



## Genetic Medicines for High Unmet Medical Needs

JPM 2026



# Forward Looking Statements and Disclosures

## Forward Looking Statements

This presentation and our discussion contain forward-looking statements that involve substantial risks and uncertainties. Any statements about future expectations, plans, and prospects for Krystal Biotech, Inc. (together with its subsidiaries, the “Company”), including but not limited to, statements about our global VYJUVEK launch; selected preliminary 2025 unaudited results; our 2030 vision and the market potential of the product candidates that could be launched over the next four years; the development and commercial potential of our product candidates, including our expectations for our product pipeline, including clinical trial plans, enrollment in clinical trials, and the timing of initiating clinical trials, dosing patients, data read-outs, and regulatory submissions; our platform technology designation and potential benefits; and other statements about our business, operations, and financial results, 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 reviews and the content and timing of regulatory authorities’ decisions; uncertainties in the initiation and conduct of clinical trials and availability and timing of data from clinical trials; whether results of early clinical trials will be indicative of the results of ongoing or future trials; the availability or commercial potential of product candidates; and such other important factors as are set forth in the Company’s filings with the U.S. SEC. The forward-looking statements represent the Company’s views as of the date of this presentation and should not be relied upon as representing the Company’s views as of any subsequent date. The Company specifically disclaims any obligation to update forward-looking statements.

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## Disclosures

Other than VYJUVEK, all products described in this presentation are investigational therapies.

Today’s discussions and presentation are intended for the investor community only; they are not intended to promote the products referenced herein or otherwise influence healthcare prescribing decisions.

The Company is using the Aerogen Solo® Nebulizer System and Aerogen® Ultra in its clinical trials evaluating KB407, KB408, and inhaled KB707.

VYJUVEK® is a registered trademark of Krystal Biotech, Inc. OXERVATE® is a registered trademark of Dompé farmaceutici S.p.A.

# Global VYJUVEK Launch



## 2023

First launch in  
the United States

**Over 1.2K**  
Identified DEB Patients  
in the United States\*

\*Estimated Total U.S. DEB Population of 3K

## 2H 2025

Approval and launch in  
France, Germany, and Japan

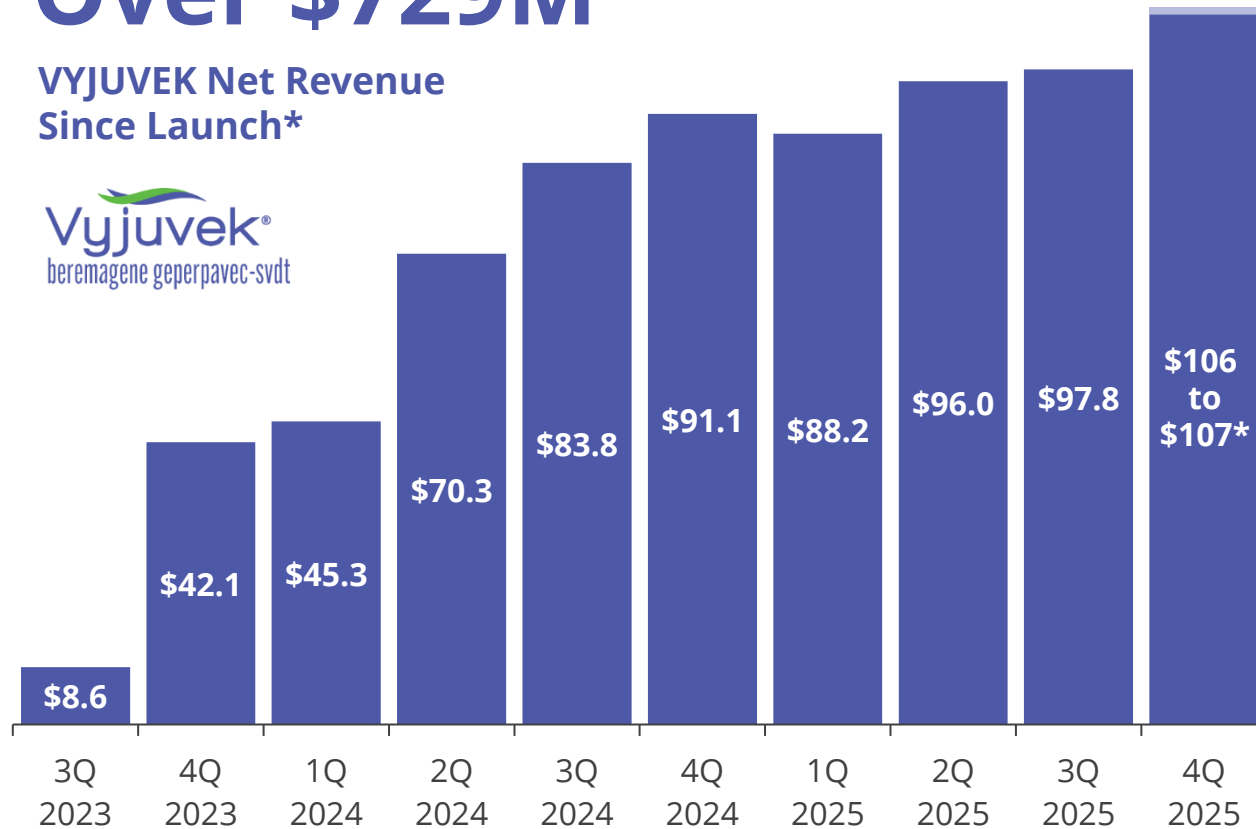
**Over 1.3K**  
Identified DEB Patients  
Across All Three Markets

