Fourth Quarter and Full Year 2023 Results Call

Corporate Update & Financial Results

February 26, 2024

Forward-looking statements

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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	**Dr. Ryan Arnold** Chief Medical Officer
	Charlie Gayer Chief Commercial Officer
Financial update	**Anthony Doyle** Chief Financial Officer
Q&A	

Patients experience excellent HAE control on ORLADEYO

LONG-TERM CLINICAL EVIDENCE

90.8%

Attack reduction vs baseline after 96 weeks on berotralstat 150mg in APeX-2 study

Source: Kiani-Alikhan S, Gower R, Craig T et al. Once-daily oral berotralstat for long-term prophylaxis of hereditary angioedema: The open-label extension of the APeX-2 randomized trial. J Allergy Clin Immunol December 2023

LONG-TERM REAL-WORLD EVIDENCE

Rapid

Rapid attack control regardless of baseline rate or C1-INH level and function

Sustained

Sustained attack control regardless of baseline rate or C1-INH level and function

~0.5

Sustained monthly attack rates after switching to ORLADEYO from other prophylaxis therapies

Zero

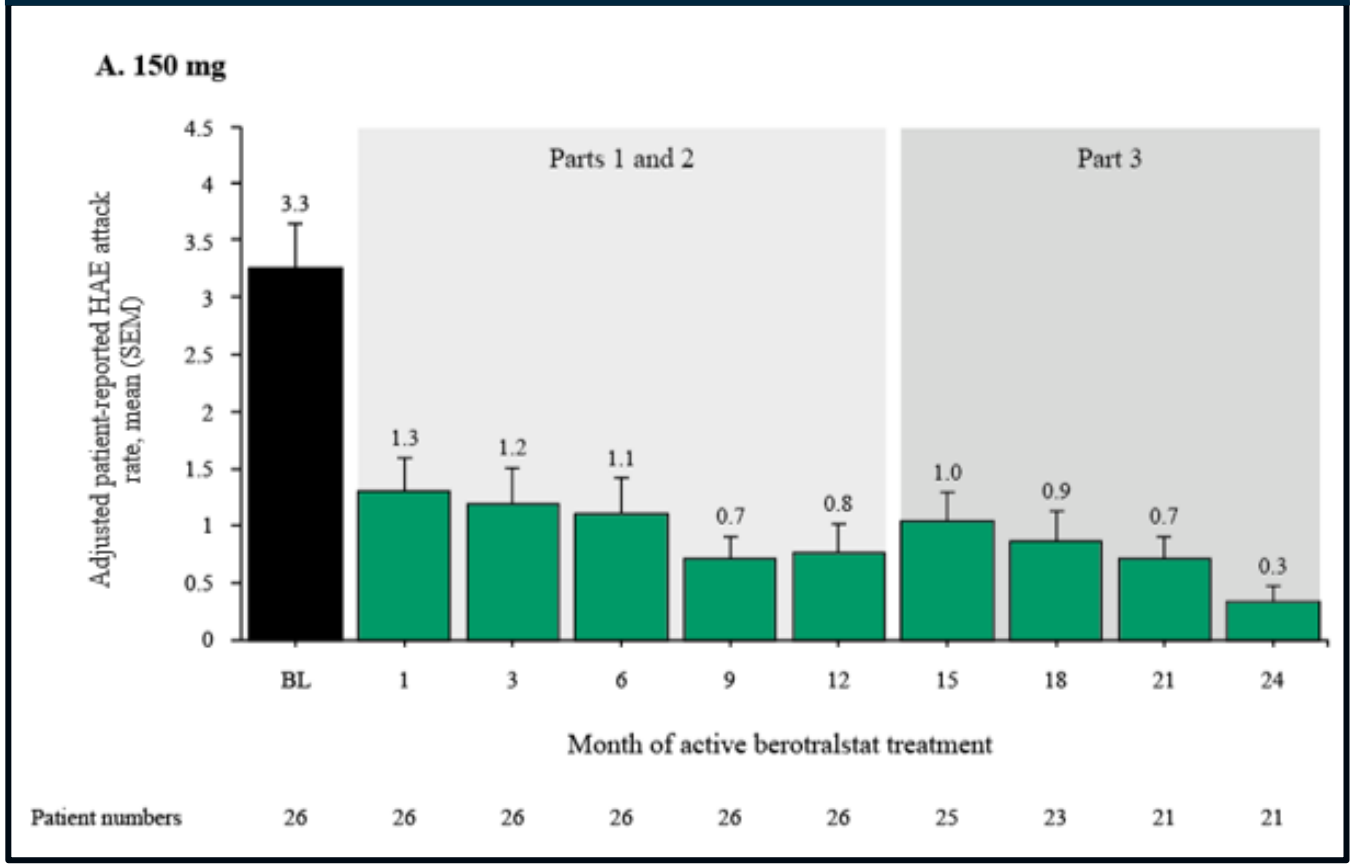
Median monthly attack rate on ORLADEYO for patients who were attack free at baseline

