

BioCryst Pharmaceuticals, Inc.

J.P. Morgan Healthcare Conference

January 12, 2026



Forward-looking statements

This presentation contains forward-looking statements, including statements regarding, among other things, preliminary, unaudited results and future results, performance or achievements, expectations regarding pipeline development, including timing of clinical trial results, expectations regarding BioCryst's growth and expenses, the expected benefits of BioCryst's acquisition of Astria (the "Merger") and BioCryst's ability to recognize the benefits of the Merger, expected Merger consideration, the anticipated financial impact of the Merger, BioCryst's or the combined company's performance following the Merger, including future financial and operating results, anticipated approval and commercialization of navenibart, pharmaceutical research and development, such as drug discovery, preclinical and clinical development activities and related timelines, and expected HAE portfolio revenue growth and addressable market, anticipated benefits, performance, and competitive positioning of, and market size for, navenibart and BCX17725, potential best-in-class or first-in-class profile of product candidates (including navenibart and BCX17725), expectations regarding ORLADEYO peak revenue, and BioCryst's plans, objectives, expectations, intentions, growth strategies and other statements that are not historical facts. These statements involve known and unknown risks, uncertainties and other factors which may cause BioCryst's actual results, performance, or achievements to be materially different from any preliminary, unaudited results and future results, performance, or achievements expressed or implied by the forward-looking statements. These statements reflect our current views with respect to future events and are based on assumptions and are subject to risks and uncertainties. Given these uncertainties, you should not place undue reliance on these forward-looking statements.

Some of the factors that could affect the forward-looking statements contained herein include: BioCryst's ability to successfully implement or maintain its commercialization plans for ORLADEYO; BioCryst's ability to successfully progress its pipeline development plans as described herein, including meeting the expected timelines; the results of BioCryst's partnerships with third parties may not meet BioCryst's current expectations; risks related to government actions, including that decisions and other actions, including as they relate to pricing, may not be taken when expected or at all, or that the outcomes of such decisions and other actions may not be in line with BioCryst's current expectations; the commercial viability of ORLADEYO, including its ability to achieve sustained market acceptance and demand; ongoing and future preclinical and clinical development of product candidates may take longer than expected and may not have positive results; the outcome of preclinical testing and early clinical trials may not be predictive of the success of later clinical trials, and interim results of a clinical trial do not necessarily predict final results; BioCryst may not be able to enroll the required number of subjects in planned clinical trials of product candidates; BioCryst may not advance human clinical trials with product candidates as expected; the FDA or other applicable regulatory agency may require additional studies beyond the studies planned for products and product candidates, may not provide regulatory clearances which may result in delay of planned clinical trials, may not review regulatory filings on our expected timeline, may impose certain restrictions, warnings, or other requirements on products and product candidates, may impose a clinical hold with respect to product candidates, or may withhold, delay or withdraw market approval for products and product candidates; product candidates, if approved, may not achieve market acceptance; BioCryst's ability to successfully commercialize its products and product candidates; BioCryst's ability to successfully manage its growth and compete effectively; timing for achieving or sustainability of profitability and positive cash flow may not meet management's expectations; statements and projections regarding financial guidance and goals and the attainment of such goals may differ from actual results or may not be achieved on the expected timelines, or at all, based on market factors and BioCryst's ability to execute its operational and budget plans; actual financial results may not be consistent with expectations, including that revenue, operating expenses and cash usage may not be within management's expected ranges; the occurrence of any event, change or other circumstances that could give rise to the right of BioCryst or Astria to terminate the definitive agreement governing the Merger; the failure to obtain Astria stockholder approval or to satisfy any of the other conditions to the Merger on a timely basis or at all; the possibility that the anticipated benefits of the Merger, including anticipated synergies, are not realized when expected or at all, including as a result of the impact of, or problems arising from, the integration of the two companies or as a result of the strength of the economy and competitive factors in the areas where BioCryst and Astria do business; the significant indebtedness BioCryst expects to incur in connection with the Merger and the need to generate sufficient cash flows to service and repay such debt; the possibility that the Merger may be more expensive to complete than anticipated; diversion of management's attention from ongoing business operations and opportunities; potential adverse reactions or changes to business or employee relationships, including those resulting from the completion of the Merger; and risks relating to the potential dilutive effect of shares of BioCryst common stock to be issued in the Merger.

Please refer to the documents BioCryst files periodically with the Securities and Exchange Commission, specifically BioCryst's most recent Annual Report on Form 10-K, Quarterly Reports on Form 10-Q, and Current Reports on Form 8-K, which identify important factors that could cause actual results to differ materially from those contained in BioCryst's projections and forward-looking statements.

Non-GAAP Financial Measures

This presentation includes non-GAAP financial measures that differ from measures calculated in accordance with generally accepted accounting principles in the United States of America (“GAAP”), including financial measures labeled as “non-GAAP.” We believe providing these non-GAAP measures, which show our results with these items adjusted, is valuable and useful since they allow management and investors to better understand the company’s financial performance in the absence of certain non-cash items such as stock-based compensation and certain special events and allow investors to more accurately understand our current and past period results and more easily compare them to future results. These non-GAAP measures also correspond with the way we expect investors and financial analysts to compare our results. Our non-GAAP measures should be considered only as supplements to, and not as substitutes for or in isolation from, our other measures of financial information prepared in accordance with GAAP, such as GAAP revenue or operating income.

Our references to the “non-GAAP” financial measure of preliminary, unaudited 2025 ORLADEYO revenue, excluding European ORLADEYO revenue for the full year 2025, constitutes a non-GAAP financial measure. It refers to our preliminary GAAP results, adjusted to show the results without including \$38 million of European ORLADEYO revenue for the nine months ended September 30, 2025.

We also provide our non-GAAP operating expense outlook for full year 2026, which refers to our expected GAAP operating expense, excluding stock-based compensation, restructuring and transaction-related costs. We have not provided a reconciliation against the comparable forward-looking GAAP measure because we are unable to predict with reasonable certainty the full amount of stock-based compensation expense or restructuring and transaction-related costs for the full year 2026 without unreasonable effort. Stock-based compensation expense is uncertain and depends on various factors, including our future hiring and retention needs, as well as the future fair market value of our common stock, which is difficult to predict and subject to change. In addition, we are unable to predict with reasonable certainty the full amount of restructuring and transaction-related costs as the closing of the proposed Astria acquisition is still pending and the related costs are dependent on various factors that have not yet occurred. The actual amount of stock-based compensation, restructuring and transaction-related costs for the full year 2026 could have a material impact on GAAP reported results for the guidance period.

