

Third Quarter 2024 Results Call

Corporate Update & Financial Results

November 4, 2024



Forward-looking statements

BioCryst's presentation contains forward-looking statements, including statements regarding future results, unaudited and forward-looking financial information and company performance, achievements, or future market share. These statements are subject to known and unknown risks, uncertainties and other factors which may cause our actual results, performance, achievements, or market share to be materially different from any future results, performance, achievements, or market share expressed or implied in this presentation. These statements reflect our current views with respect to future events and are based on assumptions and are subject to risks and uncertainties.

You should not place undue reliance on the forward-looking statements. For additional information, including important risk factors, please refer to BioCryst's documents filed with the SEC and located at ir.biocryst.com/financial-information/sec-filings.

AGENDA

Corporate update

Jon Stonehouse
President and Chief Executive Officer

ORLADEYO® update

Charlie Gayer
Chief Commercial Officer

R&D update

Dr. Helen Thackray
Chief Research and Development Officer

Financial update

Anthony Doyle
Chief Financial Officer

Q&A

Strong ORLADEYO growth and business progress continues in Q3

COMMERCIAL

- ORLADEYO revenue of \$116.3m (+35.7% y/y)
- Full year ORLADEYO guidance raised to \$430-435m (from \$420-435m)
- Introduced total product revenue guidance \$443-448m (including additional 2024 RAPIVAB revenue)

CLINICAL

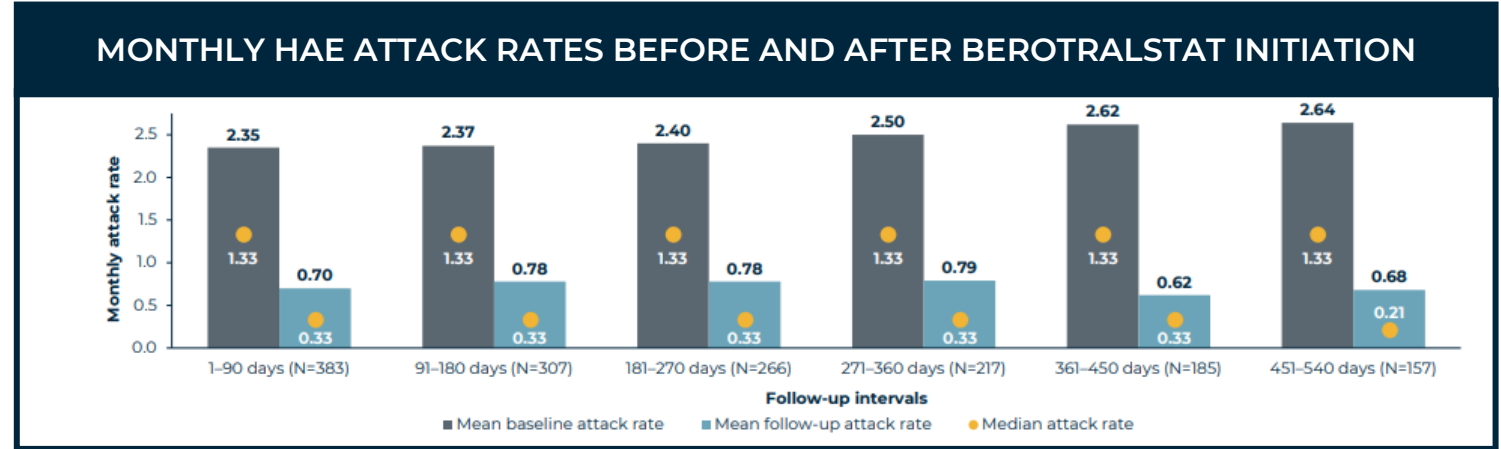
- ORLADEYO pediatric study APeX-P is fully enrolled and on track for regulatory submission in 2025
- BCX17725 advanced into clinic with first enrolled participant in Phase 1 study
 - Initial patient data expected by end of 2025
- Avoralstat remains on track to enter clinic in 2025 with initial patient data by end of 2025

FINANCIAL

- Second consecutive quarter of GAAP operating profit (+\$7.7m, +\$24.9m non-GAAP)
- On track for full-year 2024 non-GAAP operating profit
- Due to our strong financial position and capital markets independence, we elected:
 - Not to draw additional \$150m tranche of Pharmakon debt
 - Not to exercise Q3 PIK option on Pharmakon interest

REAL WORLD EVIDENCE: Type 1 and 2 HAE patients have significant and sustained attack reduction on ORLADEYO

- Median attack rate of 1/3rd of an attack per month in a study population of over 450 patients



Sustained Real-World Attack Reductions Following Berotralstat Initiation Among Patients with Hereditary Angioedema with C1-Inhibitor Deficiency

Raffi Tachdjian¹, Mark Davis-Lorton², Lorena Lopez-Gonzalez³, Sean D. MacKnight⁴, Francis Laliberte⁵, Ramya Ramasubramanian⁶, Patrick Gillard⁷, William R. Lumry⁸

¹Department of Pediatrics, University of California Los Angeles, Los Angeles, CA, USA; ²ENT and Allergy Associates, Tarrytown, NY, USA; ³BioCryst Pharmaceuticals, Durham, NC, USA; ⁴Group of Analysis, Laval, Montreal, QC, Canada; ⁵Analysis Group, Inc., Los Angeles, CA, USA; ⁶Allergy and Asthma Research Associates, Dallas, TX, USA

INTRODUCTION

- Hereditary angioedema (HAE) is a rare and potentially fatal disease, characterized by recurrent and unpredictable attacks of laryngeal and/or abdominal swelling.
- Patients with HAE due to C1-inhibitor deficiency (HAE type I) have excess bradykinin production, resulting in HAE attacks.
- Berotralstat is a targeted, once-daily oral medication for the prevention of HAE attacks in patients 12 years of age or older.
- This real-world study evaluated and compared self-reported HAE attacks before and after initiation of berotralstat among patients with C1-inhibitor deficiency HAE type I in the US.

METHODS

- This retrospective real-world study used data from Optima Specialty Pharmacy, the sole dispenser of berotralstat in the US, from December 15, 2020, to January 9, 2024.
- The follow-up period spanned from the index date (the date of berotralstat dispensing) to the last berotralstat dispensing date. No patient assessment data were collected after the last berotralstat dispensing (Figure 1).

Figure 1. Pre-Post Study Design

RESULTS

The study population consisted of 466 patients with HAE type I who had berotralstat dispensing and at least one attack report at baseline and follow-up (Figure 2).

The mean age was 49 years, most patients were female (82%), and nearly half of patients resided in the South (Table 1). Patients had significantly fewer HAE attacks after berotralstat initiation with 100% follow-up interval (0.62-0.79 attacks/month) versus baseline (2.35-2.64 attacks/month) (Figure 4).

Mean monthly attack rate reduction (95% CI) was 1.71 (1.59, 1.83) at 12 months in 271-360 days interval and 1.96 (1.84, 2.08) at 18 months in 361-450 days interval both p<0.0001 (Figure 4).