+ distribution agreements also now in place for over  
twenty countries across Europe and Middle East

# Fully Integrated with Strong Financial Foundation

## Over \$729M

VYJUVEK Net Revenue Since Launch\*



Net Revenue (\$M)

## 175K+ sq ft

Combined size of Krystal's two U.S. cGMP manufacturing facilities



ANCORIS: 20K+ sq ft



ASTRA: 150K+ sq ft

## \$955M

Approximate cash and investments as of 4Q 2025\*

## 10 Consecutive quarters of positive EPS\*

\* 4Q 2025 net VYJUVEK revenue and revenue summaries including the same, approximate cash balance as of 4Q 2025, and expectation of positive EPS for 4Q 2025 are preliminary and unaudited. These preliminary unaudited results are based on management's initial analysis of operations for the year ended December 31, 2025 and subject to adjustment. The Company will report its full financial results for the fourth quarter and full year 2025 in February 2026

cGMP, current good manufacturing practices; EPS, earnings per share; U.S., United States

# Krystal's Vision for 2030




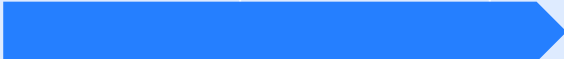
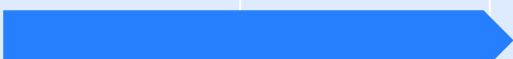
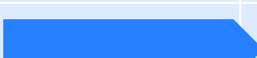
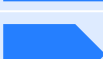



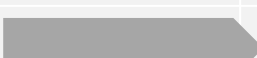

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Krystal Marketed Rare Disease Medicines, Inclusive of VYJUVEK

10,000+

Patients Treated with Krystal Rare Disease Medicines

# Deep Pipeline of Genetic Medicines That Leverage the Strengths of HSV-1

|   |  | Indication                                  | Payload    | Preclinical   | Phase 1/2   | Registrational | Commercial  |
|---|--|---|------------|---|---|----------------|---|
|  |  | Dystrophic epidermolysis bullosa (DEB)      | COL7A1     |    |   |                | U.S.<br>Europe<br>Japan   |
| Rare Disease Pipeline   | KB803  | Ocular complications of DEB                 | COL7A1     |    |   |                |   |
|   | KB801  | Neurotrophic keratitis                      | NGF        |    |   |                |   |
|   | KB407  | Cystic fibrosis                             | CFTR       |    |   |                |   |
|   | KB111  | Hailey-Hailey disease                       | ATP2C1     |    |   |                |   |
|   | Additional program(s) targeting rare diseases of the eye, lung, and skin |   |            |   |    |                |   |
| Expanded Pipeline   | KB408  | Alpha-1 antitrypsin deficiency lung disease | SERPINA1   |   |   |                | <b>+ Wholly-Owned Clinical-Stage Aesthetics Subsidiary</b><br><br> |
|   | Inhaled KB707  | Non-small cell lung cancer                  | IL2 + IL12 |  |   |                |   |
|   | Injectable KB707   | Solid tumors including cutaneous            | IL2 + IL12 |  |   |                |   |
|   | Additional exploratory preclinical program(s)                            |   |            |   |  |                |   |