Source: AAAAI Annual Meeting 2024 Poster Presentations February 2024

ORLADEYO reduced attacks by 90.8% compared to baseline after 96 weeks

- Final published analysis of 96-week data from the APeX-2 trial showed patients who received berotralstat 150 mg from Day 1 experienced an average reduction in monthly attack rate of **90.8% compared to baseline**.
- Median attack rate at Month 24 was zero.

MONTHLY ATTACK RATE REDUCTION



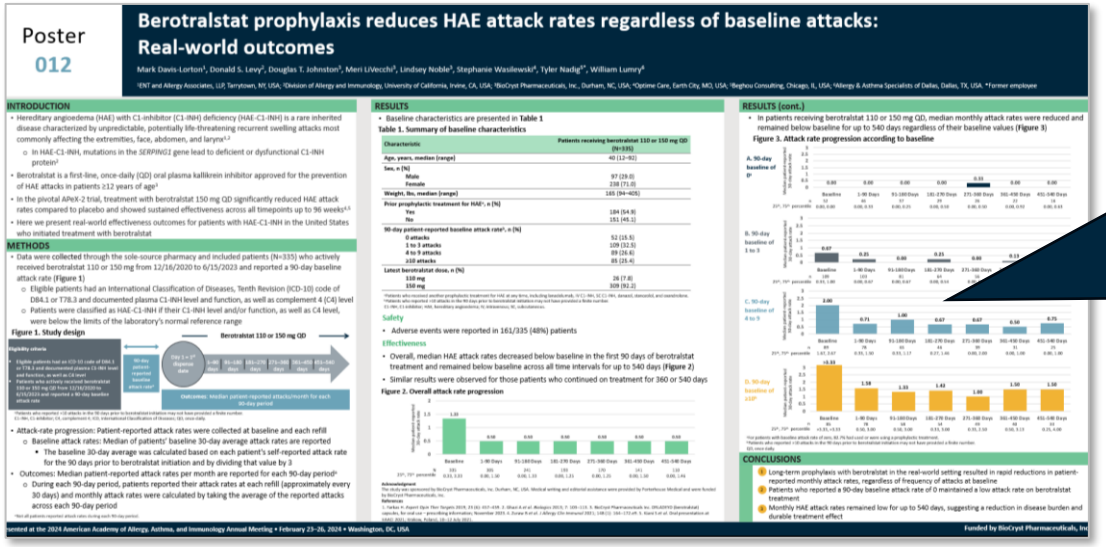
Source: Kiani-Alikhan S, Gower R, Craig T *et al.* Once-daily oral berotralstat for long-term prophylaxis of hereditary angioedema: The open-label extension of the APeX-2 randomized trial. *J Allergy Clin Immunol Pract* 2023; Epub ahead of print (DOI: 10.1016/j.jaip.2023.12.019)

Baseline adjusted patient-reported HAE attack rates are based on the number of HAE attacks experienced between screening and the start of Part 1 (i.e., the run-in period).

BL, baseline; HAE, hereditary angioedema; SEM, standard error of the mean.

Reduced attack rates, regardless of baseline attacks

- Patients experienced rapid, substantial and sustained reductions in attacks after starting ORLADEYO, regardless of baseline attack rate
- Patients with zero attacks at baseline maintained attack control on ORLADEYO



Poster 012

Berotralstat prophylaxis reduces HAE attack rates regardless of baseline attacks: Real-world outcomes

Mark Davis-Lorton[1], Donald S. Levy[2], Douglas T. Johnston[3], Meri LiVecchi[4], Lindsey Noble[5], Stephanie Wasilewski[6], Tyler Nadig[6*], William Lumry[4]