Figure 2. Eligibility Criteria and Patient Disposition

Criteria	Patients who were included	Patients who were excluded
Independent dispensing for berotralstat between index date and baseline & follow-up	375 patients were included	89 patients were excluded
Patients with at least one HAE attack report at baseline and follow-up	466 patients were included	91 patients were excluded
Patients with C1-inhibitor deficiency HAE type I	466 patients were included	0 patients were excluded
Patients with at least one HAE attack report at baseline and follow-up	466 patients were included	0 patients were excluded
Patients with at least one HAE attack report at baseline and follow-up	466 patients were included	0 patients were excluded

Table 1. Demographics and Clinical Characteristics

Characteristic	Patients (N=466)	n (%)
Demographics		
Age, mean ± SD (range), years	49.3 ± 19.2 (20)	
Female, n (%)	298 (63.9)	
Geographic region, n (%)		
North	109 (23.2)	
Midwest	74 (15.9)	
South	229 (49.1)	
West	54 (11.6)	
HAE specialty, n (%)		
Allergy/immunology	143 (30.7)	
Internal medicine	112 (24.0)	
Other	211 (45.3)	

Figure 3. Monthly HAE Attack Rates (Mean and Median) Before and After Berotralstat Initiation

Figure 4. Mean Reductions in HAE Attack Rates After Berotralstat Initiation

At 3 months, attack rates decreased by 1.65 attacks/month

At 6 months, attack rates decreased by 1.59 attacks/month

At 12 months, attack rates decreased by 1.71 attacks/month

At 18 months, attack rates decreased by 1.96 attacks/month

CONCLUSION

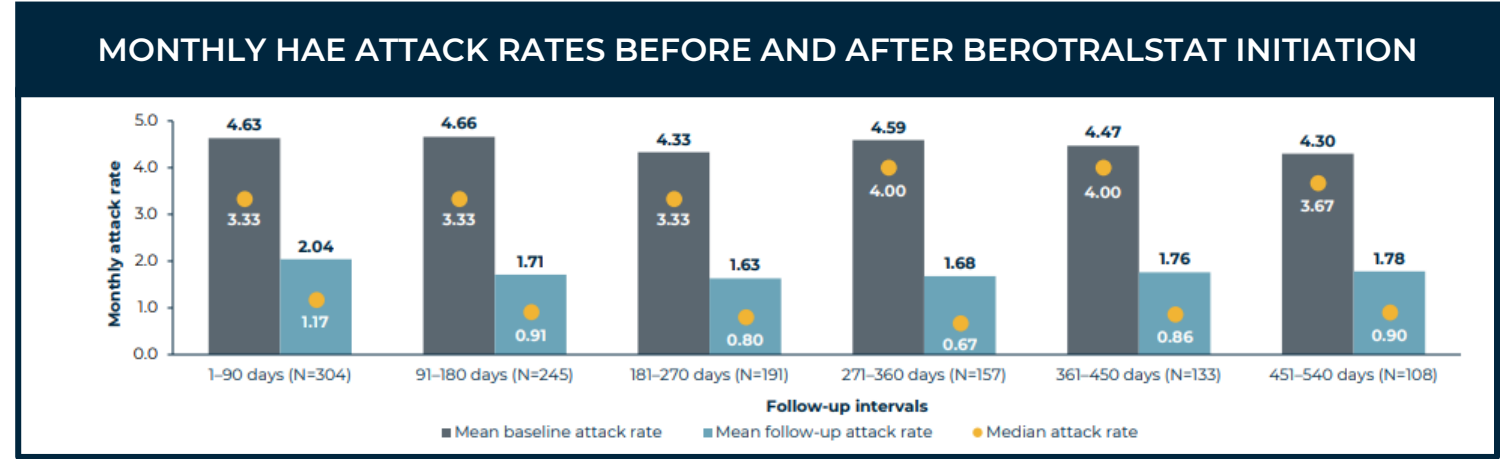
Berotralstat was associated with statistically significant and sustained reductions in HAE attack rates through 18 months following berotralstat initiation among patients with C1-inhibitor deficiency in the US.

- The primary reason for reduced patient counts over time was that patients had not been on ORLADEYO long enough to be evaluated at all time points
- Only 68 (14.6%) out of 466 patients in this study discontinued therapy

Source: Sustained Real-World Attack Reductions Following Berotralstat Initiation Among Patients with Hereditary Angioedema with C1-Inhibitor Deficiency Presented at the ACAAI Scientific Meeting 2024 · October 24-28, 2024

REAL WORLD EVIDENCE: C1 normal-inh HAE patients have significant and sustained attack reduction on ORLADEYO

- Median attack rate of <1 per month in study population of over 350 patients
- Excellent attack reduction and control for a population that has struggled to find effective therapy



Sustained Real-World Attack Reductions Following Bertralstat Initiation Among Patients with Hereditary Angioedema without C1-Inhibitor Deficiency

Mark Davis-Lorton¹, Raffi Tachdjian², Lorena Lopez-Gonzalez², Sean D. Macknight³, François Laliberté⁴, Cristina Martínez⁴, Patrick Gillard⁵, William R. Lumry⁶
¹ENT and Allergy Associates, Tarrytown, NY, USA; ²Department of Pediatrics, University of California Los Angeles, Los Angeles, CA, USA; ³BOCYPH Pharmaceuticals, Durham, NC, USA; ⁴Groupe d'Analyse, Labe, Montreal, QC, Canada; ⁵Allergy and Asthma Research Associates, Dallas, TX, USA

INTRODUCTION

- Hereditary angioedema (HAE) is characterized by recurrent and unpredictable attacks of subcutaneous and/or submucosal swelling, which can be life-threatening when affecting the upper airway.
- Some patients with HAE experience breakthrough-mediated angioedema despite normal plasma C1-inhibitor levels and function (HAE-nC1-INH).
- Bertralstat is a targeted once-daily oral medication for the prevention of HAE attacks in patients ≥12 years of age.¹
- This study evaluated and compared the real-world effectiveness of bertralstat on reducing self-reported HAE attacks among patients without C1-inhibitor deficiency (HAE-nC1-INH) in the US.

METHODS

- This retrospective real-world study used data from Optima Specialty Pharmacy, the rate dispenser of bertralstat in the US, from December 15, 2020, to January 6, 2024.
- The follow-up period was defined from the time of bertralstat dispensing initiation to the last bertralstat dispensing date; no patient assessment data were collected after the last bertralstat dispensing (Figure 1).

RESULTS

The study population consisted of 353 patients with HAE-nC1-INH with ≥2 bertralstat dispensing events at baseline and follow-up (Figure 2).

The mean age was 48 years, most patients were female (76.2%), most patients resided in the Northeast (38.5%), and nearly half of patients resided in the South (Table 1).

Patients had significantly lower HAE attack rates while on bertralstat during each 90-day follow-up interval (0.20 attacks/month) versus baseline (4.30 attacks/month) (Figure 3).

Mean monthly attack rate reduction (95% CI) was 2.91 (3.47, 2.35) at 12 months (n=157) (Figure 4).