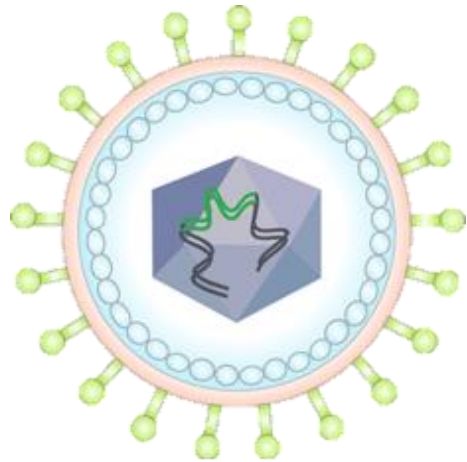
ATP2C1, ATPase secretory pathway Ca<sup>2+</sup> transporting 1; CFTR, cystic fibrosis transmembrane conductance regulator; COL7A1, collagen type VII alpha 1 chain; EMA, European Medicines Agency; FDA, U.S. Food and Drug Administration; HSV-1, herpes simplex virus 1; IL-12, interleukin-12; IL-2, interleukin-2; NGF, nerve growth factor; PMDA, Japan's Pharmaceuticals and Medical Devices Agency; SERPINA1, serpin family A member 1; U.S., United States

# The Next Four Years Could Be Transformational for Krystal

| Program      | Indication   | Estimated Prevalence | Clinical Status           | Market Potential |
|--------------|--|----------------------|---------------------------|------------------|
| <b>KB803</b> | Ocular Complications of DEB                        | 1K+ in US and Europe | Enrolling Registrational  | <b>\$300M+</b>   |
| <b>KB801</b> | Neurotrophic Keratitis                             | 68K in the US        | Enrolling Registrational  | <b>\$1B+</b>     |
| <b>KB407</b> | Modulator-Ineligible or Refractory Cystic Fibrosis | 10K to 30K           | Registrational Start 2026 | <b>\$2B+</b>     |
| <b>KB111</b> | Hailey-Hailey Disease                              | 10K to 15K           | Registrational Start 2026 | <b>\$1B+</b>     |

***Over \$4B in combined market potential from rare disease pipeline with potential launches within next four years***

# Building on our HSV-1 Advantage



**Krystal's engineered,  
replication-incompetent  
HSV-1 vectors**

## FDA Platform Technology Designation granted 4Q 2025

- ✓ Large cargo capacity
- ✓ Broad cellular tropism
- ✓ Flexible administration options
- ✓ Redosable
- ✓ Low immunogenicity and no integration risk
- ✓ Scalable manufacturing

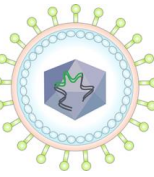
**2026 Focus:** Advance rare disease clinical pipeline through multiple value unlocking milestones

# KB803 for Treatment of Corneal Abrasions in DEB Patients

Registrational study readout expected in 2026

KB803

Krystal's HSV-1 vector encoding COL7A1 for eye drop application



## Ocular Disease Burden of DEB

- Many DEB patients suffer from ocular complications related to lack of collagen VII in the eye
- Corneal abrasion, scarring, and pannus are among the most commonly cited issues
- Can lead to progressive vision loss and even blindness
- No** corrective therapy available