What BioCryst accomplished in 2025:



ORLADEYO demand growth

2025 was the strongest year since launch for new patient prescriptions



Paid rate improvement

Paid rate reached 84% at end of Q1 and 81% at YE; approaching long-term goal of 85%¹



European sale

Sale of EU ORLADEYO business at compelling valuation; streamlines operations, improves margins



Business Development

Proposed Astria acquisition strengthens HAE portfolio with an attractive Phase III asset in navenibart



Profitability

Achieved first full year of profitability



Peds approval

Received FDA approval for ORLADEYO pellet formulation for patients 2 to <12 with HAE

1. Paid rate calculation does not include patients on Quick Start program

Strong Q4 and 2026 outlook

PRELIMINARY RESULTS <i>(in millions)</i>	Q4 2025	FY 2025
ORLADEYO revenue	\$151	\$601
ORLADEYO revenue, excluding EU ¹		\$563
Cash, cash equivalents, restricted cash & investments at YE		\$338

2026 GUIDANCE <i>(in millions)</i>	As of Jan 12, 2026
ORLADEYO revenue ¹	\$625-645
Total revenue ¹	\$635-660
Non-GAAP operating expense ²	\$380-390
Non-GAAP operating expense including Astria acquisition ²	\$450-470

Commercial Highlights

- 2025 was the strongest year since launch for new patient prescriptions
- Continued strong demand despite new market entrants
- Over 1,600 US patients on therapy at YE 2025
- Continued growth in new prescribers

1. Excludes EU ORLADEYO revenues (BioCryst divested its European ORLADEYO business in Q4 2025).

2. Excludes stock-based compensation, restructuring, and transaction-related costs.

BioCryst: delivering sustainable growth in rare disease

Value creation through
three key strategic growth pillars

Commercial Product

Steady growth with
high cash flow visibility

- Sustainable \$1B peak revenue opportunity for ORLADEYO
- >80% contribution margin¹
- IP runway into 2040²

Internal R&D

Maximizing potential of
rare disease pipeline

- Netherton syndrome: high unmet need and potential for best-in-class therapy
- Targeted rare disease-focused discovery
- Externalizing non-core assets

External Opportunities

Via strategic business
development

- Focus on de-risked late-stage rare disease assets
- Near-term value creation
- Leveraging existing operating infrastructure

1. Contribution margin defined as revenue minus direct costs (COGS + S&M)
2. Pediatric extension through May 2040; composition of matter patent

ORLADEYO: the first and only approved targeted oral for HAE prophylaxis

- ORLADEYO (berotralstat) is a plasma kallikrein inhibitor indicated for prophylaxis to prevent attacks of HAE in patients ages 2+
- Discovered in BioCryst labs
- Approved in US Dec 2020; now in 6th year of launch
- Pediatric formulation (pellets) approved Dec 12, 2025
- IP through May 2040¹



>1,600
patients on therapy²

>3,500
patients have tried since launch

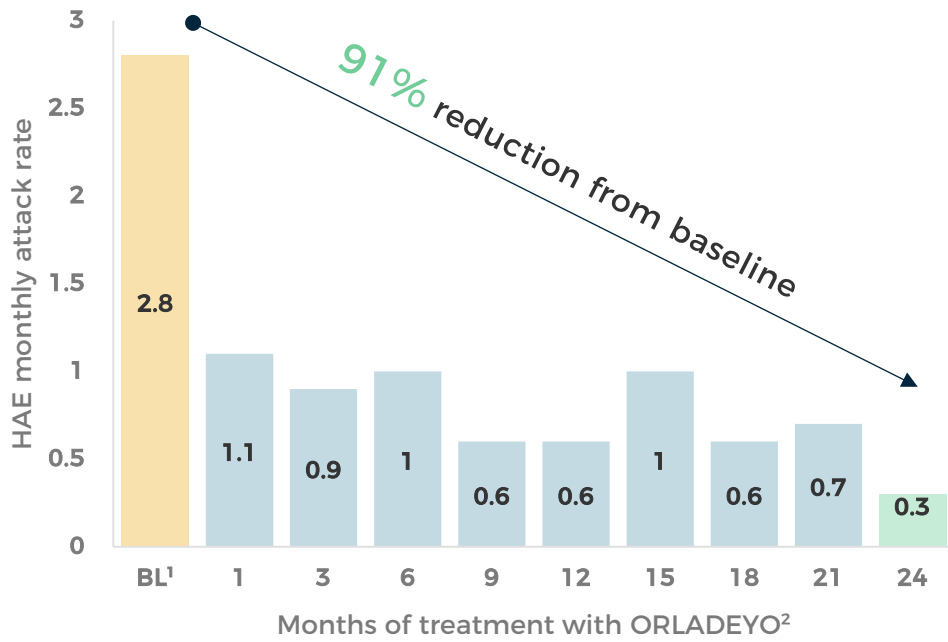
>1,500
unique prescribers

(figures reflect ORLADEYO metrics in US market)

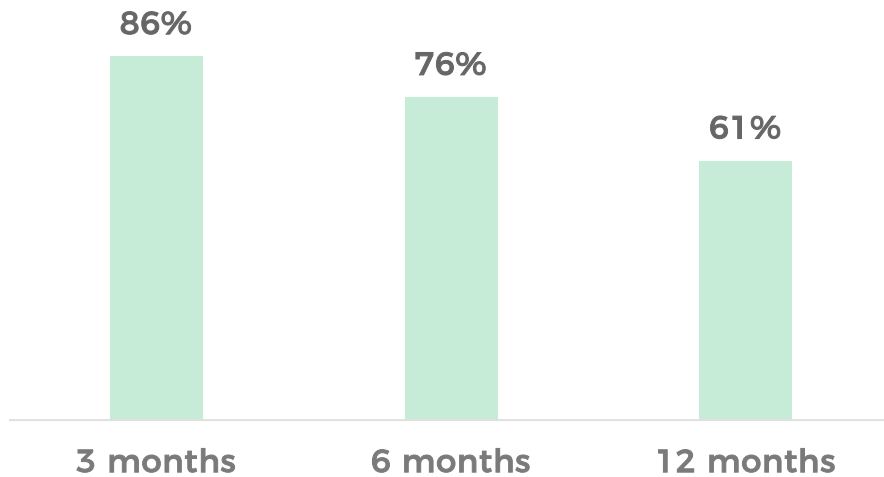
1. Pediatric extension through May 2040; composition of matter patent
2. All patients including Quick Start, paid, and patient assistance program (PAP)