Table 1. Demographics and Clinical Characteristics

Characteristic	Patients (n/353)	Region	N (%)
Age, mean ± SD (median), years	48.1 ± 16.8 (49)	North	108 (30.6)
Female, n (%)	271 (76.2)	South	168 (47.6)
Patient weight, mean ± SD (median), kg	84 ± 22 (82)	Midwest	81 (23.0)
HCP specialty, n (%)	332 (94.4)	West	54 (15.3)
Allergist/immunologist	22 (6.3)	Northwest	42 (11.9)
Primary care physician	38 (10.8)	Unknown	6 (1.7)
Other	23 (6.5)		

CONCLUSION

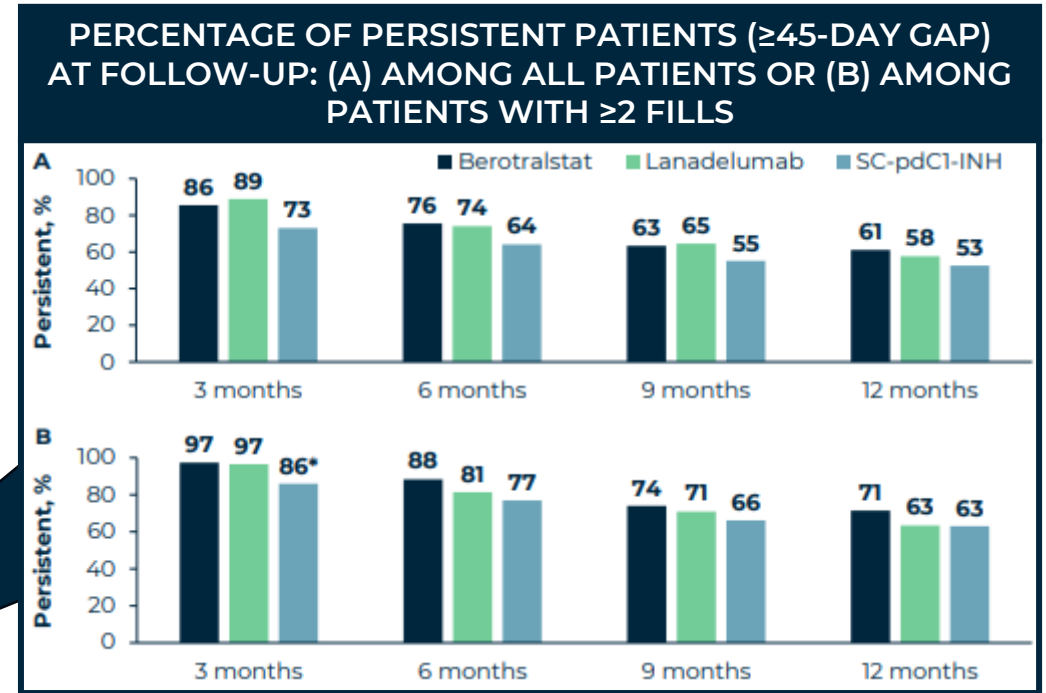
Bertralstat was associated with statistically significant and sustained reductions in HAE attack rates through 18 months following bertralstat initiation among patients without C1-inhibitor deficiency in the US.

- The primary reason for reduced patient counts over time was that patients had not been on ORLADEYO long enough to be evaluated at all time points
- Only 75 (21.2%) out of 353 patients in this study discontinued therapy

Source: Sustained Real-World Attack Reductions Following Bertralstat Initiation Among Patients with Hereditary Angioedema without C1-Inhibitor Deficiency Presented at the ACAAI Scientific Meeting 2024 • October 24-28, 2024

REAL WORLD EVIDENCE: ORLADEYO persistence is not different from other LTP products

- Adherence and persistence rates for all three LTP treatments among patients with HAE were uniformly high
- Rates of ORLADEYO adherence and persistence were comparable with those observed following lanadelumab or SC-pdC1-INH initiation



Adherence and Persistence Among Hereditary Angioedema Patients Treated With Bertralstat, Lanadelumab, and Subcutaneous Plasma-Derived C1-Inhibitor

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INTRODUCTION

- Hereditary angioedema (HAE) is a rare genetic disease characterized by potentially life-threatening recurrent swelling attacks of the extremities, face, abdomen and larynx¹.
- Bertralstat is the only targeted oral long-term prophylactic LTP treatment for HAE patients 12 years of age².
- Other targeted LTP treatments for HAE recommended by the European Academy of Allergy and Clinical Immunology, the World Allergy Organization and the United States Hereditary Angioedema Association Medical Advisory Board include lanadelumab and subcutaneous C1-inhibitor (SC-pdC1-INH)³⁻⁵.
- This study examined adherence and persistence following initiation of bertralstat, lanadelumab, and SC-pdC1-INH.

METHODS (cont'd)

Statistical Analysis

- Study measures are reported descriptively using mean and standard deviation (SD) for continuous variables and number and percent for categorical variables (Figure 2).
- P-values were calculated using chi-square tests (categorical variables) or t-tests (continuous variables).
- P-values were treated as significant if p<0.05 (Bonferroni adjustment: 0.05/3 vs comparison groups).

RESULTS (cont'd)

Treatment Retention

- Among all patients, 65% (69% of patients were adherent by PDC) (primary definition) and 62% were adherent by PDC2 (secondary definition) (Figure 2).
- Among those with ≥2 fills, PDC ranged from 76% to 80% and PDC2 ranged from 91% to 97%.
- Mean PDC1 and PDC2 were high among all patients (PDC1: 0.75-0.78; PDC2: 0.96-0.99) and among those with ≥2 fills (PDC1: 0.86-0.87; PDC2: 0.95-0.98).
- Across the 12-month follow-up, 63% of all patients and 65% of those with ≥2 fills remained persistent through the 12-month follow-up period (Figure 3).
- A sensitivity analysis which used a 45-day gap definition found that 61% of all patients and 66% of patients with ≥2 fills remained persistent through follow-up.
- A higher percentage of patients on bertralstat were persistent at 3 months compared to patients on SC-pdC1-INH.
- No other differences between cohorts were significant.

Table 1. Patient Demographics

	Bertralstat (n=90)	Lanadelumab (n=109)	SC-pdC1-INH (n=97)
Age, mean (SD), years	42.9 (12.7)	38.9 (16.2)	39.3 (14.2)
Female, n (%)	77 (81.1)	109 (73.5)	53 (57.9)
Geographic region, n (%)			
Northwest	21 (23.3)	42 (22.2)	9 (11.5)
Midwest	20 (22.2)	22 (11.6)	20 (25.6)
South	29 (32.2)	29 (15.8)	30 (44.9)
West	20 (22.2)	50 (26.5)	14 (17.9)
Other/Unknown	0 (0.0)	4 (2.1)	0 (0.0)
Payer type, n (%)			
Commercial	51 (56.7)	146 (26.3)	59 (66.8)
Medicaid	19 (21.1)	54 (28.6)	27 (34.4)
Medicare Advantage	10 (11.1)	7 (3.8)	7 (9.0)
Medicare Fee-for-Service	1 (1.1)	1 (0.5)	1 (1.3)
Other/Unknown	9 (10.0)	17 (9.2)	5 (6.4)

Table 2. Baseline Clinical Characteristics

	Bertralstat (n=90)	Lanadelumab (n=109)	SC-pdC1-INH (n=97)
HAE or angioedema codes, n (%)			
ICD-10-CM: D86.1	61 (67.8)	74 (67.9)	74 (76.3)
ICD-10-CM: D86.2	41 (45.6)	52 (47.7)	50 (51.7)
HAE signs and symptoms, n (%)			
Localized swelling/edema of breathing difficulty	50 (55.6)	111 (101.5)	59 (67.8)
Prior HAE treatment, n (%)			
LTP (any)	41 (45.6)	97 (89.3)	39 (40.0)
LTP (Bertralstat)	24 (26.7)	42 (38.5)	31 (31.9)
LTP (Lanadelumab)	0 (0.0)	42 (38.5)	0 (0.0)
LTP (SC-pdC1-INH)	0 (0.0)	0 (0.0)	31 (31.9)

Table 3. Percentage of Adherent Patients (PDC) at Follow-Up

Time Point	Bertralstat (%)	Lanadelumab (%)	SC-pdC1-INH (%)
3 months (All Patients)	86	89	73
6 months (All Patients)	76	74	64
9 months (All Patients)	63	65	55
12 months (All Patients)	61	58	53
3 months (≥2 Fills)	97	97	86*
6 months (≥2 Fills)	88	81	77
9 months (≥2 Fills)	74	71	66
12 months (≥2 Fills)	71	63	63

Table 4. Percentage of Persistent Patients (≥45-Day Gap) at Follow-Up (A) Among All Patients or (B) Among Patients With ≥2 Fills

Time Point	Bertralstat (%)	Lanadelumab (%)	SC-pdC1-INH (%)
3 months (All Patients)	86	89	73
6 months (All Patients)	76	74	64
9 months (All Patients)	63	65	55
12 months (All Patients)	61	58	53
3 months (≥2 Fills)	97	97	86*
6 months (≥2 Fills)	88	81	77
9 months (≥2 Fills)	74	71	66
12 months (≥2 Fills)	71	63	63

CONCLUSIONS

- Adherence and persistence rates for all 3 LTP treatments among patients with HAE were uniformly high.
- Rates of bertralstat adherence and persistence were comparable with those observed following lanadelumab or SC-pdC1-INH initiation.
- Similarly across cohorts suggests that choice of LTP should be individualized and based on patient and doctor shared decision-making.