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INTRODUCTION

- Hereditary angioedema (HAE) with C1-inhibitor (C1-INH) deficiency (HAE-C1-INH) is a rare inherited disease characterized by unpredictable, potentially life-threatening recurrent swelling attacks most commonly affecting the extremities, face, abdomen, and larynx[1,2]
  - In HAE-C1-INH, mutations in the *SERPING1* gene lead to deficient or dysfunctional C1-INH protein[2]
- Berotralstat is a first-line, once-daily (QD) oral plasma kallikrein inhibitor approved for the prevention of HAE attacks in patients ≥12 years of age[3]
- In the pivotal APeX-2 trial, treatment with berotralstat 150 mg QD significantly reduced HAE attack rates compared to placebo and showed sustained effectiveness across all timepoints up to 96 weeks[4,5]
- Here we present real-world effectiveness outcomes for patients with HAE-C1-INH in the United States who initiated treatment with berotralstat

METHODS

- Data were collected through the sole-source pharmacy and included patients (N=335) who actively received berotralstat 110 or 150 mg from 12/16/2020 to 6/15/2023 and reported a 90-day baseline attack rate (Figure 1)
  - Eligible patients had an International Classification of Diseases, Tenth Revision (ICD-10) code of D84.1 or T78.3 and documented plasma C1-INH level and function, as well as complement 4 (C4) level
  - Patients were classified as HAE-C1-INH if their C1-INH level and/or function, as well as C4 level, were below the limits of the laboratory's normal reference range

Figure 1. Study design

- Attack-rate progression: Patient-reported attack rates were collected at baseline and each refill
  - Baseline attack rates: Median of patients' baseline 30-day average attack rates are reported
    - The baseline 30-day average was calculated based on each patient's self-reported attack rate for the 90 days prior to berotralstat initiation and by dividing that value by 3
- Outcomes: Median patient-reported attack rates per month are reported for each 90-day period[a]
  - During each 90-day period, patients reported their attack rates at each refill (approximately every 30 days) and monthly attack rates were calculated by taking the average of the reported attacks across each 90-day period

[a]Not all patients reported attack rates during each 90-day period.

RESULTS

- Baseline characteristics are presented in Table 1

Table 1. Summary of baseline characteristics

Characteristic	Patients receiving berotralstat 110 or 150 mg QD (N=335)
Age, years, median (range)	40 (12–82)
Sex, n (%)	
Male	97 (29.0)
Female	238 (71.0)
Weight, lbs, median (range)	165 (94–405)
Prior prophylactic treatment for HAE[a], n (%)	
Yes	184 (54.9)
No	151 (45.1)
90-day patient-reported baseline attack rate[b], n (%)	
0 attacks	52 (15.5)
1 to 3 attacks	109 (32.5)
4 to 9 attacks	89 (26.6)
≥10 attacks	85 (25.4)
Latest berotralstat dose, n (%)	
110 mg	26 (7.8)
150 mg	309 (92.2)

[a]Patients who received another prophylactic treatment for HAE at any time, including lanadelumab, C1-INH, BCX7484, danazol, stanozolol, and tranexamic acid. [b]Patients who reported ≥10 attacks in the 90 days prior to berotralstat initiation may not have provided a finite number.
C1-INH, C1 inhibitor; HAE, hereditary angioedema; IV, intravenous; SC, subcutaneous.

Safety

- Adverse events were reported in 161/335 (48%) patients

Effectiveness

- Overall, median HAE attack rates decreased below baseline in the first 90 days of berotralstat treatment and remained below baseline across all time intervals for up to 540 days (Figure 2)
- Similar results were observed for those patients who continued on treatment for 360 or 540 days

Figure 2. Overall attack rate progression

RESULTS (cont.)

- In patients receiving berotralstat 110 or 150 mg QD, median monthly attack rates were reduced and remained below baseline for up to 540 days regardless of their baseline values (Figure 3)

Figure 3. Attack rate progression according to baseline

CONCLUSIONS

- Long-term prophylaxis with berotralstat in the real-world setting resulted in rapid reductions in patient-reported monthly attack rates, regardless of frequency of attacks at baseline
- Patients who reported a 90-day baseline attack rate of 0 maintained a low attack rate on berotralstat treatment
- Monthly HAE attack rates remained low for up to 540 days, suggesting a reduction in disease burden and durable treatment effect

Acknowledgments
The study was sponsored by BioCryst Pharmaceuticals, Inc., Durham, NC, USA. Medical writing and editorial assistance were provided by Parexel International (MA) Corporation and were funded by BioCryst Pharmaceuticals, Inc.