ORLADEYO offers proven long-term attack control, demonstrated by strong real-world efficacy data

Great efficacy + differentiated convenience



Strong retention over time
(% of patients persistent at various time points)³

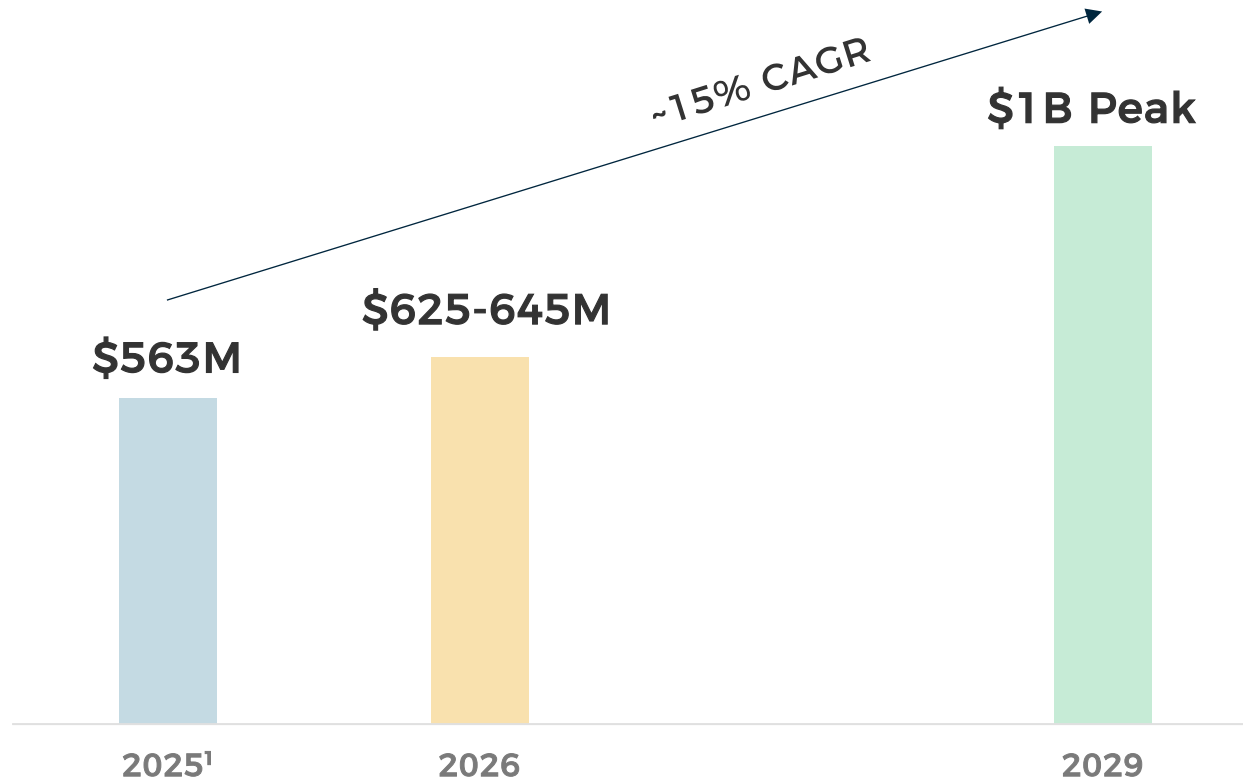


Leads to →

ORLADEYO efficacy is consistent with ~80-90% reduction in attack rate provided by injectable therapies

1. BL: baseline
 2. Data from APeX-2 open label extension study; 150mg, n=21 completers; Kiani-Alikhan S, et al. J Allergy Clin Immunol Pract. 2024;12(3):733-743.e10.
 3. Zuraw BL, et al. Allergy Asthma Proc. 2025 May 1;46(3):209-217. Persistence is defined as having no gap in treatment ≥45 days after the treatment initiation date.

ORLADEYO: highly achievable path to peak



Key drivers to \$1B peak

- ~150 net patient adds per year (adult + pediatric)
- Paid rate improvement toward 85% at YE 2029 vs. 81% at YE 2025²
- Modest annual price increases
- Contribution from ex-US geographies

1. Preliminary, unaudited revenue excluding EU for FY 2025
2. Paid rate calculation does not include patients on Quick Start program

BioCryst to acquire Astria for ~\$700M TEV

Addition of navenibart to expand and strengthen presence in HAE while transforming growth profile

Strong strategic fit

- ✓ **10+ yr double digit portfolio CAGR**

Potential to transform BioCryst's revenue profile through the next decade

- ✓ **Near-term launch anticipated**

Pivotal Phase 3 clinical trial on track for early 2027 topline results

- ✓ **Core area of expertise**

Seamlessly integrates into and complements BioCryst's existing HAE franchise

Compelling LTP asset

- ✓ **Differentiated injectable profile**

3-to-6-month dosing would be a significant improvement over available injectable options

- ✓ **Late-stage asset with strong efficacy, safety, and tolerability**

Phase 1 b/2 data indicates potential for best-in-class efficacy with favorable safety profile

- ✓ **Simple, well-understood mechanism**

Patients and physicians have long experience with plasma kallikrein inhibition

Enhances financial profile

- ✓ **Profitability maintained**

BioCryst expects to remain profitable (non-GAAP) and cash flow positive post-transaction