Limitations

- The study only included insured individuals and may not be representative of the 10% of US adults aged 18-64 years who are uninsured or insured through plans not included in the dataset (e.g. those insured through the Veterans Health Administration).
- Localized swelling/edema or breathing difficulty was less common among patients taking bertralstat than among those taking SC-pdC1-INH (56% vs. 76%, p=0.007).
- Among LTP cohorts, 46% of patients were LTP experienced, 21% were LTP naive and on-demand experienced, and 23%-28% were LTP naive and on-demand naïve.

REFERENCES

1. Zuraw BL, et al. Hereditary angioedema. *N Engl J Med*. 2018;378(25):2414-2424.
2. Zuraw BL, et al. Bertralstat for the prophylaxis of hereditary angioedema. *N Engl J Med*. 2023;389(10):911-921.
3. Zuraw BL, et al. Lanadelumab for the prophylaxis of hereditary angioedema. *N Engl J Med*. 2018;378(25):2425-2435.
4. Zuraw BL, et al. Subcutaneous plasma-derived C1-inhibitor for the prophylaxis of hereditary angioedema. *N Engl J Med*. 2018;378(25):2436-2446.
5. Zuraw BL, et al. Comparison of bertralstat, lanadelumab, and subcutaneous plasma-derived C1-inhibitor for the prophylaxis of hereditary angioedema. *N Engl J Med*. 2023;389(10):922-932.

DISCLOSURES

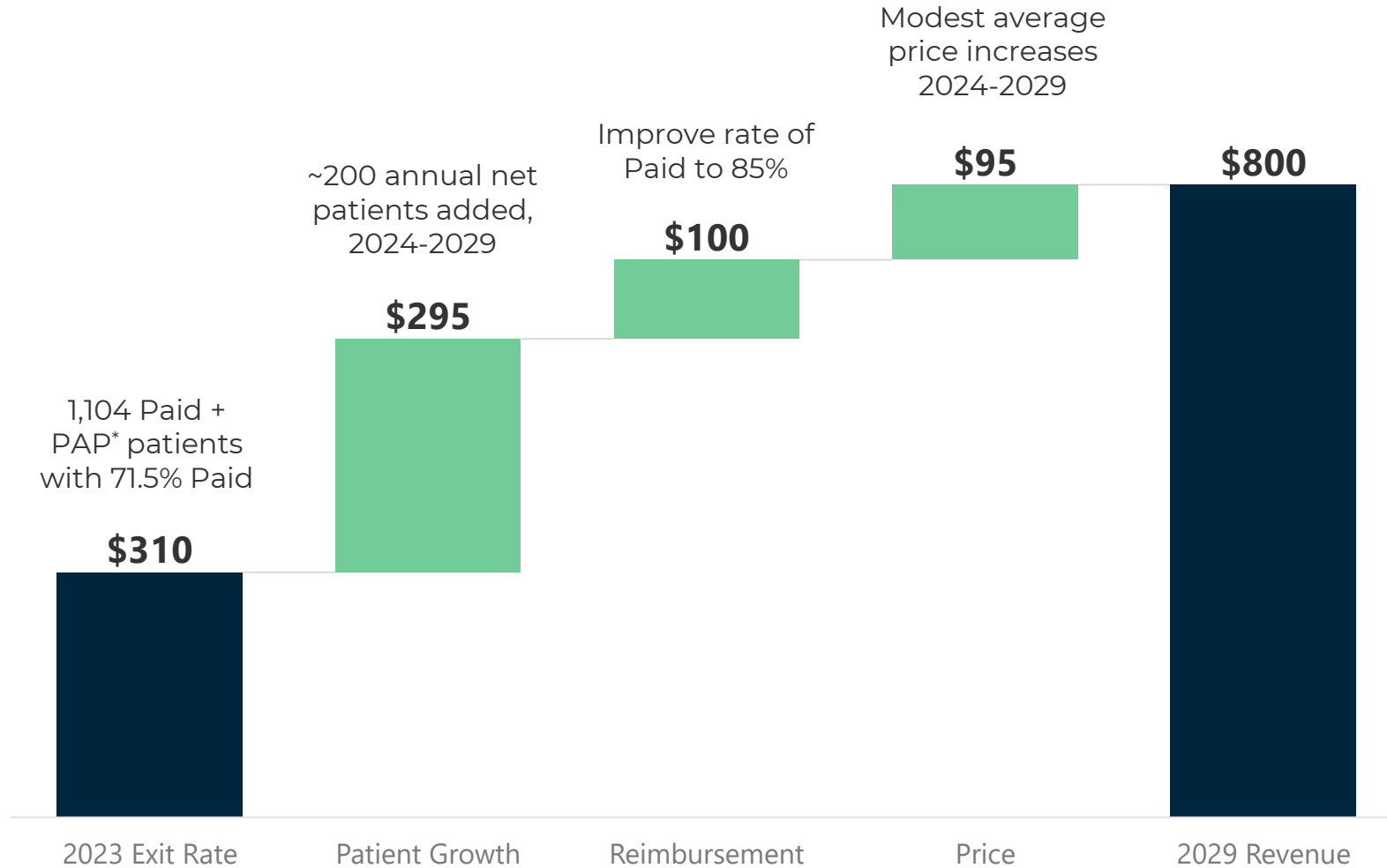
Bertralstat is a registered trademark of Biogen. Lanadelumab is a registered trademark of AstraZeneca. SC-pdC1-INH is a registered trademark of CSL Behring. The authors have nothing to disclose.

ACKNOWLEDGMENTS

This study was funded by Biogen, AstraZeneca, and CSL Behring.

Source: Adherence and Persistence Among Hereditary Angioedema Patients Treated With Bertralstat, Lanadelumab, and Subcutaneous Plasma-Derived C1-Inhibitor Presented at the ACAAI Scientific Meeting 2024 - October 24-28, 2024

Path to \$800M US revenue in 2029



ASSUMPTIONS

- 15-20% gross-to-net on Paid shipments
- Compliance in low-90s%

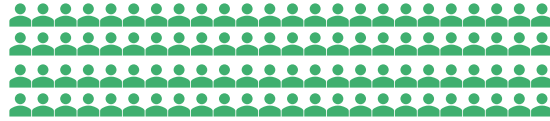
* PAP is the company's long-term patient assistance program

Comprehensive annual research + market simulation

OUR MODEL STARTS WITH PREFERENCE AND SIMULATES 6,000 MARKET INTERACTIONS BETWEEN HCPS, PATIENTS, & PAYERS

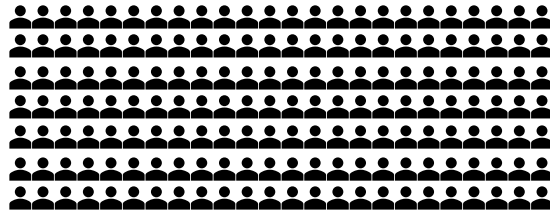
Research Sample*

PATIENTS



n=100 HAE patients

PHYSICIANS



*n=100 AIs**, and n=75 non-AIs***

PAYERS



n=56 decision makers covering over 200 million total lives.