References
1. Farkas H. Expert Opin Orphan Drugs 2019; 21:311-317. 2. Ghazi A et al. Allergy Asthma Clin Immunol 2021; 17:110-116. 3. BioCryst Pharmaceuticals Inc. ORLADEYO (berotralstat) capsules, for oral use – prescribing information; November 2021. 4. Banerji A et al. J Allergy Clin Immunol Pract 2021; 9:1051-1061.e10. 5. Riedl MA et al. Clin Pharmacol Ther 2021; 109:133-142.

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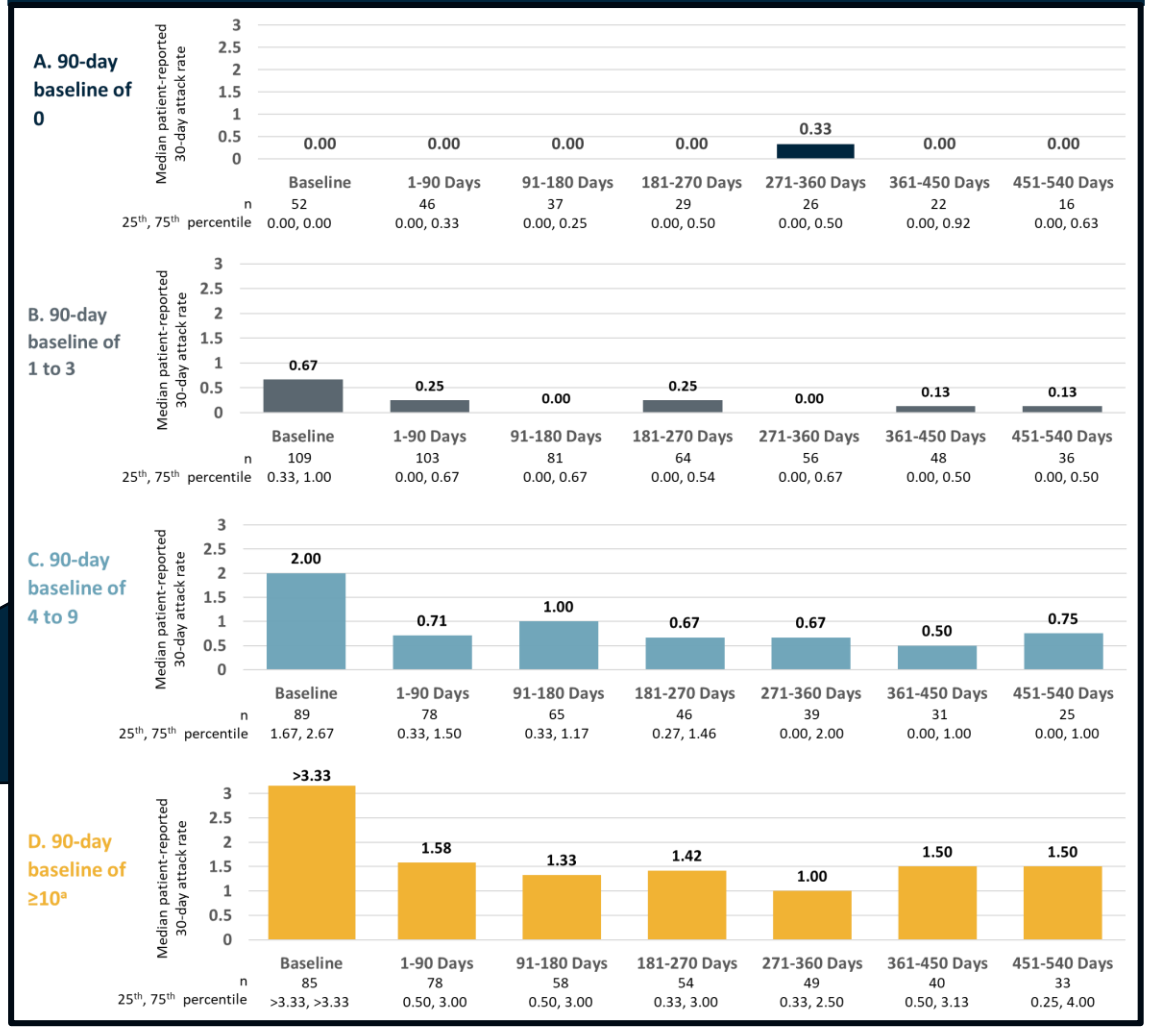
Funded by BioCryst Pharmaceuticals, Inc.

