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): April 23, 2023

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 April 23, 2023, Krystal Biotech, Inc. (the "Company") presented data entitled "Topical beremagene geperpavec (B-VEC) for the treatment of recurrent cicatrizing conjunctivitis in a patient with dystrophic epidermolysis bullosa", at the Association for Research in Vision and Ophthalmology ("ARVO") 2023 Annual Meeting in New Orleans, LA. A copy of the press release regarding the Company's presentation of the data at the ARVO meeting and a copy of the poster presented at the ARVO meeting are attached hereto as Exhibit 99.1 and Exhibit 99.2, respectively, and are incorporated herein by reference. The poster is also available on the "Investors" section of the Company's website at www.krystalbio.com.

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.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits.

Exhibit No.	Description
99.1	Press Release dated April 24, 2023.
99.2	Poster entitled "Topical beremagene geperpavec (B-VEC) for the treatment of recurrent cicatrizing conjunctivitis in a patient with dystrophic epidermolysis bullosa".
104	Cover Page Interactive Data file (embedded within the Inline XBRL document)

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: April 24, 2023

KRYSTAL BIOTECH, INC.

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

Krystal Biotech Announces Clinical Data on Topical Application of B-VEC to the Eye to Treat Ocular Complications in a Patient with Dystrophic Epidermolysis Bullosa Under a Compassionate Use Program

- Data presented at Association for Research in Vision and Ophthalmology Annual Meeting on April 23, 2023
- Topical application of B-VEC to the eye was well tolerated and patient showed significant improvement of visual acuity and complete corneal re-epithelization

PITTSBURGH, April 24, 2023 (GLOBE NEWSWIRE) – Krystal Biotech, Inc. (the "Company") (NASDAQ: KRYS), a biotechnology company focused on developing and commercializing genetic medicines for patients with rare diseases, announced today that the Company presented new data on the compassionate use of topical beremagene geperpavec (B-VEC) to treat a patient with dystrophic epidermolysis bullosa (DEB) with recurrent cicatrizing conjunctivitis at the Association for Research in Vision and Ophthalmology (ARVO) 2023 Annual Meeting on April 23, 2023.

"DEB is a devastating disease with limited treatment options, and there is a substantial population of DEB patients with ocular complications for which treatment options are limited and often include surgery," said Alfonso L. Sabater, M.D., PhD, Assistant Professor of Clinical Ophthalmology at the Bascom Palmer Eye Institute at the University of Miami Miller School of Medicine. "It is exciting to potentially advance a topical treatment for patients with ocular complications associated with DEB."

The data presented describes the first application of B-VEC to treat ocular complications in a patient with DEB under a compassionate use program. The patient presented with cicatrizing conjunctivitis and underwent surgical symblepharon lysis with pannus removal in the right eye. B-VEC was administered to the patient's right eye at regular intervals following surgery in addition to routine post-surgical management.

B-VEC was well tolerated and associated with full corneal healing by 3 months as well as significant visual acuity improvement from hand motion to 20/40 at 7 months, the latest time point of the on-going treatment effect evaluation.

Figure 1: Slit lamp pictures of the right eye. **A:** Baseline ankyloblepharon. The visual acuity was hand motion (HM) **B:** Ocular surface of the right eye 6 months after the surgery after B-VEC applications.

No drug-related adverse events (AE) have been observed. Two non drug related, serious AEs were reported: 1) Prolonged hospitalization due to complications post-gastrointestinal surgery, and 2) Prolonged hospitalization due to complications post-esophageal dilation. B-VEC treatment was not interrupted during either event.