Market Model Simulation (Monte Carlo)

- 1 A patient, physician, and payer are randomly selected from survey respondents.
- 2 The model evaluates individual prescribing decisions based on patient preference, physician preference & payer approval within a framework of market dynamics (e.g., awareness, adoption, launch timing)
- 3 For a single simulation run, the process is repeated 30 times for each patient category
- 4 The simulation is then repeated 50 times (6,000 interactions) to create a generalized distribution, then scaled and weighted to HAE total population

Modeling Process - Visual Example

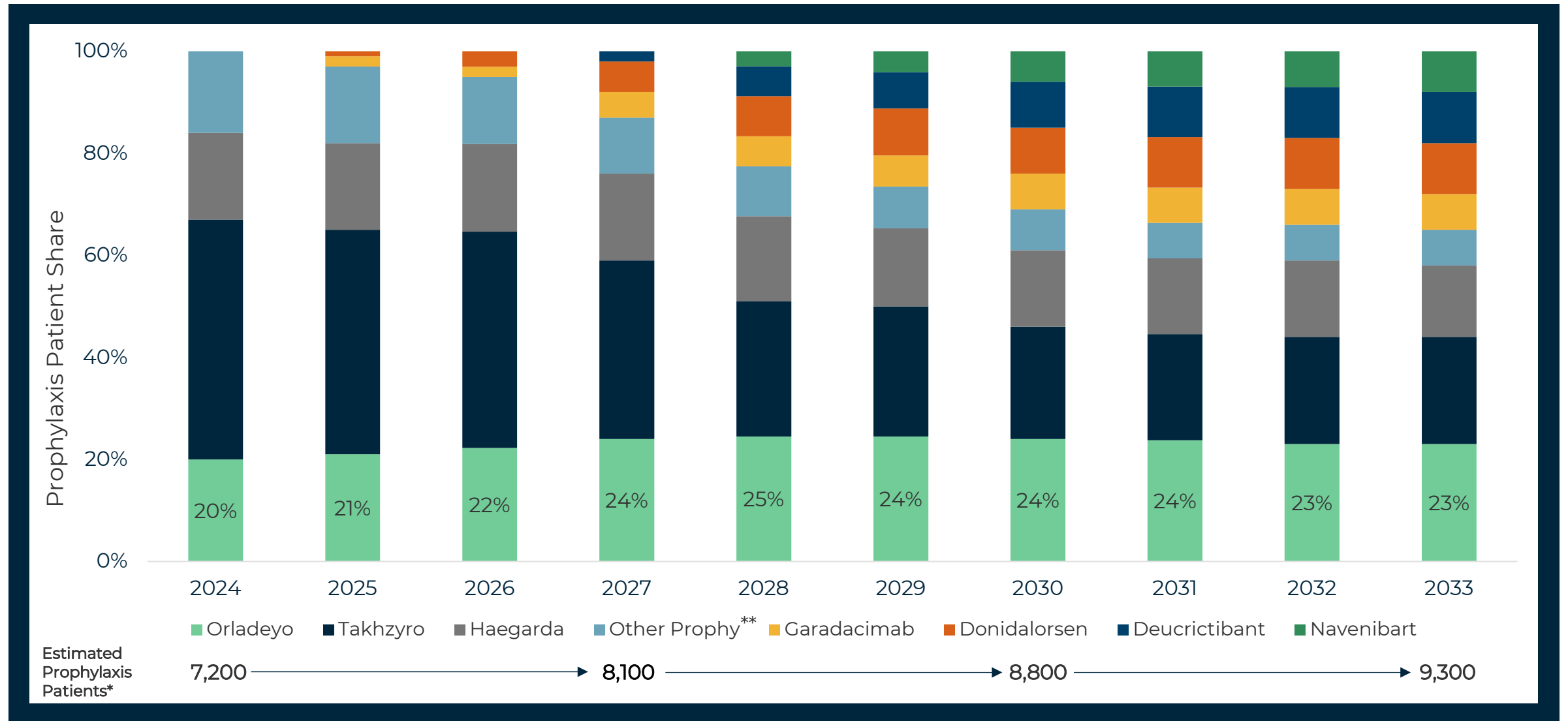
1	Single Interaction & Decision	
3	Single Simulation Run	<p><i>Prophy</i></p> <p><i>Acute Only</i></p> <p><i>No HAE Med</i></p> <p><i>New Patient</i></p>
4	Full Market Simulation Approach	

* Choice-based conjoint

** HAE treaters: Allergists & Immunologists

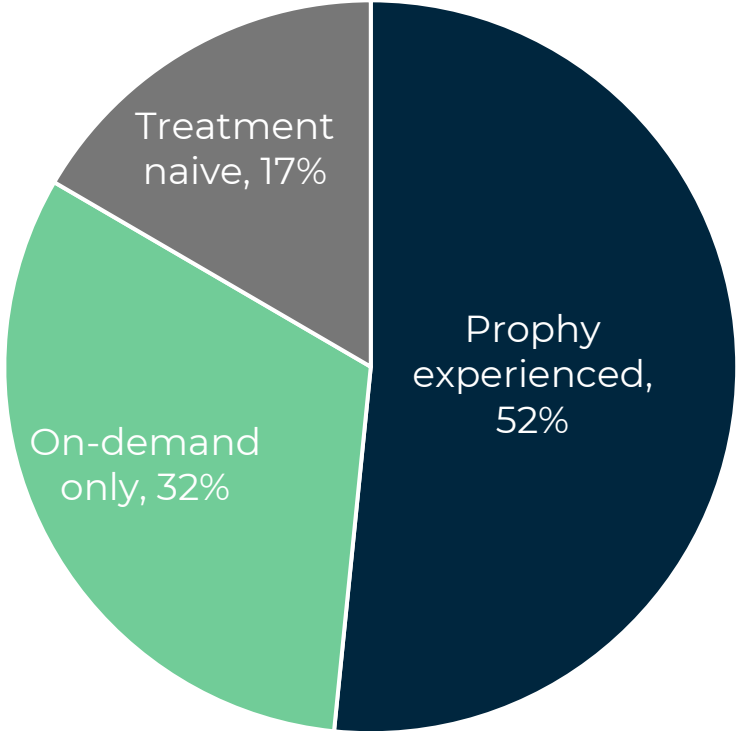
Monte Carlo simulation outcome: U.S. prophylaxis market share