- ✓ **Significant operating leverage**

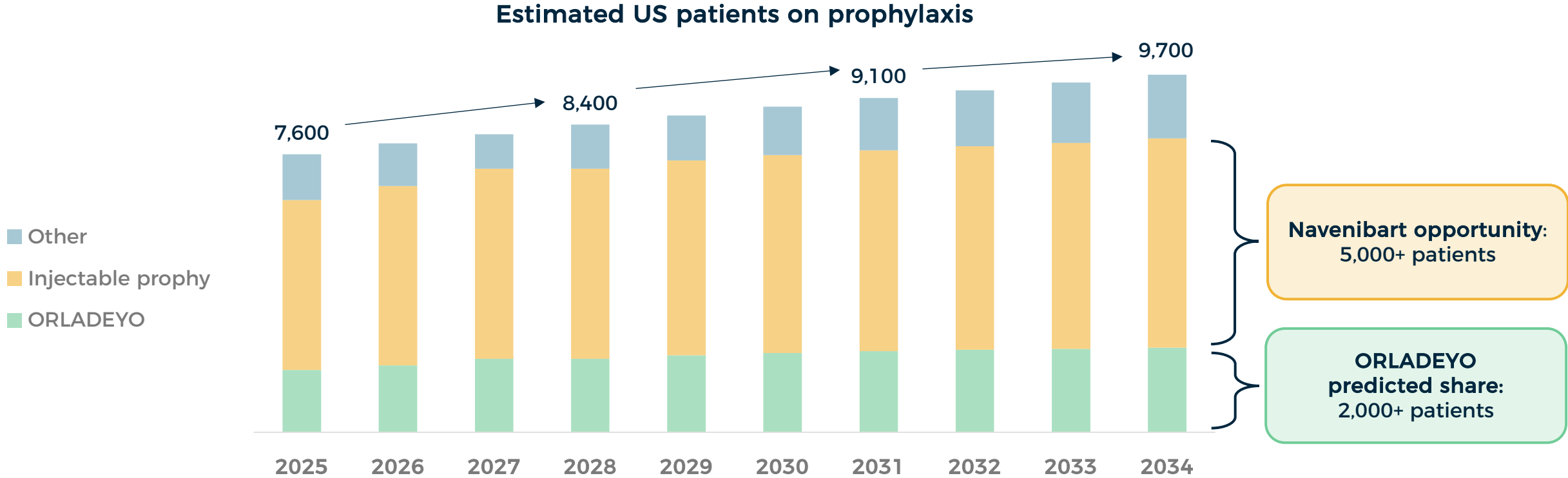
Driven by BioCryst's leading commercialization infrastructure

- ✓ **Strong cash flow generation**

Expected cash balance of \$1B+ by 2029, enabling optionality for other growth opportunities

Significant addressable opportunity in HAE

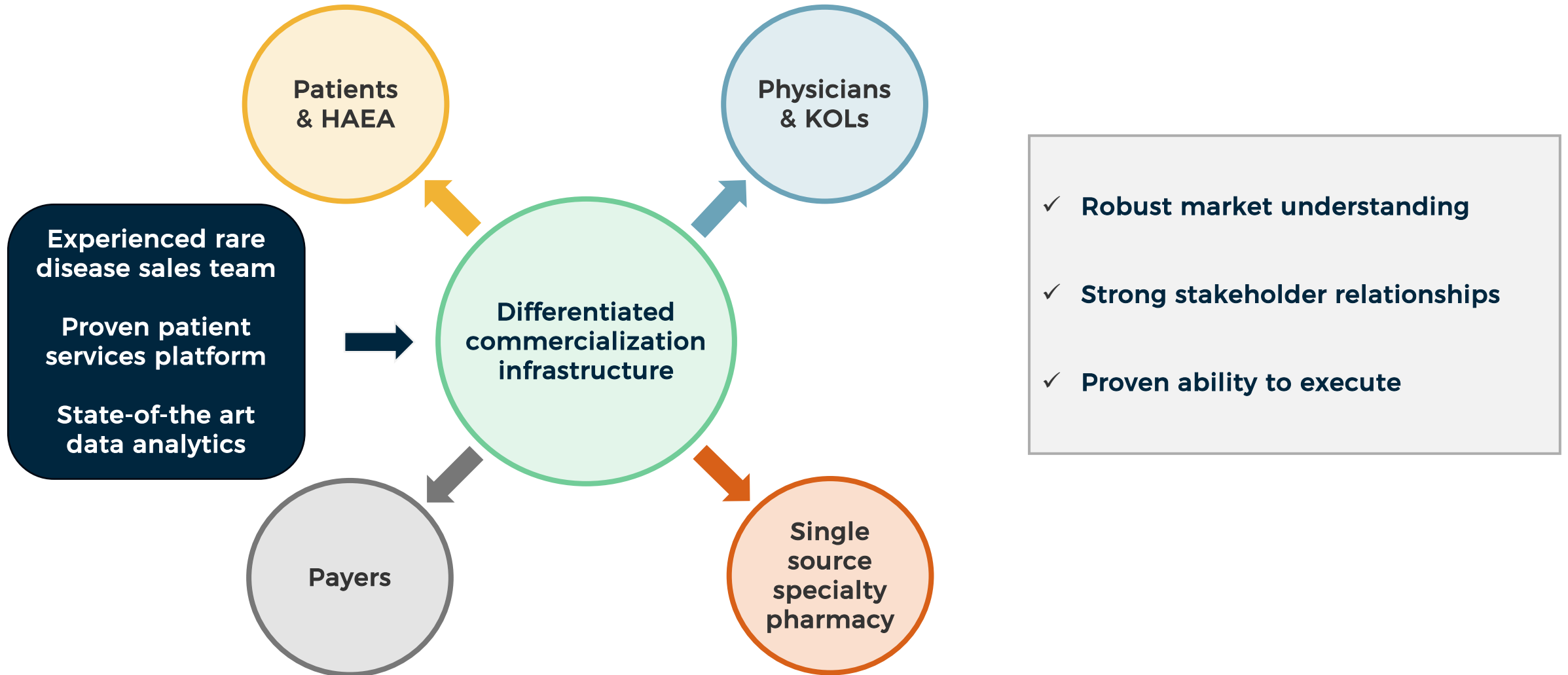
Astria deal to augment HAE portfolio with the potential best-in-class option for any route of administration preference



Source: BioCryst Internal Market Research Study (Conducted Jun 2025), 2018-2023 administrative claims data

BioCryst's commercial footprint primed for next product

Commercial strategy for ORLADEYO is a repeatable playbook for additional products



Navenibart could become the 1st choice injectable therapy



✓ Trusted mechanism & modality

Monoclonal antibody inhibitor of plasma kallikrein



✓ Compelling efficacy data

High affinity and potency with fast onset delivers rapid, effective prevention against attacks