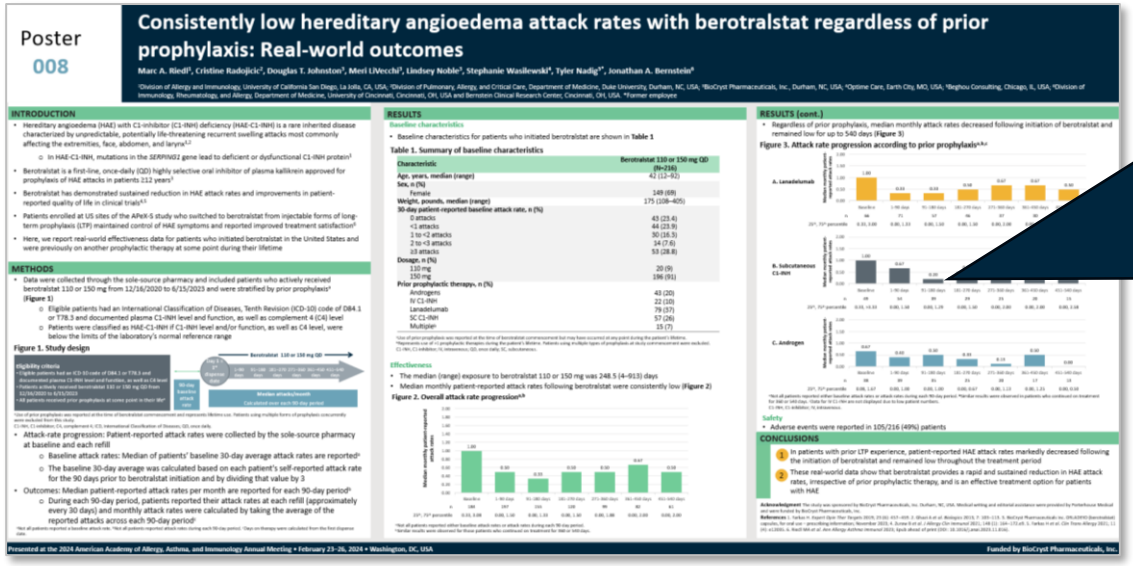
MEDIAN MONTHLY ATTACK RATE PROGRESSION BY BASELINE RATE



Source: Berotralstat prophylaxis reduces HAE attack rates regardless of baseline attacks: Real-world outcomes
Presented at the AAAAI Congress 2024 • February 23–26, 2024

Sustained attack control, regardless of prior prophy

- Patients experienced sustained median attack rates of approximately 0.5 per month on ORLADEYO, regardless of prior prophylaxis therapy


Poster 008

Consistently low hereditary angioedema attack rates with berotralstat regardless of prior prophylaxis: Real-world outcomes

Marc A. Riedl[1], Cristine Radojicic[2], Douglas T. Johnston[3], Meri LiVecchi[3], Lindsey Noble[3], Stephanie Wasilewski[4], Tyler Nadig[4*], Jonathan A. Bernstein[5]

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INTRODUCTION

- Hereditary angioedema (HAE) with C1-inhibitor (C1-INH) deficiency (HAE-C1-INH) is a rare inherited disease characterized by unpredictable, potentially life-threatening recurrent swelling attacks most commonly affecting the extremities, face, abdomen, and larynx[1,2]
  - In HAE-C1-INH, mutations in the *SERPING1* gene lead to deficient or dysfunctional C1-INH protein[3]
- Berotralstat is a first-line, once-daily (QD) highly selective oral inhibitor of plasma kallikrein approved for prophylaxis of HAE attacks in patients ≥12 years[3]
- Berotralstat has demonstrated sustained reduction in HAE attack rates and improvements in patient-reported quality of life in clinical trials[4,5]
- Patients enrolled at US sites of the APeX-S study who switched to berotralstat from injectable forms of long-term prophylaxis (LTP) maintained control of HAE symptoms and reported improved treatment satisfaction[6]
- Here, we report real-world effectiveness data for patients who initiated berotralstat in the United States and were previously on another prophylactic therapy at some point during their lifetime