ORLADEYO REACHES A STEADY STATE OF OVER 2,000 PATIENTS IN U.S. DURING 2028, EVEN AS NEW PRODUCTS GAIN SHARE



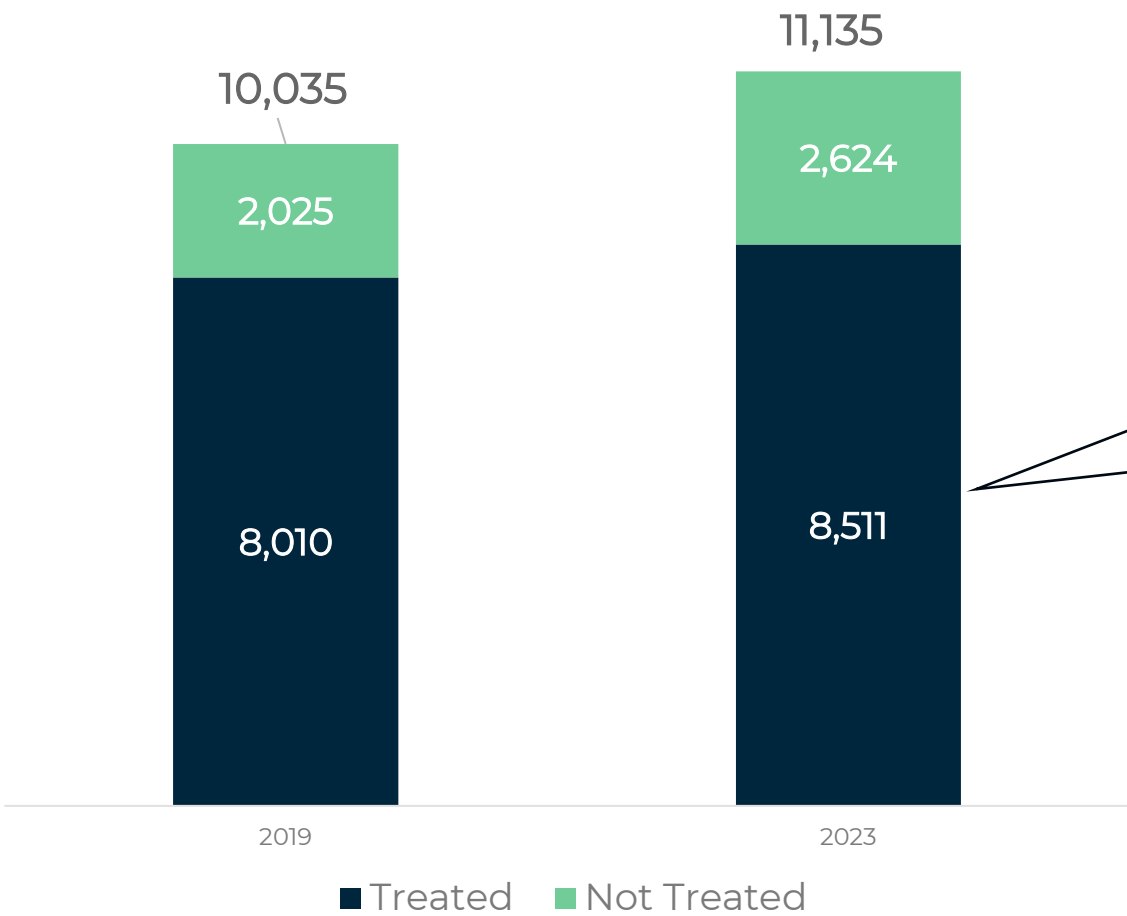
Source: BioCryst Internal Market Research Study (Conducted Jun 2024) *Source: 2018-2023 administrative claims data
 **Other Prophylaxis: Any other current medication (including acute) taken prophylactically for HAE

Over 50% of patients trying ORLADEYO launch-to-date had prior experience on another prophylaxis therapy



Source: Specialty Pharmacy patient-reported data through June 3, 2024, supplemented with 2015-2024 administrative claims data.

The HAE market of diagnosed and treated patients continues to grow



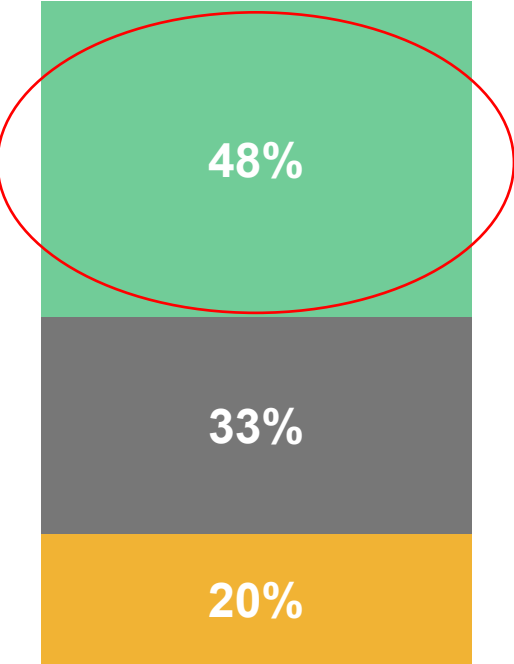
Between 2019 and 2023:

- Over 1,000 more diagnosed patients
- Over 500 more patients treated for HAE

Source: 2018-2023 administrative claims data

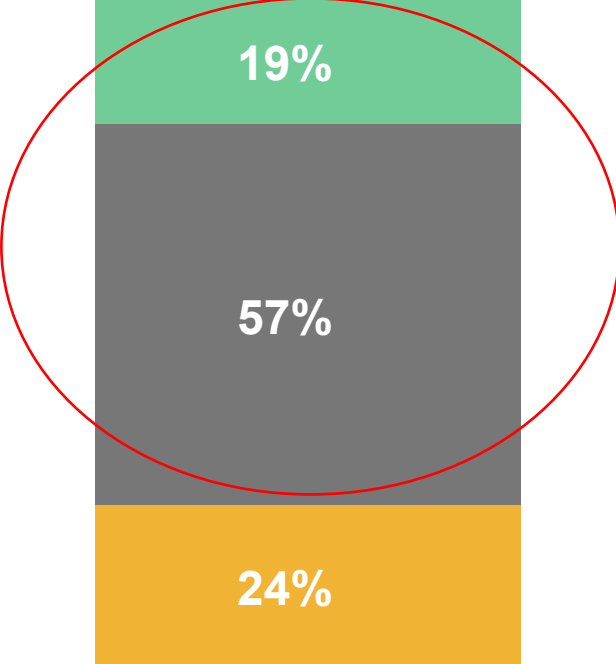
MARKET RESEARCH: 1 in 2 injectable prophylaxis users prefers oral ROA, 3 in 4 are willing to switch

PREFERENCE AMONG CURRENT INJECTABLE PROPHY USERS
(n=120)



- Prefers oral prophylaxis admin
- No preference
- Prefers injection/infusion prophylaxis admin

WILLINGNESS TO SWITCH HAE LTP AMONG CURRENT INJECTABLE PROPHY USERS
(n=120)



- Not at all willing
- Somewhat willing
- Extremely willing

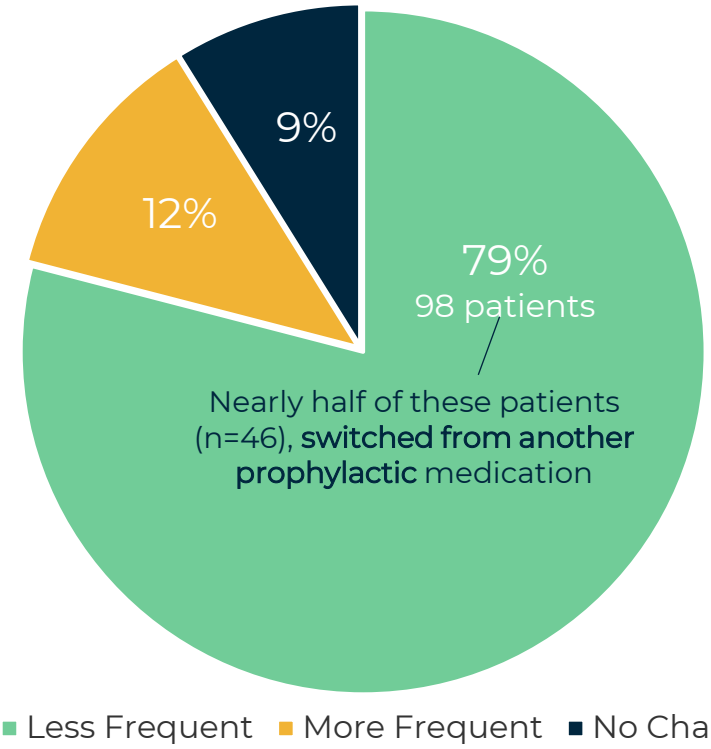
Source: BioCryst Internal Market Research Study (Conducted Oct 2023)

