healing at six-month timepoints, and its secondary endpoint of complete wound healing at three-month timepoints

- VYJUVEK™ was well tolerated, with no drug-related serious adverse events or discontinuations

- Biologics License Application (BLA) on track to be submitted to U.S. Food and Drug Administration (FDA) in 1H22

- Conference call to discuss results scheduled for today, Monday November 29, 2021 at 8:00 a.m. EST

PITTSBURGH, November 29, 2021 – Krystal Biotech, Inc., ("Krystal") (NASDAQ: KRYS), the leader in redosable gene therapies for rare diseases, today announced positive topline results from the pivotal GEM-3 trial of investigational beremagene geperpavec (B-VEC), now known as VYJUVEK™, for the treatment of dystrophic Epidermolysis Bullosa (dystrophic EB).

The primary endpoint of the trial evaluated complete wound healing of topical VYJUVEK™ compared to placebo at six-month timepoints and met statistical significance. VYJUVEK™ is the first non-invasive, topical and redosable gene therapy in development, and the only genetically corrective approach to treat dystrophic EB that has successfully completed a double blinded Phase 3 trial.

Highlights of Topline Results from the GEM-3 Trial

- 31 patients (31 primary matched-wound pairs) were enrolled and evaluable for safety and efficacy per the primary intent-to-treat (ITT) analysis
- 67% of wounds treated with VYJUVEK™ achieved the primary endpoint of investigator assessed complete wound healing at the six-month timepoints as compared to 22% of wounds treated with placebo (absolute difference (95% CI): 45.8% (23.6%-68.0%); p<0.005)
- 71% of wounds treated with VYJUVEK™ achieved the secondary endpoint of investigator assessed complete wound healing at the three-month timepoints as compared to 20% of wounds treated with placebo (absolute difference (95% CI): 51.0% (29.3%-72.6%); p<0.005)

- 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™ was well tolerated. No drug-related serious adverse events or discontinuations due to treatment were reported. One mild drug-related AE was reported during the trial.
- The immunogenicity profile of VYJUVEK™ (as measured by anti-HSV-1 and anti-COL7 antibodies) was consistent with the prior GEM-1/2 study where we observed no meaningful change in anti-HSV-1 or anti-COL7 antibodies

“Dystrophic Epidermolysis Bullosa is referred to as ‘the worst disease you’ve never heard of’ because of the incredibly devastating reality that patients with this genetic condition face, and we are thrilled to announce positive results from our pivotal GEM-3 trial of VYJUVEK™ which showed that this topical gene therapy led to durable wound healing in dystrophic EB wounds,” said Suma Krishnan, Founder and Chief Operating Officer of Krystal. “With these results in hand, we look forward to advancing discussions with regulatory authorities and will work quickly to bring this potential first-ever treatment to patients with dystrophic EB and their families who are in desperate need.”

“Today’s positive B-VEC results represent the culmination of years of study on the molecular basis and genetic correction of this disease. Finally, dystrophic EB patients may have an easily administered genetically targeted therapy which has been shown to promote durable wound healing in this clinical trial. This is a long overdue milestone for patients living with this disease, and one that has potential to drastically change the treatment paradigm.” said Dr. Peter Marinkovich, M.D., Bullous Disease Clinic Director and Associate Professor of Dermatology at Stanford University.

Next Steps

Based on these results, Krystal intends to file a Biologics License Application (BLA) with the U.S. Food and Drug Administration (FDA) in the first half of 2022 as the first step in executing its global regulatory and commercialization strategy to bring this investigational therapy to patients in need. The company expects to submit a Marketing Authorization Application (MAA) in Europe shortly after the BLA. Exploration of the potential regulatory path forward in other geographies, including Japan, is underway. Krystal will continue to manufacture VYJUVEK™ using the commercial scale process at its in-house cGMP manufacturing facility, ANCORIS, which was designed to support potential launch. The Company is currently constructing its 2nd, larger, facility ASTRA which is expected to come on-line in 2022 to help support a potential global launch and the pipeline.

“We founded Krystal less than six years ago with the goal of developing a non-invasive, genetically corrective therapy for dystrophic EB. We offer our deepest gratitude to the patients, caregivers, investigators, and of course to the broader Krystal team who worked tirelessly to help us reach this exciting moment in the progression of the VYJUVEK™ program,” said Krish Krishnan, Chairman and CEO of Krystal. “These pivotal data provide important validation of our redosable gene delivery technology, emboldening us to expand our pipeline to address other genetic skin diseases, continue to explore the potential in genetic lung diseases, and invest in growing the platform capability to address new organ systems as well.”

Investor Conference Call, Webcast and Presentation Information

Krystal will host an investor conference call and webcast today, Monday, November 29, at 8:00 a.m. ET, to discuss topline results from the pivotal GEM-3 trial and the VYJUVEK™ program. To participate in the conference call, please dial 1-877-407-4018 (domestic) or 1-201-689-8471 (international) and refer to conference ID 13725260. The webcast, which will include presentation slides, will be available live and for replay on Krystal's website at www.krystalbio.com in the Investors section.

About the GEM-3 Trial

The GEM-3 trial (NCT04491604) was a randomized, double-blind, intra-patient placebo-controlled study designed to evaluate the efficacy and safety of VYJUVEK™ for the treatment of dystrophic EB. Thirty-one (31) patients were enrolled across three sites and ranged in ages from one (1) year to forty-four (44) years old.