Ocular complications are common in patients with DEB, with over half of the patients diagnosed with recessive DEB potentially affected. Typical ocular manifestations include corneal abrasion, as well as corneal scarring, pannus, eyelid ectropions and blisters.[1,2] There are no specific FDA-approved treatment options for ocular manifestations of DEB.[3]

“Ocular complications impose a heavy burden on DEB patients. Based on this promising initial data, we plan to engage with regulatory authorities and explore how we can expand the utility of B-VEC to address this urgent need,” said Suma Krishnan, President, Research & Development, Krystal Biotech. “We are also excited about the implications for our platform as this clinical data, together with ongoing preclinical studies evaluating intravitreal and subretinal routes of delivery to the eye, suggests significant potential to treat multiple ocular diseases with few or no treatment options.”

The poster was available to conference attendees and is available on the Investor section of the Company’s website.

About Dystrophic Epidermolysis Bullosa (DEB)

DEB is a rare and severe 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 production of the protein type VII collagen (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 in DEB patients 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 an aggressive form of squamous cell carcinoma which, in severe cases, can be fatal.

About B-VEC

B-VEC is an investigational non-invasive, topical, redosable gene therapy designed to deliver two copies of the *COL7A1* gene when applied directly to DEB wounds. B-VEC 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 EMA have each granted B-VEC orphan drug designation for the treatment of DEB, and the FDA has granted B-VEC fast track designation and rare pediatric designation for the treatment of DEB. In addition, the FDA granted Regenerative Medicine Advanced Therapy (RMAT) to B-VEC for the treatment of DEB and the EMA granted PRIority MEdicines (PRIME) eligibility for B-VEC to treat DEB.

About Krystal Biotech, Inc.

Krystal Biotech, Inc. (NASDAQ: KRYS) is a biotechnology company focused on developing and commercializing genetic medicines for patients with rare diseases. The Company's wide-ranging pipeline is based on its proprietary redosable HSV vector. Headquartered in Pittsburgh, Pennsylvania, the Company is led by an experienced management team, is fully-integrated and has core capabilities in viral vector design, vector optimization, gene therapy manufacturing and commercialization. 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 statements about our plans to engage with regulatory authorities to explore how we can expand the utility of B-VEC; the significant potential of our platform to treat multiple ocular diseases with few or no treatment options; 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: uncertainties associated with regulatory review of clinical trials and applications for marketing approvals, the availability or commercial potential of B-VEC, 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 the Company'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 the Company's views as of the date of this release. 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 release.

CONTACT
Investors and Media:
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mdodge@krystalbio.com

1. Tang JY, Marinkovich MP, Lucas E, et al. A systematic literature review of the disease burden in patients with recessive dystrophic epidermolysis bullosa. *Orphanet J Rare Dis*. 2021 Apr 13; 16(1): 175. doi: 10.1186/s13023-021-01811-7.

2. Tong L, Hodgkins PR, Denyer J, et al. The eye in epidermolysis bullosa. *Br J Ophthalmol*. 1999 Mar; 83(3): 323-6. doi:10.1136/bjo.83.3.323.

3. Chen VM, Mehta N, Robbins CC, et al. Anterior-segment spectral domain optical coherence tomography in epidermolysis bullosa. *Ocul Surf.* 2020 Oct; 18(4): 912-919. doi: 10.1016/j.jtos.2020.08.010

UNIVERSITY OF MIAMI

Topical beremagene geperpavec (B-VEC) for the treatment of recurrent cicatrizing conjunctivitis in a patient with dystrophic epidermolysis bullosa

Bascom Palmer Eye Institute
UNIVERSITY OF MIAMI HEALTH SYSTEM

Sabater, Alfonso L, MD, PhD[1]; Tovar, Arianna, MD[1]; Gomez, Jennifer[1]; Parry, Trevor, PhD[2]; Chen, Hubert, MD[2]; Agostini, Brittani, BSN[2]; Krishnan, Suma, MS[2].

1. University of Miami Health System Bascom Palmer Eye Institute, Miami, FL, United States. 2. Krystal Biotech, Pittsburgh, PA, United States.