ROA = route of administration
LTP = long-term prophylaxis

MARKET RESEARCH: 4 out of 5 patients report having fewer attacks after starting ORLADEYO and 3 out of 4 report less severe attacks

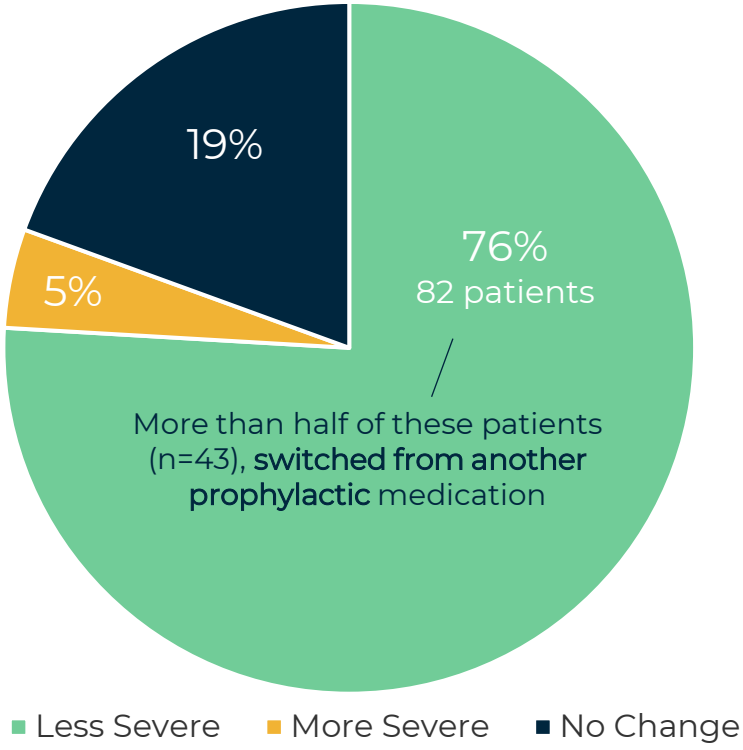
HAE ATTACK FREQUENCY ON ORLADEYO

All Respondents (n=124)



HAE ATTACK SEVERITY ON ORLADEYO

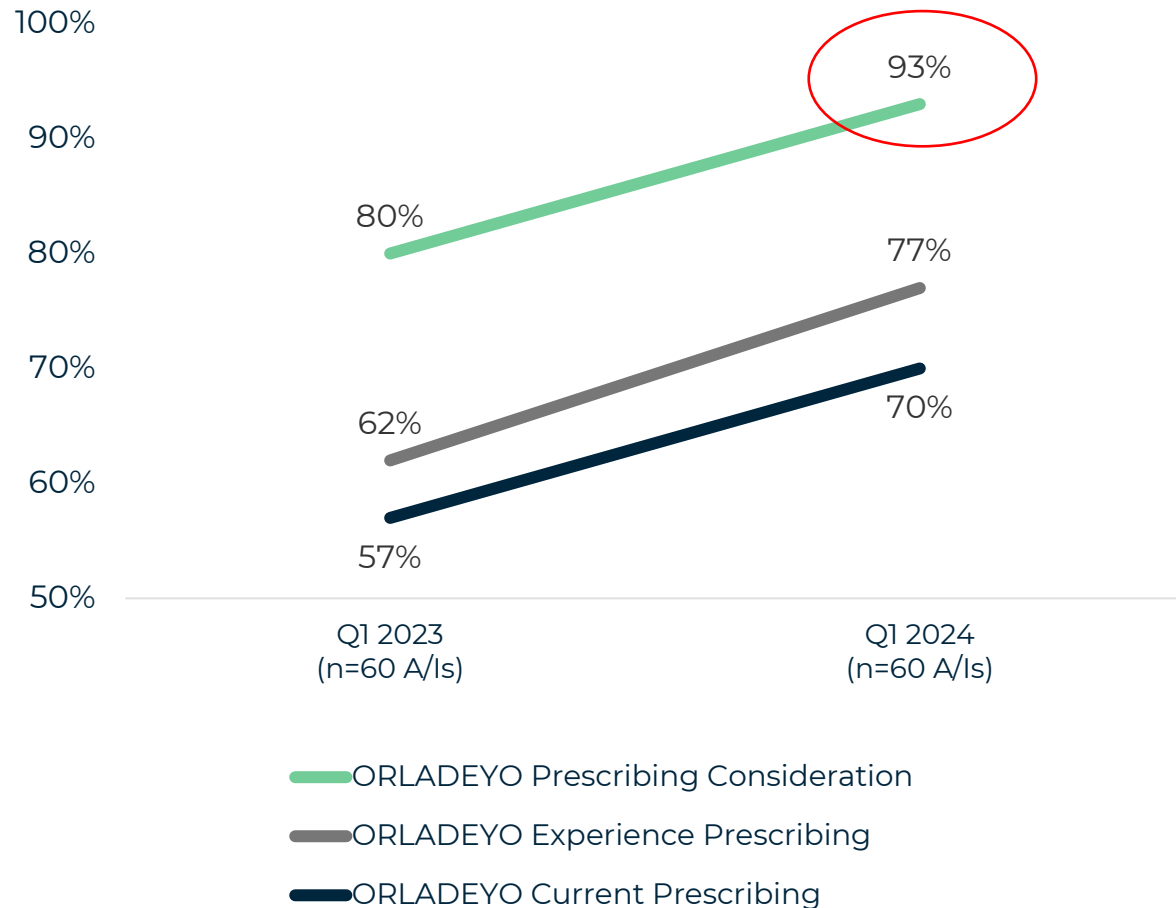
All Respondents (n=108)