In each patient, a primary wound pair was identified by the investigator; one wound was randomized to receive a weekly topical application of VYJUVEK™ and the other to receive placebo. Wounds were dosed once-weekly with either VYJUVEK™ or placebo until closure. Weekly application was resumed if wounds re-opened at any point in the study.

The primary outcome measure was complete wound healing determined by the Investigator in VYJUVEK™ treated wounds versus placebo treated at the six-month timepoints, meaning week 22 and Week 24 *or* Week 24 and Week 26. Secondary endpoints included investigator assessed complete wound healing at the three-month timepoints, meaning weeks 8 and 10 *or* 10 and 12 and mean change in pain severity using either a VAS or FLACC-R Scale at weeks 22, 24 and 26.

In addition to the primary target wound pair(s), additional wounds (secondary wounds) were selected and treated with VYJUVEK™ giving the treating physicians and patients flexibility to treat multiple wounds during the weekly application. For more information about the pivotal GEM-3 study, visit www.clinicaltrials.gov (NCT04491604).

Subjects returned to the clinical site 30 days following the last dosing visit (Week 26) for safety evaluation by the investigator and subsequently had the option to roll into the Open Label Extension (OLE) Study (NCT04917874). In addition, new participants who were unable to participate in the Phase 3 study but met all enrollment criteria are eligible to enroll in the OLE.

About Dystrophic EB (DEB)

DEB is a rare and severe monogenic disease that affects the skin and mucosal tissues. It is caused by one or more mutations in a gene called *COL7A1*, which is responsible for the formation of the protein type VII collagen protein (COL7) that forms anchoring fibrils that bind the dermis (inner layer of the skin) to the epidermis (outer layer of the skin). The lack of functional anchoring fibrils leads to extremely fragile skin that blisters and tears from minor friction or trauma. DEB patients suffer from open wounds, which leads to skin infections, fibrosis which can cause fusion of fingers and toes, and ultimately an increased risk of developing squamous cell carcinoma which in severe cases can be fatal.

About VYJUVEK™

VYJUVEK™ is an investigational non-invasive, topical gene therapy designed to deliver two copies of the *COL7A1* gene when applied directly to DEB wounds. Unlike the current standard of care, VYJUVEK™ was designed to treat DEB at the molecular level by providing the patient's skin cells the template to make normal COL7 protein, thereby addressing the fundamental disease-causing mechanism.

The FDA and the EMA have each granted VYJUVEK™ orphan drug designation for the treatment of DEB, and the FDA has granted VYJUVEK™ fast track designation and rare pediatric designation for the treatment of DEB. In addition, in 2019, the FDA granted Regenerative Medicine Advanced Therapy ("RMAT") to VYJUVEK™ for the treatment of DEB and the EMA granted PRIority MEdicines ("PRIME"), eligibility for VYJUVEK™ to treat DEB.

About Krystal Biotech

Krystal Biotech, Inc. (NASDAQ:KRYS) is a pivotal-stage gene therapy company leveraging its novel, redosable gene therapy platform and in-house manufacturing capabilities to develop therapies to treat serious rare diseases. For more information, please visit http://www.krystalbio.com, and follow @KrystalBiotech on LinkedIn and Twitter.

Forward-Looking Statements

Any statements in this press release about future expectations, plans and prospects for Krystal Biotech, Inc., including but not limited to statements about the development of Krystal's product candidates, such as plans for the design, conduct and timelines of clinical trials of VYJUVEK™; the clinical utility of VYJUVEK™, and Krystal's plans for filing of regulatory approvals and efforts to bring VYJUVEK™ to market; plans to pursue research and development of other product candidates;; 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 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 trials 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 including VYJUVEK™, the sufficiency of cash resources and need for additional financing and such other important factors as are set forth under the caption "Risk Factors" in Krystal's annual and quarterly reports on file with the U.S. Securities and Exchange Commission. In addition, the forward-looking statements included in this press release represent Krystal's

views as of the date of this release. Krystal anticipates that subsequent events and developments will cause its views to change. However, while Krystal 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 Krystal's views as of any date subsequent to the date of this release.

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Source: Krystal Biotech, Inc.

Exhibit 99.2

Krystal

The Leader in Redosable Gene Therapy for Rare Disease

Topline GEM-3 Trial Results Call

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), KB105, KB104, KB301, KB407, and KB408 and the Company's other product candidates; conduct and timelines of preclinical and clinical trials, the clinical utility of VYJUVEK™ , KB105, KB104, KB301, KB407 and KB408 and the Company's other product candidates; plans for and timing of the review of regulatory filings, efforts to bring VYJUVEK™ , KB105, KB104, KB301, KB407 and KB408 and the Company's other product candidates to market; the market opportunity for and the potential market acceptance of VYJUVEK™ , KB105, KB104, KB301, KB407 and KB408 and the Company's other product candidates, the development of VYJUVEK™ , KB105, KB104, KB301, KB407 and KB408 and the Company's other product candidates for additional indications; the development of additional formulations of VYJUVEK™, KB105, KB104, KB301, KB407 and KB408 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.

This presentation also contains estimates and other statistical data made by independent parties and by us relating to market size and growth and other data about our industry. This data involves a number of assumptions and limitations, and you are cautioned not to give undue weight to such estimates. Neither we nor any other person makes any representation as to the accuracy or completeness of such data or undertakes any obligation to update such data after the date of this presentation. In addition, projections, assumptions and estimates of our future performance and the future performance of the markets in which we operate are necessarily subject to a high degree of uncertainty and risk.