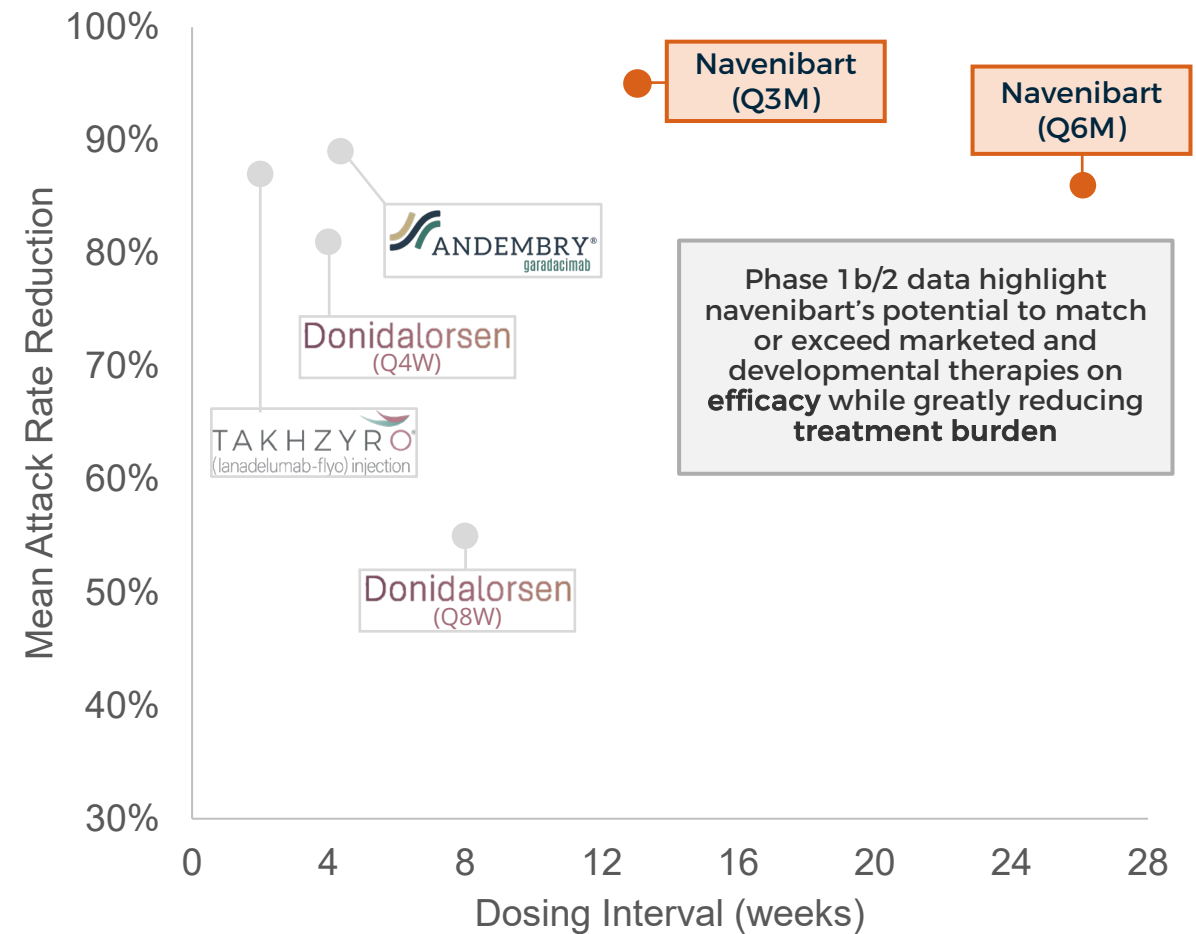
✓ Infrequent dosing schedule

YTE modification for extended half-life enables dosing every 3 or 6 months



✓ Pain-free administration

Citrate-free, high-concentration formulation, delivered via autoinjector

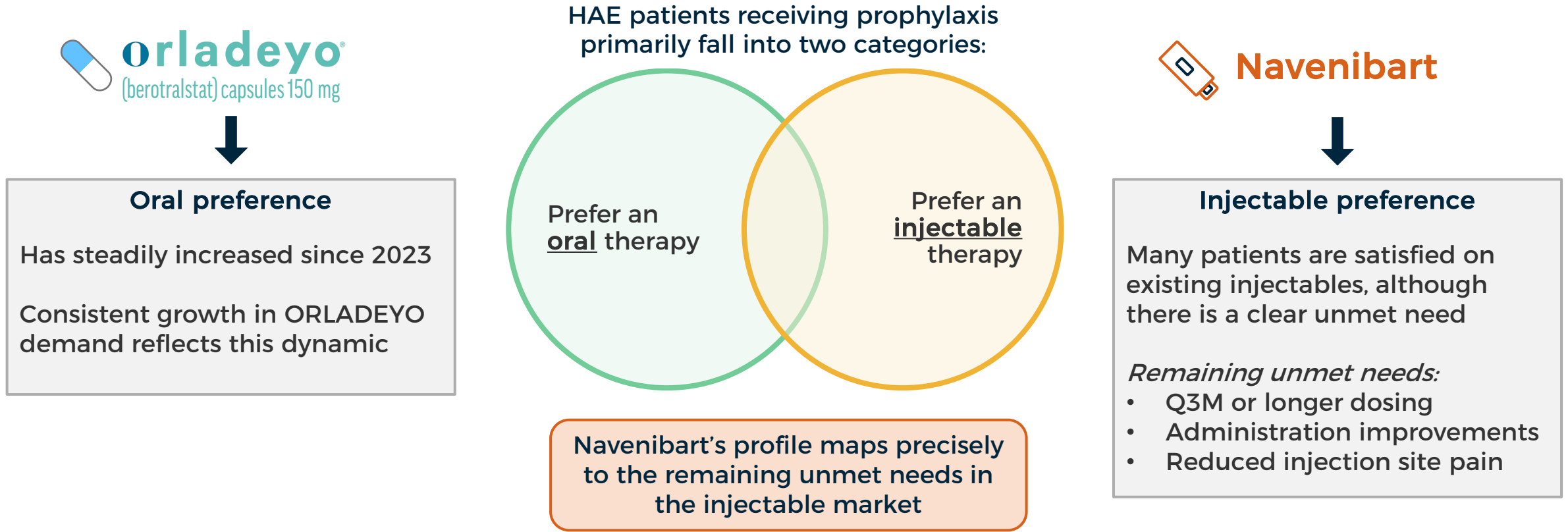


Q3M/Q6M, 3/6-month dosing

NOTE: Efficacy data presented are derived from different clinical trials conducted at different times by different sponsors, with differences in trial design and patient populations. As a result, cross-trial comparisons cannot be made, and no head-to-head clinical trials have been conducted. ANDEMBRY: US Prescribing Information (Jun 2025). TAKHZYRO: US Prescribing Information (Jan 2025). Donidalorsen: Riedl et al (2024), NEJM. Navenibart data is from the ALPHA-SOLAR study in which Arm A consisted of D1 600 mg, then 300 mg Q3M (n=10) and Arm B consisted of D1 600mg, D28 600 mg, then 600 mg Q6M (n=6).