**Over 50%**

**Proportion of Recessive DEB Patients Affected**

## Compassionate Use Case

Treated Eye  
  
Baseline



6 Months



## Visual Acuity in Treated Eye

| Time                        |          | Visual Acuity |
|-----------------------------|----------|---------------|
| Baseline / Prior to Surgery |          | HM            |
| After Surgery               | 1 Week   | 20/400        |
|                             | 1 Month  | 20/200        |
|                             | 2 Months | 20/150        |
|                             | 3 Months | 20/100        |
|                             | 4 Months | 20/80-2       |
|                             | 5 Months | 20/80-1       |
|                             | 6 Months | 20/70         |
|                             | 7 Months | 20/40         |
| 8 Months                    | 20/25    |               |

**Phase 3 study evaluating KB803 for the treatment and prevention of corneal abrasions in DEB patients is underway**

Tang JY, et al. *Orphanet J Rare Dis.* 2021;16(1):175; Tong L, et al. *Br J Ophthalmol.* 1999; 83(3):323-326; Chen VM, et al. *Ocul Surf.* 2020; 18(4):912-919; Krystal Biotech. Data on File

Sabater A et al., Poster # 787 - C0388. at the 2023 Association for Research in Vision and Ophthalmology Annual Meeting; Vetencourt AT, et al. *N Engl J Med.* 2024;390:530-535

BLA, Biologics License Application; COL7A1, collagen type VII alpha 1 chain; DEB, dystrophic epidermolysis bullosa; HM, hand motion; HSV-1, herpes simplex virus 1

# KB801 for the Treatment of Neurotrophic Keratitis

Currently enrolling in upsized registrational study and expecting top-line data in 2026

KB801

Krystal's HSV-1 vector encoding NGF for eye drop application



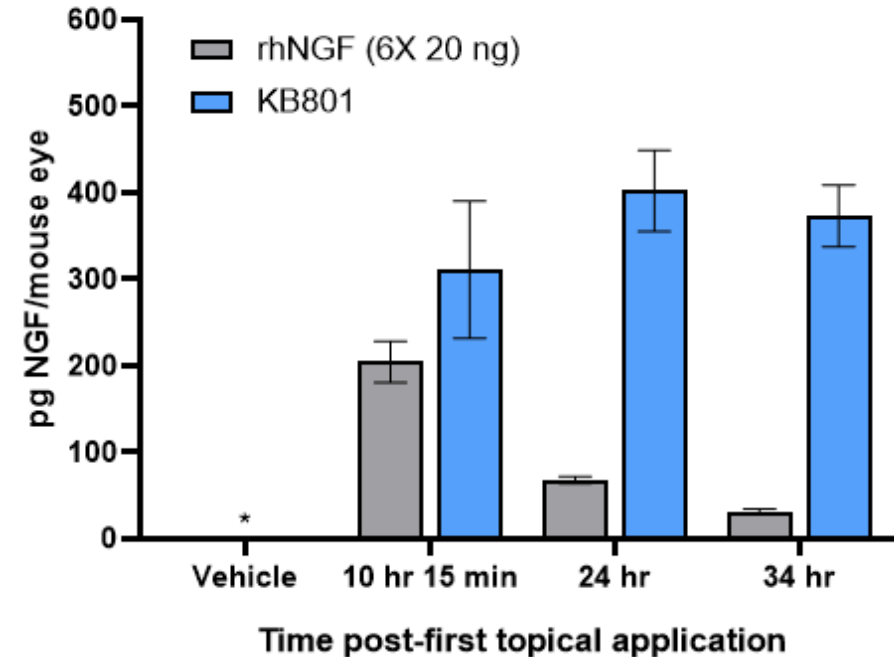
## Neurotrophic Keratitis

- Degenerative disease of the cornea triggered by corneal nerve damage and leading progressive and persistent defects in the corneal epithelium
- All cases associated with some degree of vision impairment, severe cases lead to blindness
- Rare but awareness is rising with over 68K patients in the United States based on claims analysis
- Only available therapy is recombinant NGF product Oxervate® that must be dosed 6x daily for eight weeks