Call Agenda

1 **Introductory Comments**
Krish Krishnan – Chairman and CEO

2 **Dystrophic Epidermolysis Bullosa Background**
Dr. Peter Marinkovich

3 **GEM-3 Results and Next Steps**
Suma Krishnan – Founder and COO

4 **Commercial Preparations**
Andy Orth – Chief Commercial Officer

5 **Q&A**

Krish Krishnan

Chairman and CEO

Novel viral vector platform positively differentiated from other viral vector technologies

1 **Robust tropism to target cells of interest upon local administration**

2 **Large payload capacity allows for delivery of two copies of large gene**

3 **Immune evasive properties of proprietary vectors enables redosability**

GEM-3 results provide significant platform validation

Engineered HSV-1 Vector Platform

GEM-3 Study

Platform fully integrated from research to development to manufacturing to commercialization

1 Confirms safety and efficacy of VYJUVEK™* (beremagene geperpavec) for dystrophic EB

- Most advanced clinical application of platform

2 Validates therapeutic vector in dermatologic applications

- Skin pipeline covers rare and aesthetic conditions (via wholly owned subsidiary Jeune Aesthetics)
- Potential to deliver diverse genetic cargo

3 Validates therapeutic vector for broader redosable gene delivery

- Tropism to lung and additional types under exploration
- Potential to deliver diverse of genetic cargo with a variety of delivery mechanisms

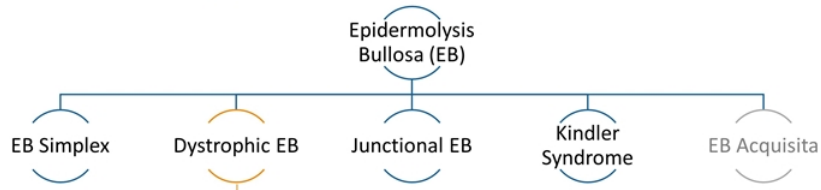
*VYJUVEK is the current proprietary name for beremagene geperpavec, formerly known as B-VEC
VYJUVEK is an investigational therapy being studied in clinical trials

Dr. Peter Marinkovich

Associate Professor of Dermatology at Stanford University
Blistering Disease Clinic Director

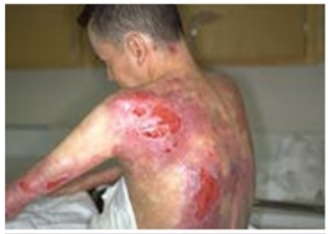
Disclosures: Principal investigator in GEM-3 trial

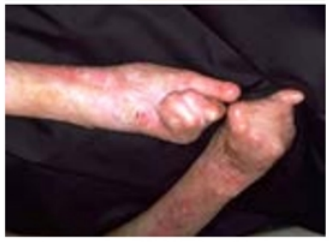
Epidermolysis Bullosa is a group of rare diseases associated with fragile skin, causing skin to blister easily



Dystrophic EB

- One of four inherited forms of EB
- Dystrophic EB can be inherited dominantly (DDEB) or recessively (RDEB); the recessive form of Dystrophic EB is the most severe, chronic type of EB
- Blisters occur in the lower layer of the skin, just beneath the lamina densa in the most superficial portion of the dermis
- Produces debilitating scarring to hands and other parts of the body
- Constant cycle of blistering, wounding and re-healing greatly increases risk of squamous cell carcinoma (SCC) which can be fatal
- Diagnosis has traditionally been made based on skin biopsy and is often incorrect; genetic testing provides the most accurate diagnosis

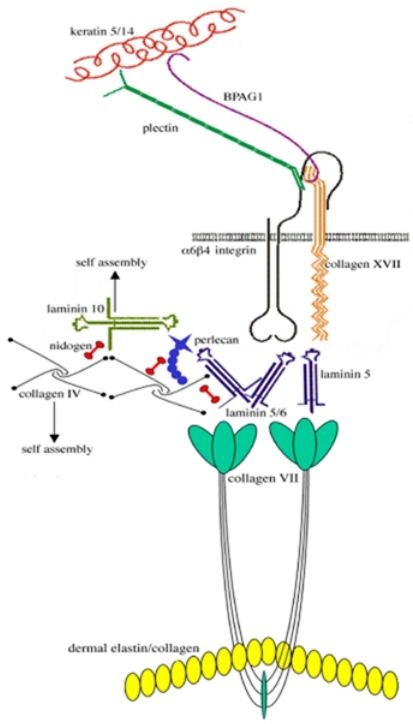




Images courtesy of Dr. Peter Marinkovich

The dystrophic form of EB is caused by mutations in the *COL7A1* gene

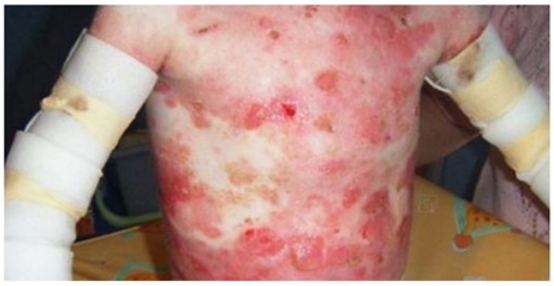
- The location of the blisters (below the lamina densa) corresponds to level of the “anchoring fibrils”
- Anchoring fibrils are the molecular glue that holds the dermis to the epidermis, and are mainly composed of type VII collagen protein (COL7)
- Mutations in the *COL7A1* gene lead to missing or dysfunctional forms of the protein; mutations can be dominant (DDEB) or recessive (RDEB)
- Without functional anchoring fibrils, the skin is fragile and easily shears with even slight friction (holding a pencil, putting on a shirt)