METHODS

- Data were collected through the sole-source pharmacy and included patients who actively received berotralstat 110 or 150 mg from 12/16/2020 to 6/15/2023 and were stratified by prior prophylaxis[a] (Figure 1)
  - Eligible patients had an International Classification of Diseases, Tenth Revision (ICD-10) code of D84.1 or T78.3 and documented plasma C1-INH level and function, as well as complement 4 (C4) level
  - Patients were classified as HAE-C1-INH if C1-INH level and/or function, as well as C4 level, were below the limits of the laboratory's normal reference range

Figure 1. Study design

- Attack-rate progression: Patient-reported attack rates were collected by the sole-source pharmacy at baseline and each refill
  - Baseline attack rates: Median of patients' baseline 30-day average attack rates are reported[b]
  - The baseline 30-day average was calculated based on each patient's self-reported attack rate for the 90 days prior to berotralstat initiation and by dividing that value by 3
- Outcomes: Median patient-reported attack rates per month are reported for each 90-day period[b]
  - During each 90-day period, patients reported their attack rates at each refill (approximately every 30 days) and monthly attack rates were calculated by taking the average of the reported attacks across each 90-day period[b]

[a]Not all patients received a baseline attack rate. [b]Not all patients reported attack rates during each 90-day period. Days on therapy were calculated from the first dispense date.

RESULTS

Baseline characteristics

- Baseline characteristics for patients who initiated berotralstat are shown in Table 1

Table 1. Summary of baseline characteristics

Characteristic	Berotralstat 110 or 150 mg QD (N=216)
Age, years, median (range)	42 (12–92)
Sex, n (%)	
Female	149 (69)
Weight, pounds, median (range)	175 (108–405)
30-day patient-reported baseline attack rate, n (%)	
0 attacks	43 (23.4)
<1 attacks	44 (23.9)
1 to <2 attacks	30 (16.3)
2 to <3 attacks	14 (7.6)
≥3 attacks	53 (28.8)
Dosage, n (%)	
110 mg	20 (9)
150 mg	196 (91)
Prior prophylactic therapy, n (%)	
Androgens	43 (20)
IV C1-INH	22 (10)
Lanadelumab	79 (37)
SC C1-INH	57 (26)
Multiple[c]	15 (7)

[c]Use of prior prophylaxis was reported at the time of berotralstat commencement but may have occurred at any point during the patient's lifetime. [d]Represents use of ≥1 prophylactic therapies during the patient's lifetime. Patients using multiple types of prophylaxis at study commencement were excluded. C1-INH, C1-inhibitor; IV, intravenous; QD, once daily; SC, subcutaneous.

Effectiveness

- The median (range) exposure to berotralstat 110 or 150 mg was 248.5 (4–913) days
- Median monthly patient-reported attack rates following berotralstat were consistently low (Figure 2)

Figure 2. Overall attack rate progression[a,b]

[a]Not all patients reported either baseline attack rates or attack rates during each 90-day period. [b]Median results were observed for those patients who continued on treatment for 180 or 540 days.

RESULTS (cont.)

- Regardless of prior prophylaxis, median monthly attack rates decreased following initiation of berotralstat and remained low for up to 540 days (Figure 3)

Figure 3. Attack rate progression according to prior prophylaxis[a,b,c]

[a]Not all patients reported either baseline attack rates or attack rates during each 90-day period. [b]Median results were observed for patients who continued on treatment for 180 or 540 days. Data for IV C1-INH are not displayed due to low patient numbers. [c]C1-INH, C1-inhibitor; IV, intravenous.

Safety

- Adverse events were reported in 105/216 (49%) patients

CONCLUSIONS

1. In patients with prior LTP experience, patient-reported HAE attack rates markedly decreased following the initiation of berotralstat and remained low throughout the treatment period
2. These real-world data show that berotralstat provides a rapid and sustained reduction in HAE attack rates, irrespective of prior prophylactic therapy, and is an effective treatment option for patients with HAE

Acknowledgment The study was sponsored by BioCryst Pharmaceuticals, Inc. Durham, NC, USA. Medical writing and editorial assistance were provided by Porterhouse Medical and were funded by BioCryst Pharmaceuticals, Inc.

References 1. Farkas H. Expert Opin Ther Targets 2019;23:651–659. 2. Ghazi A et al. Allergy Clin Immunol 2021;148:351–364. 3. BioCryst Pharmaceuticals Inc. ORLADEYO (berotralstat) capsules, for oral use – prescribing information. November 2023. 4. Busse P et al. J Allergy Clin Immunol 2021;148:151–158. 5. Farkas H et al. Clin Trans Allergy 2021;11:e12001. 6. Riedl MA et al. Ann Allergy Asthma Immunol 2023. Epub ahead of print DOI: 10.1016/j.anai.2023.11.016.