Over \$1B

Oxervate® Net Revenue in the United States in 2024

## Head to Head Comparison of Eye NGF Levels in Wounded Cornea Model



Sacchetti M, et al. *Clin Ophthalmol*. 2014. 8: 571-579; Bian Y, et al. *Ophthalmology*. 2022. 129: 1255-1262; Rama P, et al. *Orphanet*. Neurotrophic Keratopathy. 2017 [accessed Sept 28 2023]; Gablson EE et al. *Invest Ophthalmol Vis Sci*. 2018. 59: 1800; Komodo Health H16.23\* Claims Analysis [June 2025]; Dana R, et al. *BMC Ophthalmol*. 2021. 21: 327; CADTH Review – Cenegermin (Oxervate). 2022. Vol 2, Issue 9; Pflugfelder SC, et al. *Ophthalmology*. 2020. 127: 14-26; Bonini S, et al. *Ophthalmology*. 2018. 125: 1332-1343; Oxervate® 2019 FDA Label; Komodo Health Drug Projections [June 2025]

Cartwright HN, et al. Poster #2467 at the 2025 Association for Research in Vision and Ophthalmology (ARVO) Annual Meeting

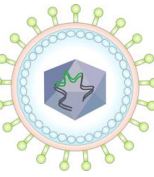
HSV-1, herpes simplex virus 1; NGF, nerve growth factor; rhNGF, recombinant human nerve growth factor

# KB407 for the Treatment of Cystic Fibrosis

Registrational study expected to start in 1H 2026

KB407

Krystal's HSV-1 vector encoding *CFTR* for delivery via inhalation



## Cystic Fibrosis

- Progressive lung disease caused by mutations in the *CFTR* gene and loss of CFTR-mediated ion transport
- Life-span limiting disease characterized by airway obstruction and inflammation, cough, shortness of breath, and infection
- Many patients with cystic fibrosis either ineligible or underserved by currently available modulator therapies

**10K**

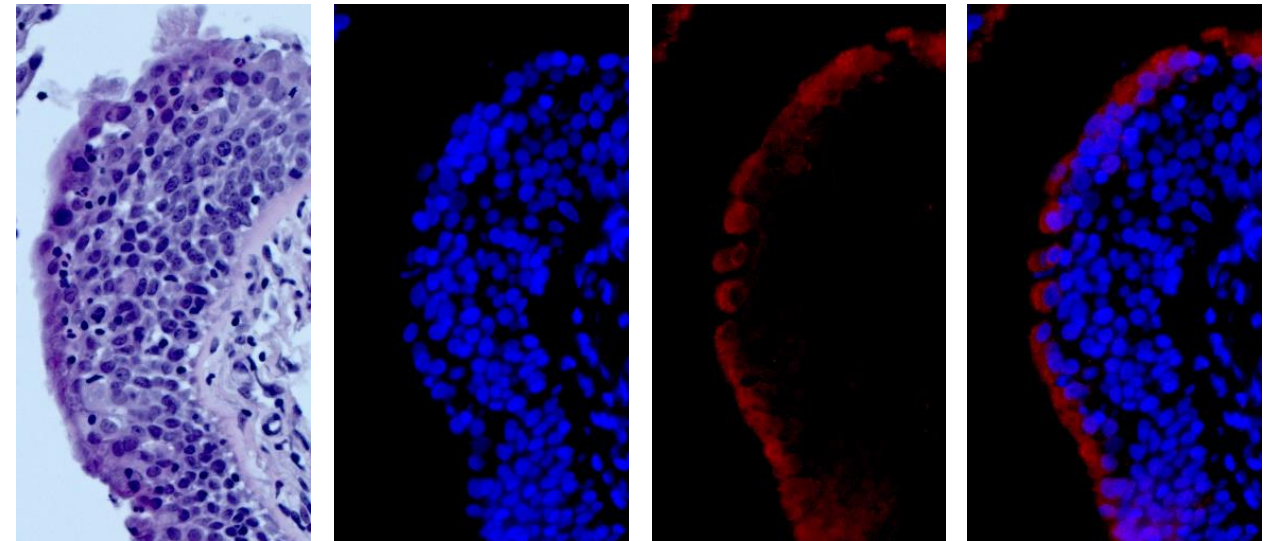
Modulator  
Ineligible CF  
Patients

**20K**

CF Patients with  
Suboptimal Modulator  
Responses

## Successful Delivery and Expression of CFTR Confirmed in Class I CF Patient Lungs

### Representative Image



H&E

DAPI

CFTR

Merge

**29% to 42%**

Conducting Airway Cells  
Transduced with KB407  
(n = 6 CF patients)