Images courtesy of Dr. Peter Marinkovich

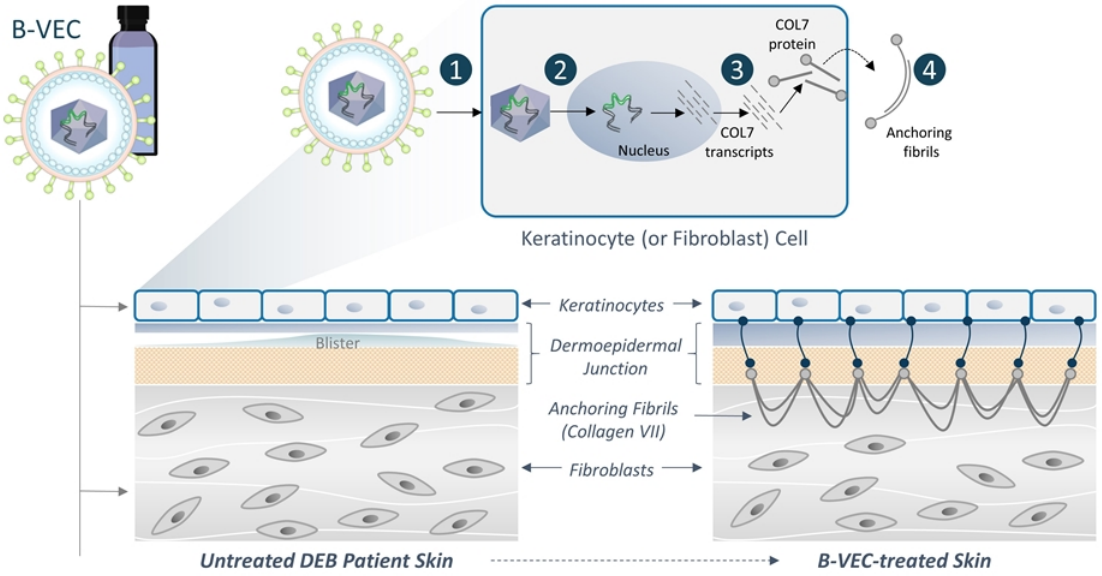
There are currently no approved corrective treatments for dystrophic EB

- Current treatment options for dystrophic EB are largely palliative in nature, involving wound care regimens similar to the care provided to burn victims
- Blistered areas are wrapped in special bandages which must be changed frequently, often daily, which is time consuming and painful
- The goal of treatment is to promote wound healing, prevent infection, and protect the skin form trauma to minimize blister formation
- Multidisciplinary care is often needed, and includes pain management, nutritional support, physical therapy, and other supportive care
- 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

B-VEC is an investigational, off-the-shelf, topical gene therapy designed to correct the underlying molecular defect in DEB wounds



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

B-VEC is an investigational therapy being studied in clinical trials

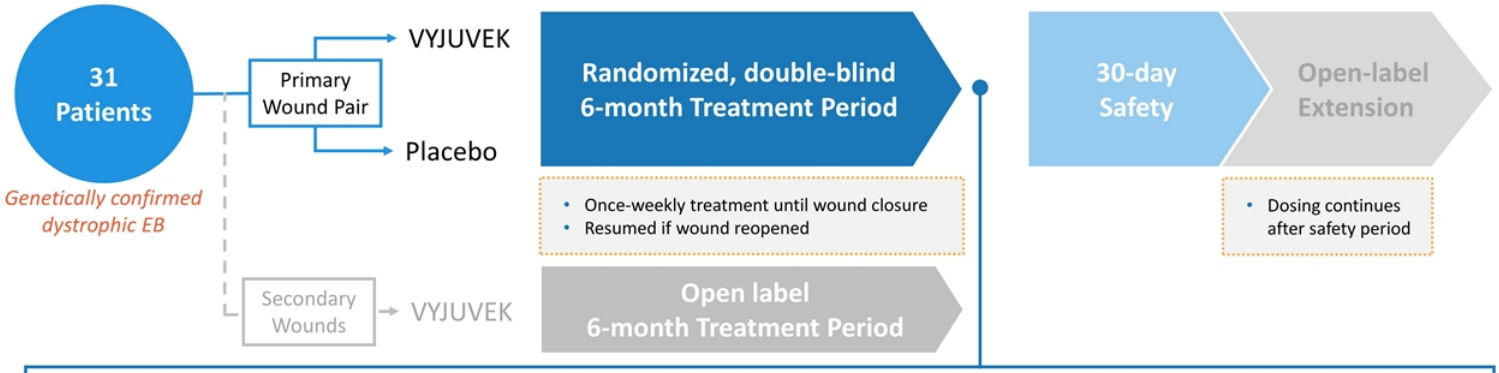
Suma Krishnan

Founder and COO

VYJUVEK™ represents important firsts

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**

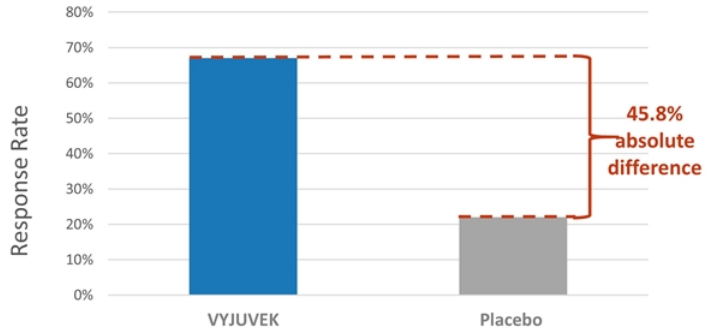
2 **No drug-related serious AEs or discontinuations due to treatment were reported**