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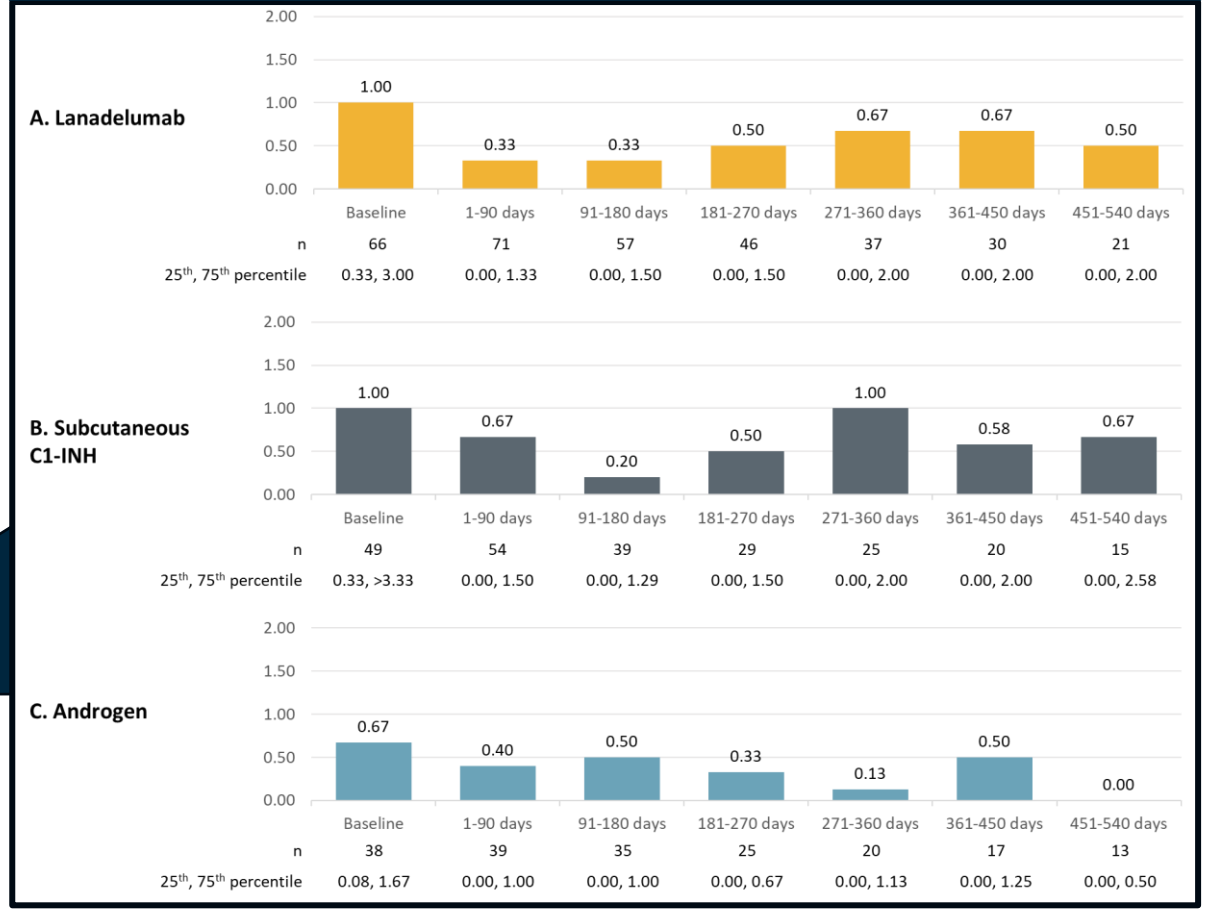
Funded by BioCryst Pharmaceuticals, Inc.