BACKGROUND/PURPOSE

- Dystrophic epidermolysis bullosa (DEB) is a rare genetic blistering skin disease caused by mutations in the *COL7A1* gene. It can be inherited in an autosomal dominant (DDEB) or recessive (RDEB) manner.[1]
- A subset of individuals with DEB may develop ocular surface involvement, including abrasions, blistering and scarring, that can lead to impaired vision and eventual blindness. Current therapies are limited to removal of scar tissue and ophthalmic lubrication.[2-4]
- Using a replication deficient herpes simplex virus type 1 (HSV-1)-based gene delivery platform, beremagene geperpavec (B-VEC) has been engineered to deliver functional human type VII collagen (COL7).
- B-VEC was evaluated in a phase 3, double-blind, placebo-controlled trial of 31 patients with DEB. B-VEC treatment demonstrated a statistically significant improvement in complete wound healing at 3 and at 6 months compared with placebo. B-VEC was well tolerated and is an investigational topical gene therapy currently under review by the FDA.[5]
- Based on the mechanism of action of B-VEC and the biochemical and ultrastructural similarities between the skin and the cornea, it was hypothesized that B-VEC may also potentially provide a therapeutic benefit for the ocular manifestations in patients with DEB.
- We hereby present a case of a male with RDEB and a history of corneal blindness (**Figure 1A**). Approval from the FDA for the compassionate use of B-VEC was obtained in 2021 (IND #27789), and in 2022 from the University of Miami's Institutional Review Boards (#20211165). **To our knowledge, our patient is the first human treated with a topical ocular gene therapy.**

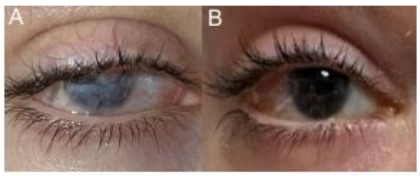


Figure 1: Slit lamp pictures of the right eye. **A:** Baseline ankyloblepharon. The visual acuity was hand motion (HM) **B:** Ocular surface of the right eye 6 months after the surgery and 23 B-VEC applications.

MATERIALS AND METHODS

- Compassionate use study in a 13-year-old male with RDEB at the Bascom Palmer Eye Institute (Miami, FL) who presented with bilateral advanced cicatrizing conjunctivitis (CC). The patient presented with blisters in the skin since birth, and the diagnosis of RDEB was confirmed via genetic analysis at age 7. There was no family history of the disease. He has bilateral contraction and syndactyly of hands and fingers, and to date, his whole body is always wrapped in elastic bandages, except for his face and neck .
- He developed conjunctival blisters at ages 4 and 6 in the left and right eye, respectively. He underwent repeated superficial keratectomy and symblepharon lysis surgeries with amniotic membrane transplantation in his left eye (x2). These resulted in temporary visual acuity improvement, with posterior recurrence and regression to baseline in less than 3 months. He also has bilateral limbal stem cell deficiency since age 8.
- The patient enrolled in the phase 3 trial of B-VEC in 2020 and received treatment of B-VEC on skin wounds. After completion of the phase 3 trial, patient continued receiving B-VEC for wounds in the open-label extension (OLE) trial.
- Surgical symblepharon lysis with pannus removal, immediate ophthalmic B-VEC drug product application (5×10^9 PFU/mL) and suturing of an amniotic membrane were performed on his right eye in August 2022. A bandage contact lens (BCL) was placed on top of the amniotic membrane. Simultaneously, he got an esophageal dilation with gastrostomy tube placement, as he was having severe dysphagia caused by esophageal strictures.
- After the surgery, topical B-VEC was instilled continuously: 3 times/week for the first 2 weeks; then once weekly until the corneal epithelium healed completely (assessed via slit lamp and anterior segment optical coherence tomography-OCT); and then, once monthly. The patient was concurrently using ophthalmic prednisolone, moxifloxacin, allogeneic immunosafe PRGF, insulin eyedrops and artificial tears.