- 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**

Topline Ph3 efficacy data summary

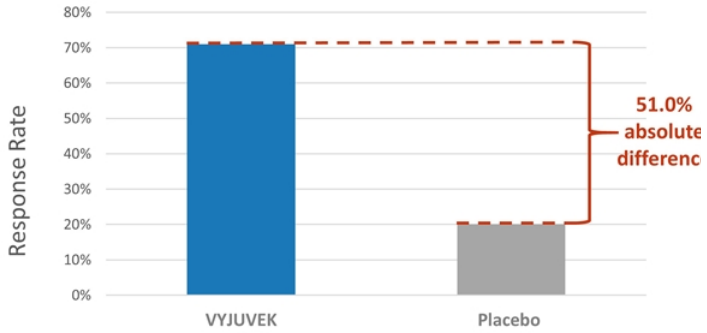
Met **primary endpoint** of complete wound healing at 6-month timepoints



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

*based on McNemar test

Met **secondary endpoint** of complete wound healing at 3-month timepoints



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

*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)

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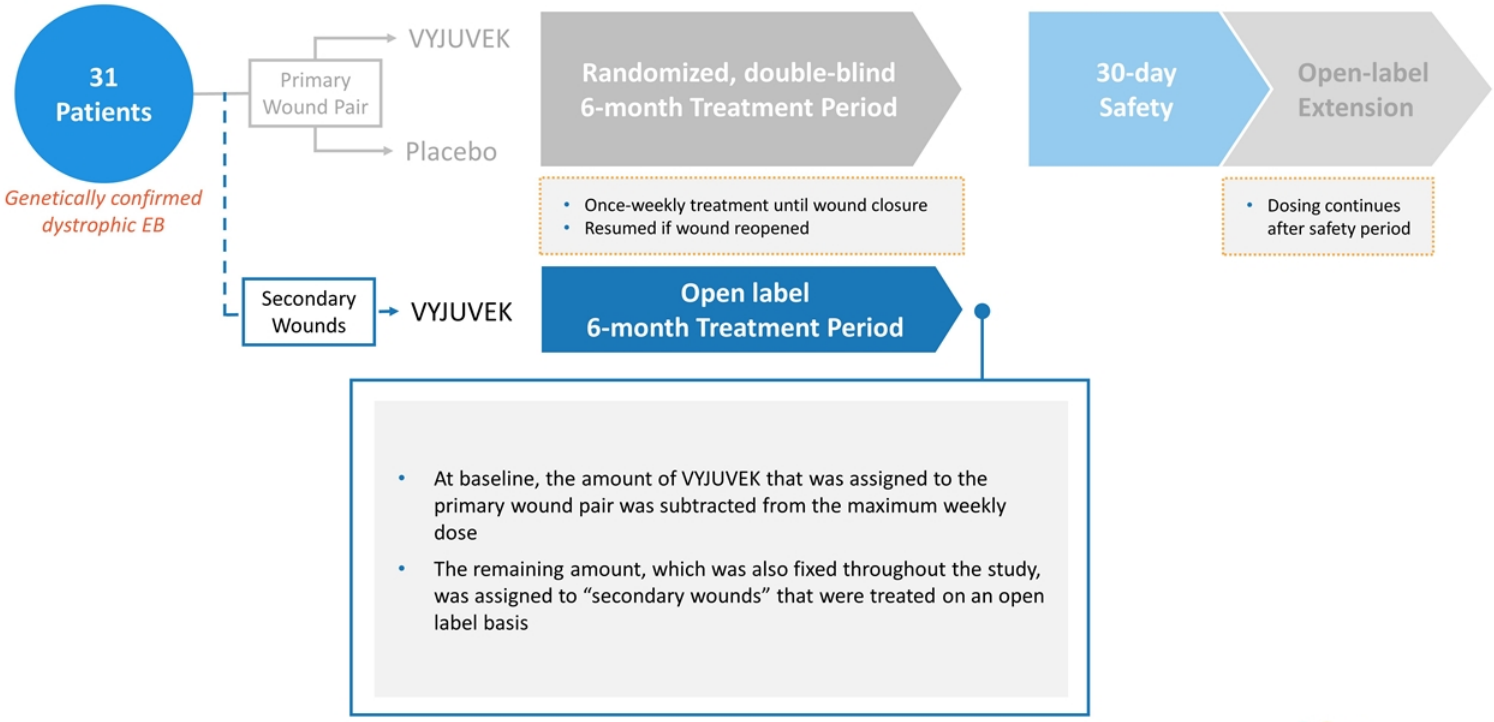
Secondary wounds received open-label VYJUVEK™ throughout the study



- At baseline, the amount of VYJUVEK that was assigned to the primary wound pair was subtracted from the maximum weekly dose
- The remaining amount, which was also fixed throughout the study, was assigned to "secondary wounds" that were treated on an open label basis