O'Sullivan BP, et al. *Lancet* 2009;373:1891-904; Elborn JS, et al. *Lancet* 2016; 388:2519-31; Sanders DB, et al. *Pediatr Clin North Am.* 2016;63:567-84; Stoltz DA, et al. *N Engl J Med.* 2015, 372 (4): 351-362; Cystic Fibrosis Foundation (2022) Patient Registry Annual Data Report; Hapnadak SG, et al. *J Cyst Fibros.* 2020;19(3):344-354

Krystal estimates based on CFF Patient Registries 2019 and 2022, ECFS Patient Registry 2018; Middleton PG, et al. *N Engl J Med.* 2009;381:1809-1819; Heijerman HG, et al. *Lancet* 2019;394:1940-1948; Trikafta® FDA Label, Revised 10/2021

Krystal Biotech, Data on File.

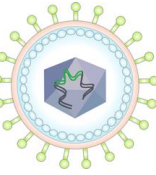
CF, cystic fibrosis, CFTR, cystic fibrosis transmembrane conductance regulator; HSV-1, herpes simplex virus 1

# KB111 for the Treatment of Hailey-Hailey Disease

Registrational study expected to start in 2H 2026

KB111

Krystal's HSV-1 vector  
encoding *ATP2C1* for  
topical application to skin



- Hailey-Hailey disease (HHD) is a rare monogenic disease characterized by painful rash and blistering in skin folds
- Prevalence not well characterized and may be underreported, estimated at 1 per 50,000
- HHD patients report high levels of psychological distress and severe impacts on quality of life, intimacy issues, as well as pain, itch, burning, body odor, and infections
- Disease is linked to mutations in the *ATP2C1* gene and low expression levels of calcium-transporting ATPase ATP2C1 in keratinocytes
- **No** specific therapy approved by the FDA or EMA for the treatment of HHD
- KB111 builds on Krystal's know-how and VYJUVEK experience in skin gene delivery to increase ATP2C1 levels in skin cells after topical administration to HHD lesions
- Delivery of functional ATP2C1 confirmed preclinically and IND cleared in October

**10K-15K**  
Estimated HHD Patients  
in the U.S. and Europe\*

\*Based on 1:50K prevalence estimates

Nmezi B et al., Poster #0554 at the 2025 Society for Investigative Dermatology (SID) Annual Meeting; Krystal Biotech, Data on File.

Konstantinou MP, et al. StatPearls [Internet]. 2022. <https://www.ncbi.nlm.nih.gov/books/NBK585136> [accessed October 30 2025]; Micaroni M, et al. *Cell Death Dis.* 2016. 7: e2259; Hu Z, et al. *Nat Genet.* 2000. 24: 61-65; Sudbrak R, et al. *Hum Mol Genet.* 2000. 9: 1131-1140; Shibata A, et al. *Acta Derm Venereol.* 2013. 93: 719-720; Fairclough RJ, et al. *J Invest Dermatol.* 2004. 123: 67-71; Foggia L, et al. *Am J Med Genet C Semin Med Genet.* 2004. 131C: 20-31

ATP2C1, calcium-transporting ATPase type 2C member 1; HSV-1, herpes simplex virus 1; IND, investigational new drug; U.S., United States

# Clear Path to Building Shareholder Value in 2026 and Beyond

## Global VYJUVEK Launch Execution



- **Launch VYJUVEK in at least one more major European market**
- **Expand specialty distributor network to cover over 40 countries**

## Clinical Pipeline Execution and Readouts

- KB803 Phase 3 readout for corneal abrasions in DEB patients
  - KB801 registrational readout for neurotrophic keratitis
  - Start and fully enroll registrational KB407 repeat dosing study for CF
  - Initiate registrational study of KB111 for Hailey-Hailey disease
- + updates for KB408 in AATD and inhaled KB707 for NSCLC



Developing Genetic Medicines to Treat Diseases with High Unmet Medical Needs