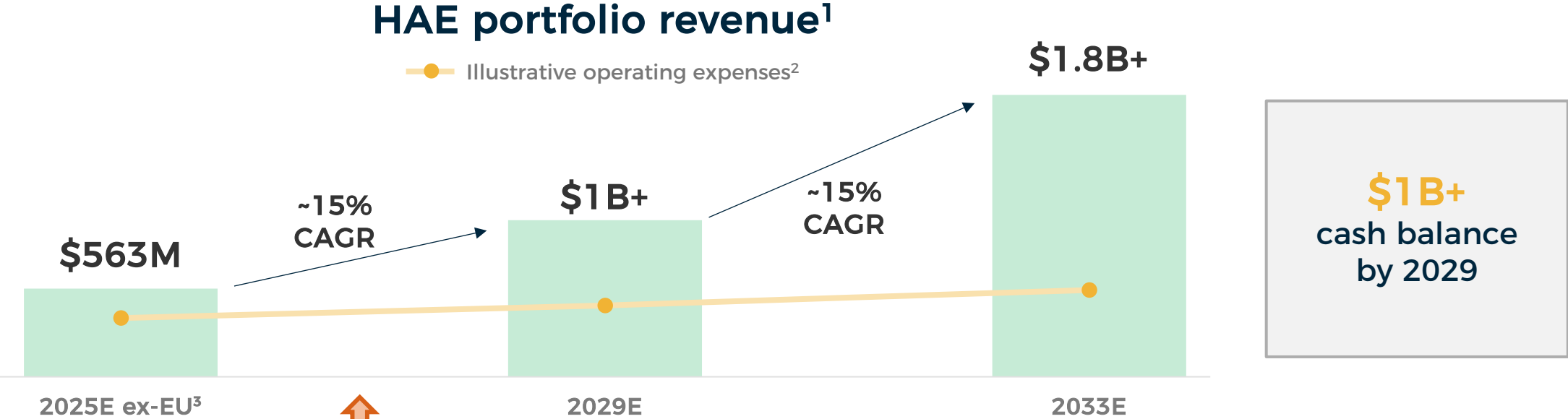
Positioned to offer leading oral and injectable therapies

Combined portfolio to reach distinct, durable, and growing segments, covering full spectrum of patient preference

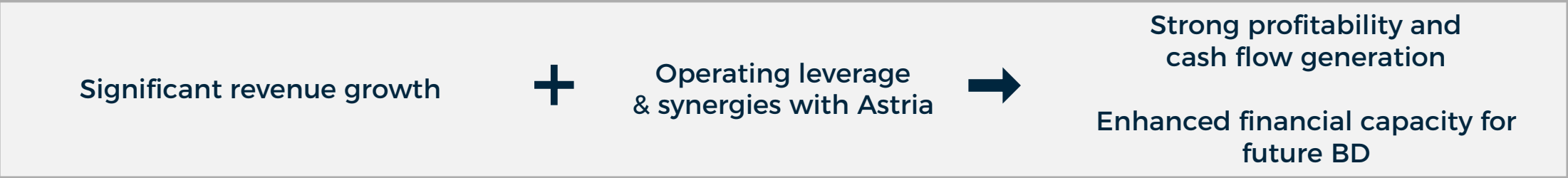


Opportunity to offer the most patient-friendly options, optimally serving the HAE patient community

BioCryst HAE franchise expected to generate double digit revenue growth into the next decade



↑
Early 2027 - navenibart pivotal topline results expected



1. Implied projections for navenibart based on Wall Street analyst research
 2. Non-GAAP operating expenses expected to grow at a mid-single digit CAGR
 3. Preliminary, unaudited revenue excluding EU for FY 2025

Our pipeline

ASSET	PROGRAM	PRE-CLINICAL	PHASE 1	PHASE 2	PHASE 3/PIVOTAL	APPROVED / COMMERCIAL
CORE PROGRAMS						
ORLADEYO® (berotralstat) Oral Plasma Kallikrein Inhibitor	Hereditary Angioedema (HAE)	[Green arrow indicating progression through Pre-clinical, Phase 1, Phase 2, and Phase 3/Pivotal]				
ORLADEYO® (berotralstat) Oral Plasma Kallikrein Inhibitor in Pediatrics	Hereditary Angioedema (HAE)	[Green arrow indicating progression through Pre-clinical, Phase 1, Phase 2, and Phase 3/Pivotal]				
BCX17725 Protein Therapeutic	Netherton Syndrome	[Orange arrow indicating progression through Pre-clinical and Phase 1]				
Undisclosed	Rare Diseases	[Yellow arrow indicating progression through Pre-clinical]				
NON-CORE PROGRAMS						
RAPIVAB® (peramivir injection)	Infectious Diseases	[Green arrow indicating progression through Pre-clinical, Phase 1, Phase 2, and Phase 3/Pivotal]				
Avoralstat Ocular Plasma Kallikrein Inhibitor	Diabetic Macular Edema (DME)	[Orange arrow indicating progression through Pre-clinical and Phase 1]				

**BCX17725 and avoralstat are investigational and have not been deemed safe and effective by the FDA.*

BCX17725: a targeted KLK5 inhibitor for Netherton syndrome (NS)

What is Netherton syndrome?

- A severe, rare, genetic disorder with widespread skin involvement and systemic complications
- Causes premature separation of skin layers, severe inflammation, and infection risk
- Diagnosed US population of ~1,600¹ with potential to grow to 3,000-5,000 with greater diagnosis and treatment
- No approved targeted therapies



Why Netherton syndrome?

Aligned with core rare disease focus

- High unmet need
- Potential for market expansion over time due to misdiagnosis or underdiagnosis
- Prescriber landscape comparable to HAE's: addressable by small sales team
- In-house analytics expertise primed to aid diagnosis and market development

Clear biology

- Validated target and known cause of disease: *SPINK5* gene variant causes KLK5 overactivity
- BCX17725 is a systemically-administered KLK5 inhibitor