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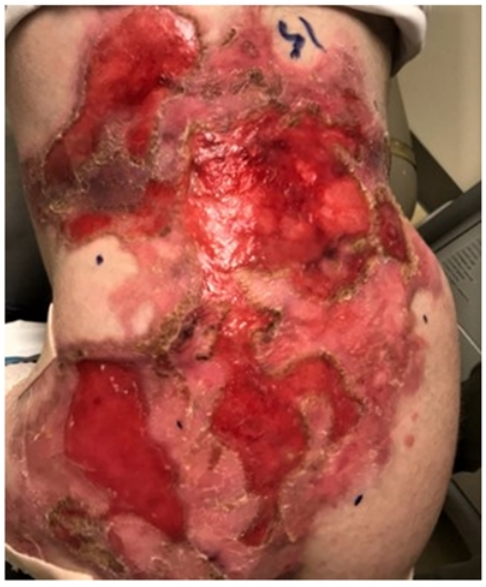
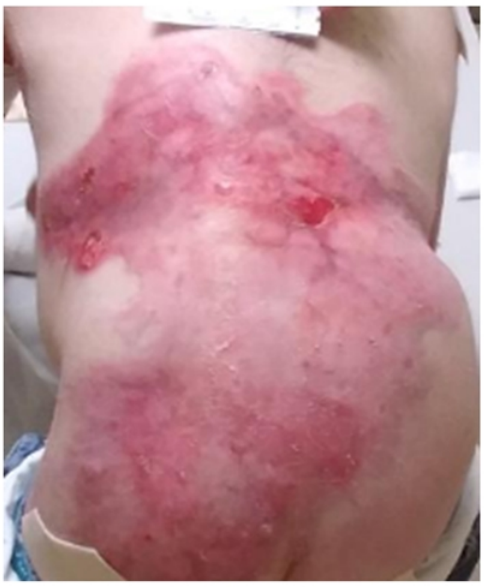
Secondary wound (Illustrative)

Large, chronic back wound in 21 year old RDEB patient



Baseline



End of Study

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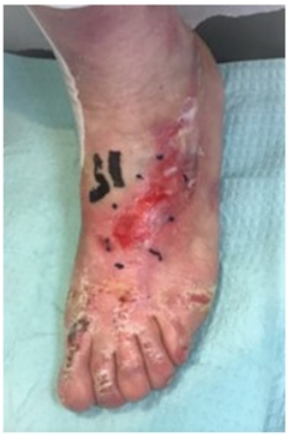
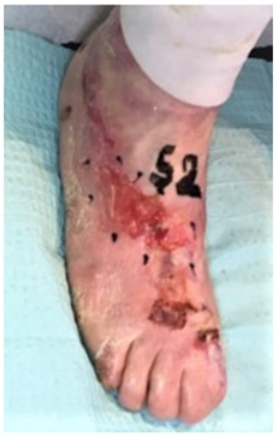
Secondary wound (Illustrative)

Recurring foot wound in 34 year old RDEB patient

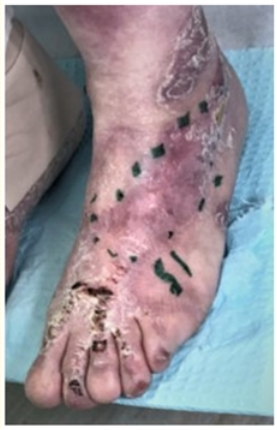


left foot



Right foot

Baseline



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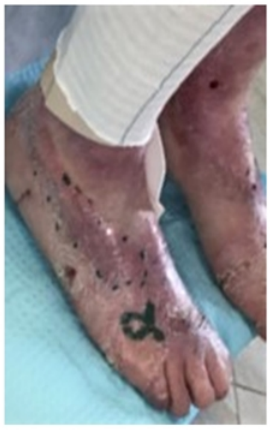
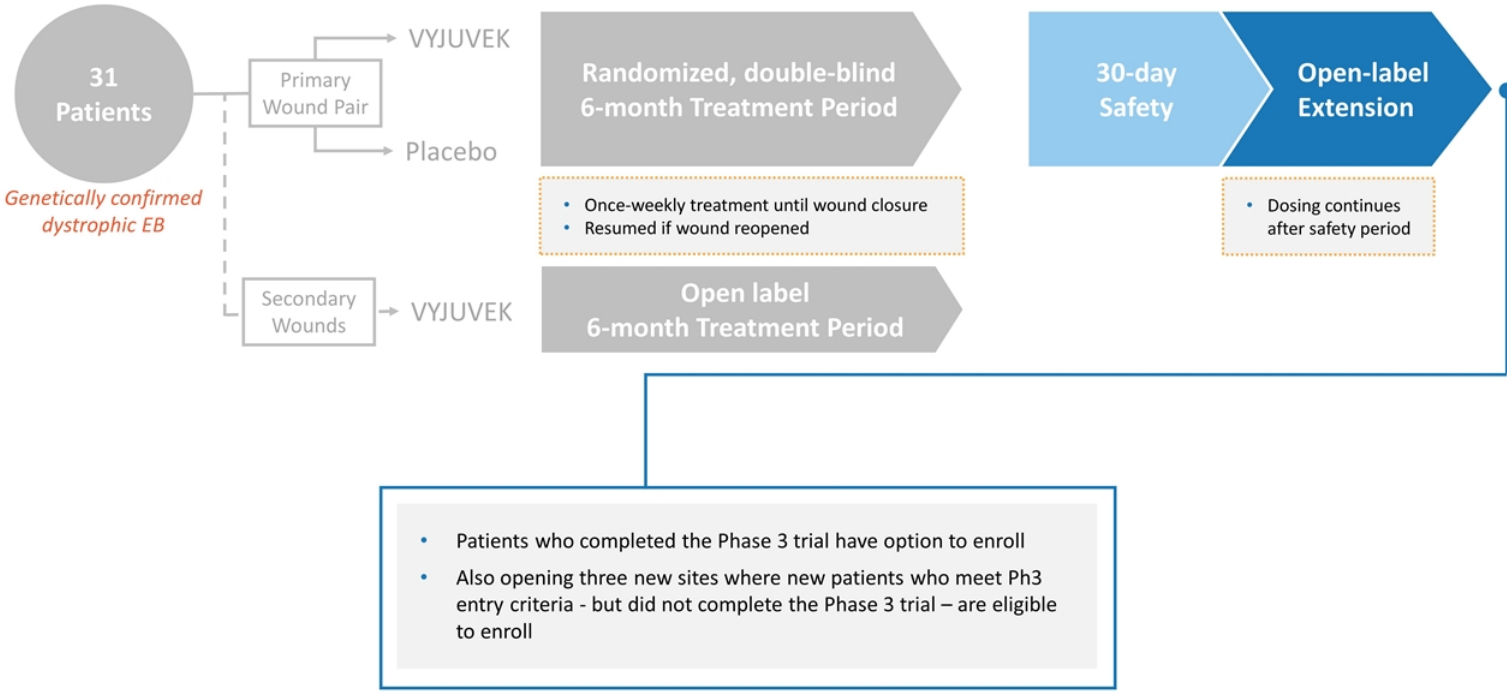