RESULTS

Corneal epithelium healing: Full corneal epithelial healing was observed at 3-months after the surgery with topical B-VEC applications (19 doses). 6-months after the surgery and continuous B-VEC therapy (23 doses), there were no signs of symblepharon recurrence or corneal scarring. **(Figures 1B and 2)**

Figure 2: Anterior segment OCT, with no evidence of corneal scarring or infiltrates.

Retina: After 7 months and 24 doses of B-VEC, no abnormalities were observed in the retina **(Figure 3).**

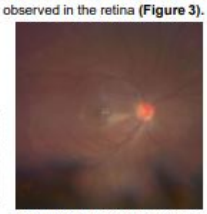
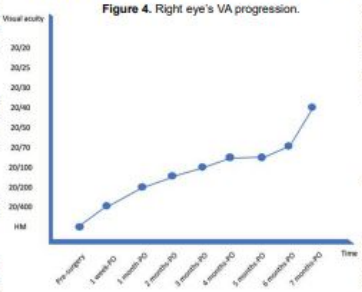


Figure 3: Fundoscopic image of right eye's retina.

Visual acuity (VA)

The patient's right eye VA went from hand motion (HM) prior to the interventions to 20/40 7 months post-surgery and 24 doses of B-VEC **(Figure 4).**

Figure 4. Right eye's VA progression.



Safety: No drug-related adverse events (AE) have been observed.

Two non-related, serious AEs were reported:

1) Prolonged hospitalization due to complications post-gastrointestinal surgery, and
2) Prolonged hospitalization due to complications post-esophageal dilation

B-VEC treatment was not interrupted during either event.

CONCLUSIONS

- Topical ocular application of B-VEC in this 13-year-old patient with RDEB was well-tolerated with no signs of ocular HSV-1-like disease. VA improved without recurrence of symblepharon through 7 months of B-VEC use.
- Based on this first case, B-VEC has the potential to be a safe and effective treatment for recurrent cicatrizing conjunctivitis in patients with DEB. Longer follow-up and future, larger studies are required for further and consistent evidence

REFERENCES

1. Boeira VL, Souza ES, Rocha Bde O, et al. Inherited epidermolysis bullosa: clinical and therapeutic aspects. An Bras Dermatol 2013;88:185-98
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3. Bachir Y, Daruich A, Marie C, Robert MP, Bremond-Gignac D. Eye Involvement and Management in Inherited Epidermolysis Bullosa. Drugs 2022;82:1277-85.
4. Yaycioglu R, Akova YA, Oto S. Amniotic membrane transplantation for treatment of symblepharon in a patient with recessive dystrophic epidermolysis bullosa. Cornea 2006;25:971-3.
5. Guide SV, Gonzalez ME, Bagci IS, et al. Trial of Beremagene Geperpavec (B-VEC) for Dystrophic Epidermolysis Bullosa. N Engl J Med 2022;387:2211-9.

DISCLOSURES

Commercial Relationships Disclosure: Alfonso Sabater: Commercial Relationship(s):Code C (Consultant/Contractor):Abbvie;Code C (Consultant/Contractor):GlaxoSmithKline;Code C (Consultant/Contractor):Brill Pharma;Code C (Consultant/Contractor):Laboratorios Sophia;Code O (Owner):TissueCor;Code O (Owner):LycOrop;Code O (Owner):PlasmaCord | Arianna Tovar: Commercial Relationship: Code N (No Commercial Relationship) || Jennifer Gomez: Commercial Relationship: Code N (No Commercial Relationship) | Trevor Parry: Commercial Relationship(s):Code E (Employment):Krystal Biotech | Hubert Chen: Commercial Relationship(s):Code E (Employment):Krystal Biotech | Brittani Agostini: Commercial Relationship(s):Code E (Employment):Krystal Biotech | Suma Krishnan: Commercial Relationship(s):Code E (Employment) |:Krystal Biotech