Potential for BioCryst to be first mover

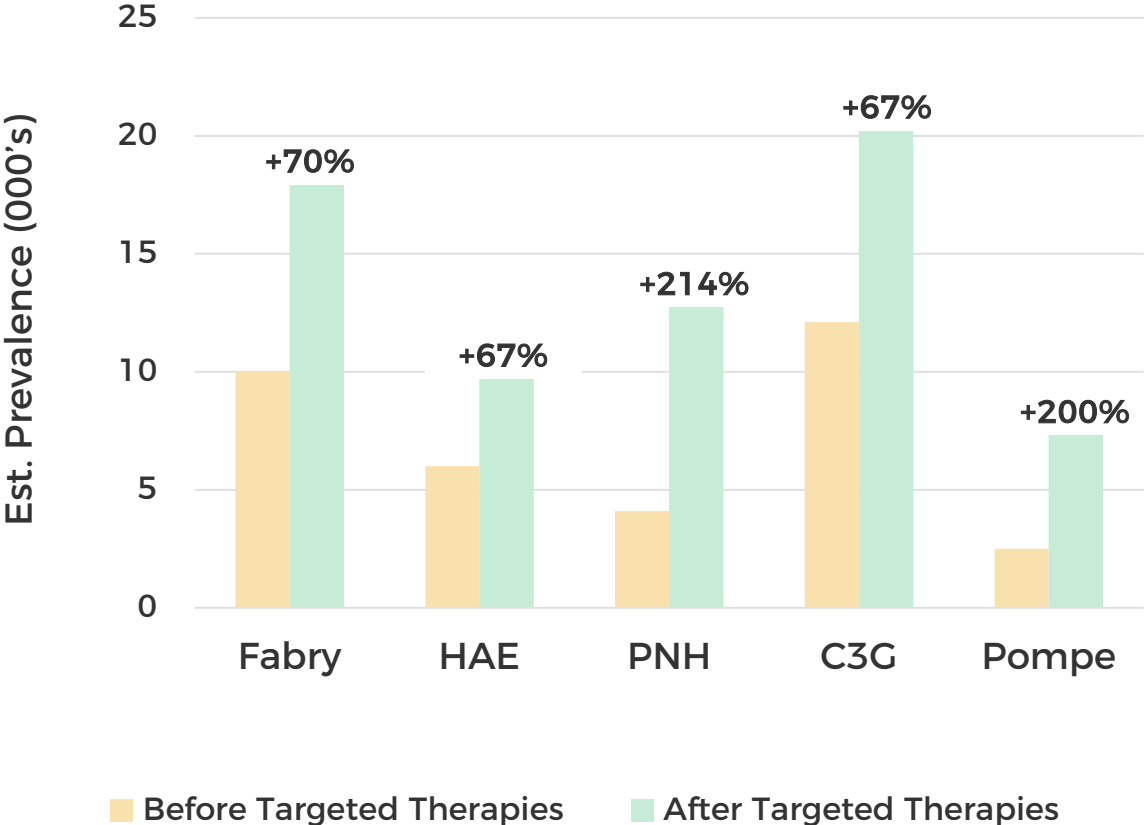
- BCX17725 could be the first-in-class targeted systemic therapy

1. Based on healthcare claims analysis

Image: <https://www.nethertonsyndrome.com/about-nethertons.php>

Targeted therapies drive diagnosis of rare diseases

Change in US Prevalence After Launch of Targeted Therapy



Netherton Syndrome Population Assumptions

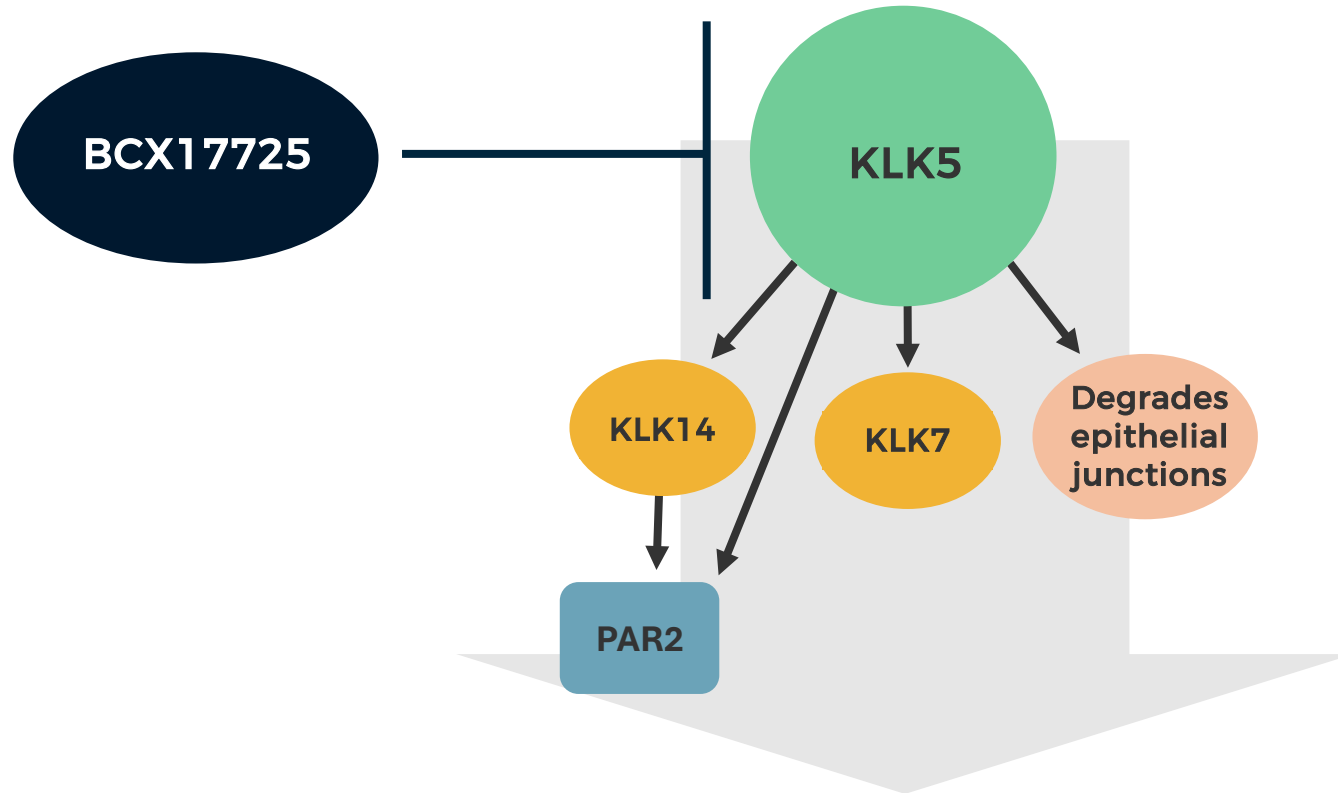
Initial healthcare claims analysis indicates a diagnosed US prevalence of ~1,600



Ongoing analysis leveraging NLP models, EMR data, and rare disease analogs suggest potential for US diagnosed population to increase to greater than 3,000