Right foot

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

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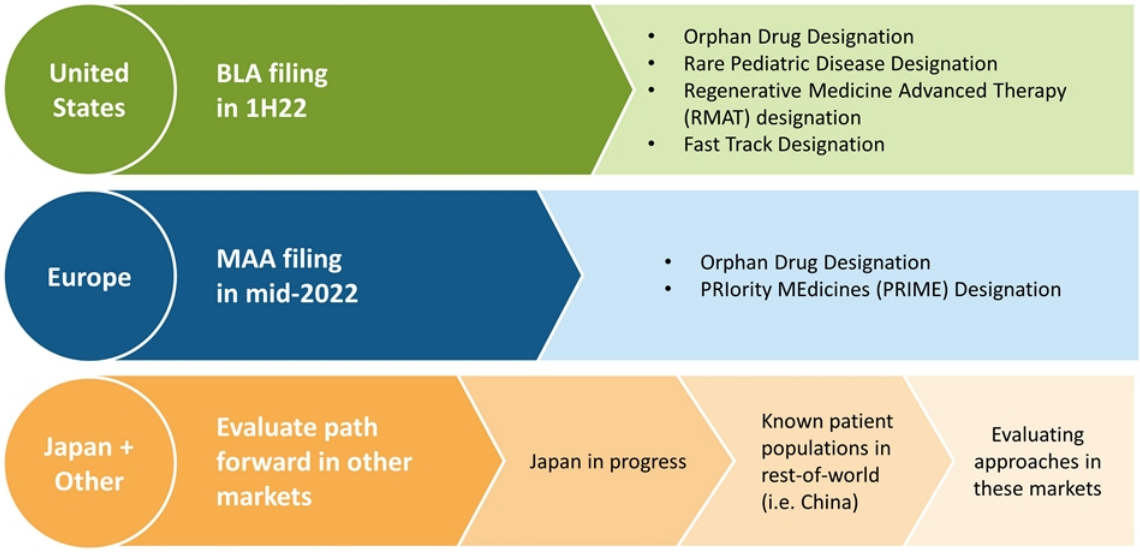
VYJUVEK™ regulatory next steps



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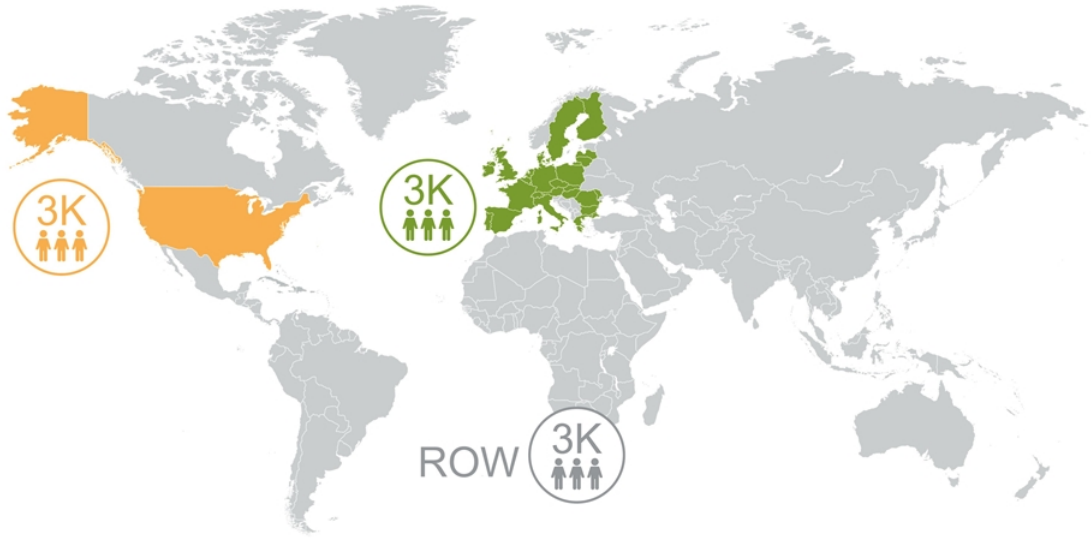
Andy Orth