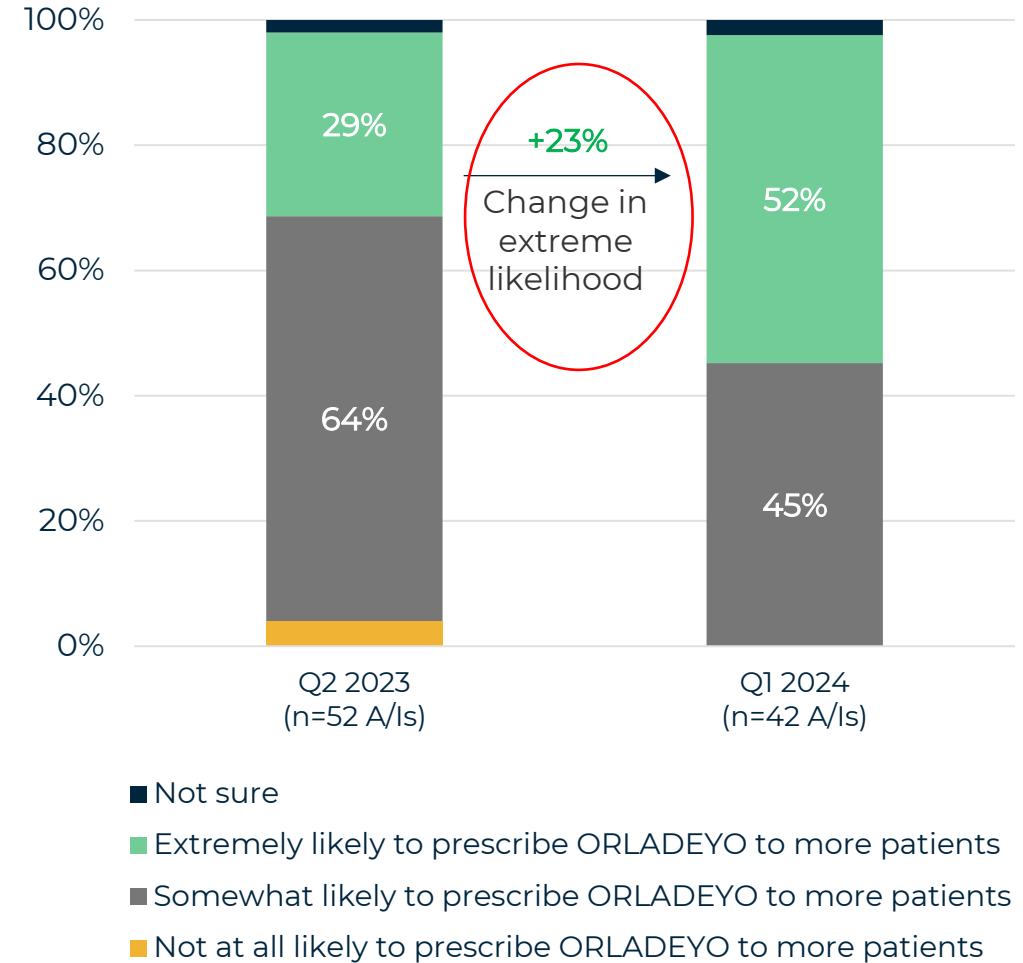
Source: BioCryst Internal Market Research Studies (Conducted Jan 2023, Apr 2024)

MARKET RESEARCH: Allergist/Immunologist intent to prescribe has increased strongly since the first half of 2023

ORLADEYO PRESCRIBING METRICS



LIKELIHOOD TO PRESCRIBE ORLADEYO TO MORE PATIENTS



Source: BioCryst Internal Market Research Studies (Conducted Feb-Mar 2023, May 2023, Feb 2024)

Our pipeline

ASSET	PROGRAM	LEAD OPTIMIZATION	PRE-CLINICAL	PROOF OF CONCEPT†	PIVOTAL‡	APPROVED / COMMERCIAL
ORLADEYO® (berotralstat) Oral Plasma Kallikrein Inhibitor	Hereditary Angioedema (HAE)	[Progress bar spanning all stages]				
ORLADEYO® (berotralstat) Oral Plasma Kallikrein Inhibitor (age ≥2 years)	Hereditary Angioedema (HAE)	[Progress bar spanning all stages]				
BCX17725 Protein Therapeutic	Netherton Syndrome	[Progress bar spanning all stages]				
Avoralstat Ocular Plasma Kallikrein Inhibitor	Diabetic Macular Edema (DME)	[Progress bar spanning all stages]				
Oral C5 Inhibitor	Complement-Mediated Diseases	[Progress bar spanning all stages]				
Oral C2 Inhibitor	Complement-Mediated Diseases	[Progress bar spanning all stages]				
Bifunctional Complement Inhibitor	Complement-Mediated Diseases	[Progress bar spanning all stages]				

*ORLADEYO (age ≥ 2 years), BCX17725, and avoralstat are investigational and have not been deemed safe and effective by the FDA.

†Proof of Concept is typically Phase 1 or 2.

‡Pivotal is typically Phase 3.

BCX17725 Phase 1 enrollment begins

ASSET	2024	2025	2026	2027	2028
BCX17725 Protein Therapeutic	Start Phase 1	Start POC	POC data		

Next catalyst: initial patient data by end of 2025

What is Netherton syndrome?

- A severe, rare, genetic skin condition, caused by a loss-of-function mutation in SPINK5 gene
- SPINK5 is a natural inhibitor of KLK5, a serine protease that regulates skin turnover
- Lack of KLK5 inhibition is the underlying cause of disease
- Causes full-body redness, inflammation, infection risk, itching, fragile hair, and high infant mortality

Market opportunity

Population

- Estimated 1,600 in US based on claims analysis
- Up to 5,000 with improved diagnosis (no existing ICD code)

Competition

- No approved disease-modifying therapies

Pricing

- Typical pricing for rare disease

BCX17725 is a highly specific fusion protein KLK5 inhibitor that targets the underlying cause of disease

Finance summary

(FIGURES IN MILLIONS)

Q3 2024 CASH POSITION

Cash, cash equivalents, restricted cash & investments at December 31, 2023	\$391
Cash, cash equivalents, restricted cash & investments at June 30, 2024	\$338
Cash, cash equivalents, restricted cash & investments at September 30, 2024	\$352
Senior credit facility ^A	\$324

2024 FY GUIDANCE

ORLADEYO revenue	\$430-435
Total product revenue	\$443-448
Operating expenses (excluding non-cash comp)	\$380-390

A – From Pharmakon Advisors, \$300M drawn at issuance in Q2 2023. The \$324M balance above represents \$300M initial issuance plus PIK interest to-date (did not elect the PIK option for Q3 2024; the PIK option has now expired)

Traditional debt and royalty breakdown

	September 30, 2024	December 31, 2023
Royalty financing obligations - current	33,000	23,565
Royalty financing obligations - long-term	481,775	508,034
Total royalty financing obligations	514,775	531,599
Secured term loan	314,333	303,231

	Traditional Debt	Commercial Royalty
Initial amount	\$300M term loan	\$425M royalty upfronts
Partner(s)	Pharmakon (2023)	RP (2020, 2021) ^A OMERS (2021) ^A
Description	<ul style="list-style-type: none"> Rate: 3 mo. SOFR +7.00% (With PIK option: +7.25%) Maturity: April 2028 bullet Financial covenants: None PIK option: 50% of interest for first six quarters 	<ul style="list-style-type: none"> Non-recourse (payments funded with revenues) Considered a “debt instrument” per GAAP An effective interest rate is calculated based on forecasted royalties, which determines interest expense Current balance = prior balance + interest expense – royalty paid If interest expense > royalties paid, balance increases If royalties paid > interest expense, balance decreases

A – Royalty terms described on next slide

Royalty obligations: terms

	Upfront	Product	Rate Tiers (Key Territories ^B)	Rate Tiers (Other Markets ^B)	Cumulative Payback Cap
RP 2020	\$125M	ORLADEYO	\$0-350M: 8.75% \$350M-550M: 2.75% Over \$550M: None	\$0-150M: 20% \$150M-230M: 10% Over \$230M: None	None
RP 2021	\$150M ^A	ORLADEYO	\$0-350M: 0.75% \$350M-550M: 1.75% Over \$550M: None	\$0-150M: 3% \$150M-230M: 2% Over \$230M: None	None
OMERS 2021	\$150M	ORLADEYO	\$0-350M: 10% \$350M-550M: 3% Over \$550M: None	\$0-150M: 20% \$150M-230M: 10% Over \$230M: None	1.55x

A – Royalty Pharma made an additional \$50M equity investment in conjunction with the 2021 Royalty Purchase Agreement

B – The “Key Territories” include the United States, key European markets and other markets where ORLADEYO is sold directly or through distributors. The “Other Markets” include revenue from licensees outside the Key Territories.

Third Quarter 2024 Results Call

Corporate Update & Financial Results

November 4, 2024