HAE, hereditary angioedema; PNH, paroxysmal nocturnal hemoglobinuria; C3G, Complement 3 glomerulopathy; NLP, natural language processing; EMR, electronic medical records
Sources: Cantor Analysis 2024; Internal Analysis

BCX17725 targets KLK5, the key player in Netherton syndrome



- KLK5 initiates the pathologic protease cascade (KLK7, KLK14) and inflammation (via PAR2) in the skin
- BCX17725 designed to stop KLK5 overactivity at the top of the pathway

Downstream consequences of KLK5 activation

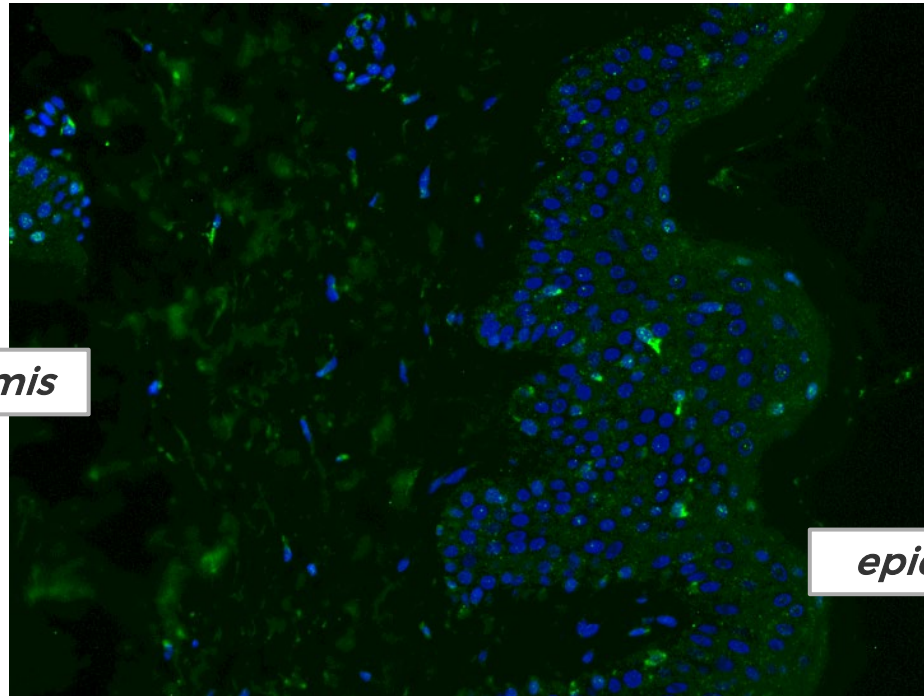
Skin barrier dysfunction

Inflammatory cascade

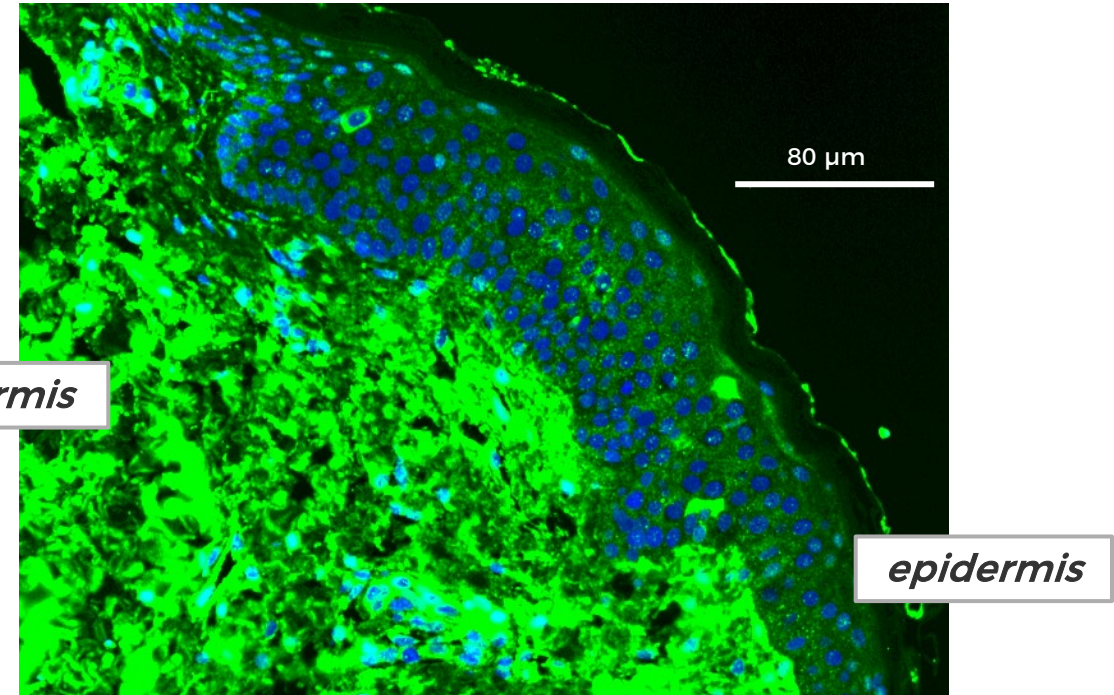
Atopy

BCX17725 in healthy volunteers: encouraging distribution to epidermis after IV dosing

Pre-dose



Post-dose: **BCX17725** 12 mg/kg IV



Notes:
Immunofluorescence (IF) using capture antibody specific to BCX17725 and fluor-tagged (green) detection antibody.
Cell nuclei - DAPI fluor (DNA, blue).
Post-dose samples obtained five hours after third dose.

BCX17725 Phase 1 study design

Parts 1 & 2:
Healthy
volunteers

SAD

☑ Completed

MAD

☑ Completed

Part 3:

NS patients
N = 1-3
anticipated

Multiple Dose

4 weeks of treatment, open-label, safety and PK

Part 4:

NS patients
N = up to 12

Multiple Dose

12 weeks of treatment, open-label, safety and efficacy

This study is designed to help us understand:

1. Preliminary safety
2. Systemic exposure
3. Distribution into skin
4. Early efficacy signals
5. Dosing for pivotal study

Data from Part 4 expected by YE 2026

Goal of study: inform and plan for pivotal study in 2027

Key upcoming milestones

January 2026

Acquisition of Astria Therapeutics: expected close

YE 2026

BCX17725 in NS: Proof of concept results

Early 2027

Navenibart in HAE: Pivotal topline results

BioCryst Pharmaceuticals, Inc.

J.P. Morgan Healthcare Conference

January 12, 2026