CCO

Dystrophic EB patient population and VYJUVEK™ opportunity



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Launch readiness / efforts

Education + Awareness

- Patient and Caregiver facing Community Educational Liaisons in the field
- Health Care Professional and Patient focused Disease State Awareness programming underway
- Medical Affairs Key Opinion Leader engagement underway
- Exploring all access pathways in Europe

Krystal Decode DEB™

- No-charge genetic testing available to eligible US residents who are suspected of having EB and have not yet been genetically confirmed.
- Comprehensive testing panel to identify Dystrophic EB or conditions with similar phenotypes, including other EB types and some non-EB genetic blistering conditions.
- Excellent EB community response to date

Payer / Reimbursement

- Early engagement with US payer partners to educate on Dystrophic EB, Krystal and B-VEC
- Will pursue an aggressive and progressive value-based strategy to ensure timely and open access for B-VEC

VYJUVEK is an investigational therapy being studied in clinical trials

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 & 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

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

Krish Krishnan

Chairman and CEO

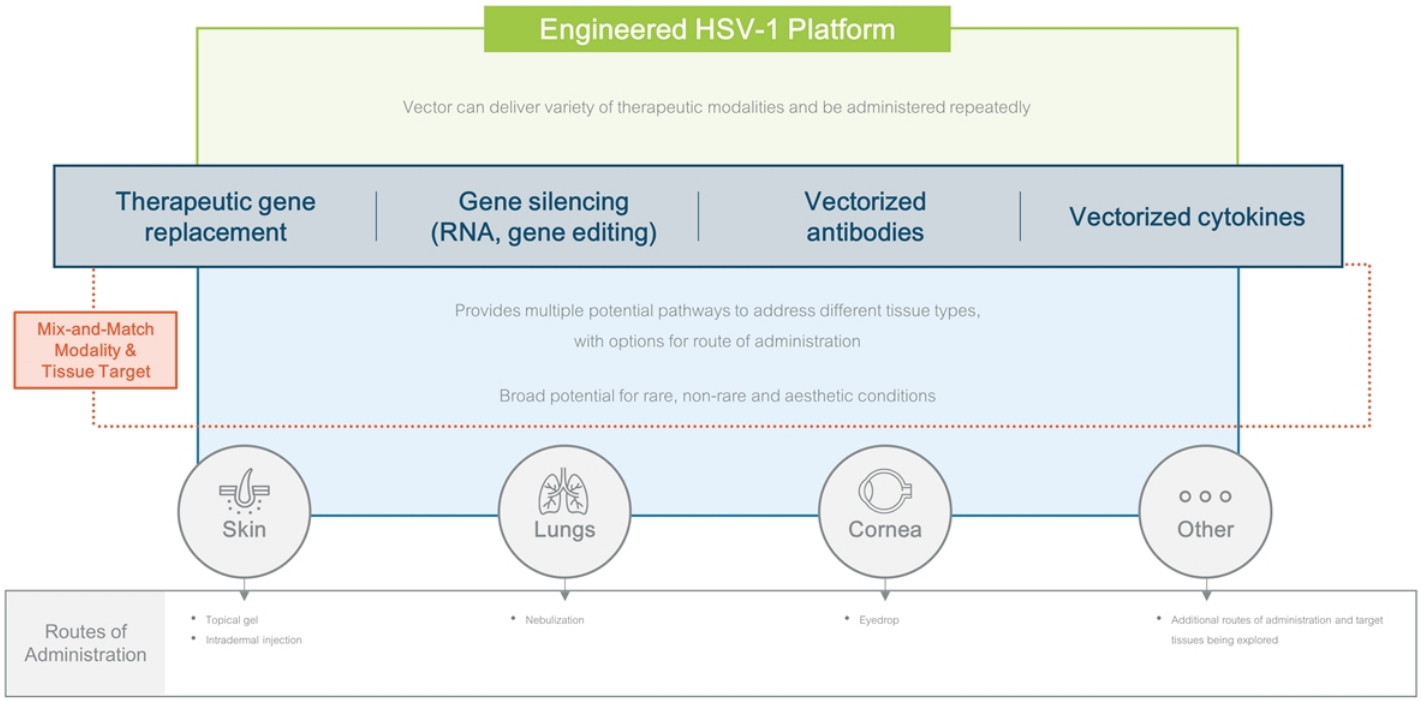
Pipeline upcoming events

	Product	Protein	Indication	Discovery	Preclinical	Phase 1/2	Phase 3	Key Upcoming Milestone	Ownership
Dermatology	VYJUVEK™ †¤•Δ‡§	Type VII collagen (COL7)	Dystrophic EB					File BLA in 1H22	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
Dermatology	KB301	Type III collagen (COL3)	Aesthetic skin conditions					Ph1 efficacy data in 1Q22	JEUNE
Respiratory	KB407†¤‡	Cystic fibrosis transmembrane conductance regulator (CFTR)	Cystic fibrosis					Initiate Ph1 study in 4Q21	Wholly owned
Respiratory	KB408	Alpha-1 antitrypsin (AAT)	Alpha-1 antitrypsin deficiency						Wholly owned
Respiratory	KB4XX	Undisclosed programs							Wholly owned

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

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

Redosable gene delivery technology has broad potential



Questions & Answers