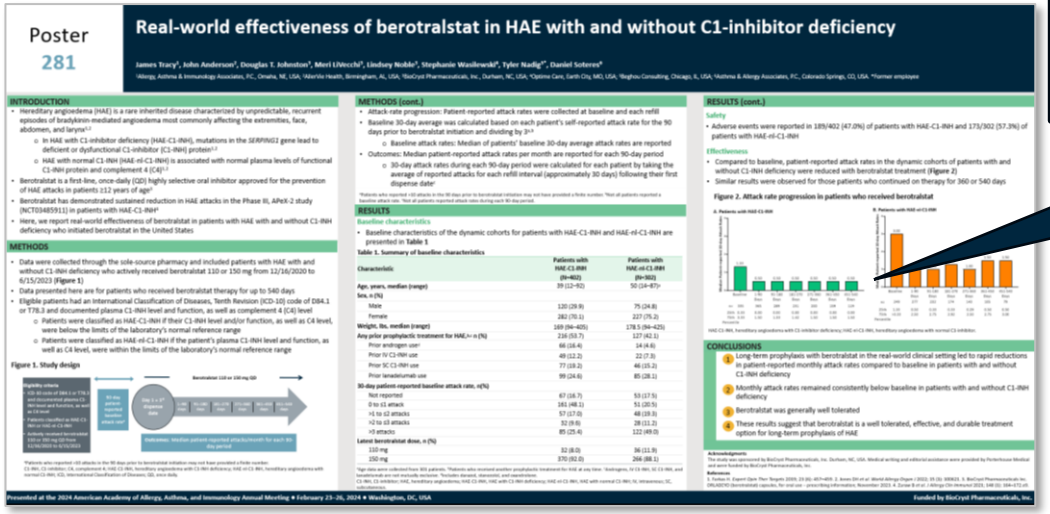
MEDIAN MONTHLY ATTACK RATE BY PRIOR PROPHYLAXIS



Source: Consistently low hereditary angioedema attack rates with berotralstat regardless of prior prophylaxis: Real-world outcomes
Presented at the AAAAI Congress 2024 • February 23–26, 2024

Reduced attack rates, regardless of C1-INH level and function

- Patients who initiated ORLADEYO experienced rapid, substantial and sustained reductions in attack rates through 18 months of treatment regardless of their C1-inhibitor (C1-INH) level and function



Poster 281

Real-world effectiveness of berotralstat in HAE with and without C1-inhibitor deficiency

James Tracy[1], John Anderson[2], Douglas T. Johnston[3], Meri Livecchi[4], Lindsey Noble[5], Stephanie Wasilewski[6], Tyler Nadig[5*], Daniel Soteres[6]

[1]Allergy, Asthma & Immunology Associates, P.C., Omaha, NE, USA; [2]Allervie Health, Birmingham, AL, USA; [3]BioCryst Pharmaceuticals, Inc., Durham, NC, USA; [4]Optimix Care, Earth City, MO, USA; [5]Boghou Consulting, Chicago, IL, USA; [6]Asthma & Allergy Associates, P.C., Colorado Springs, CO, USA; *Former employee

INTRODUCTION

- Hereditary angioedema (HAE) is a rare inherited disease characterized by unpredictable, recurrent episodes of bradykinin-mediated angioedema most commonly affecting the extremities, face, abdomen, and larynx[1,2]
  - In HAE with C1-inhibitor deficiency (HAE-C1-INH), mutations in the *SERPING1* gene lead to deficient or dysfunctional C1-inhibitor (C1-INH) protein[1,2]
  - HAE with normal C1-INH (HAE-nl-C1-INH) is associated with normal plasma levels of functional C1-INH protein and complement 4 (C4)[1,2]
- Berotralstat is a first-line, once-daily (QD) highly selective oral inhibitor approved for the prevention of HAE attacks in patients ≥12 years of age[3]
- Berotralstat has demonstrated sustained reduction in HAE attacks in the Phase III, APeX-2 study (NCT03485911) in patients with HAE-C1-INH[4]
- Here, we report real-world effectiveness of berotralstat in patients with HAE with and without C1-INH deficiency who initiated berotralstat in the United States

METHODS

- Data were collected through the sole-source pharmacy and included patients with HAE with and without C1-INH deficiency who actively received berotralstat 110 or 150 mg from 12/16/2020 to 6/15/2023 (**Figure 1**)
- Data presented here are for patients who received berotralstat therapy for up to 540 days
- Eligible patients had an International Classification of Diseases, Tenth Revision (ICD-10) code of D84.1 or T78.3 and documented plasma C1-INH level and function, as well as complement 4 (C4) level
  - Patients were classified as HAE-C1-INH if their C1-INH level and/or function, as well as C4 level, were below the limits of the laboratory's normal reference range
  - Patients were classified as HAE-nl-C1-INH if the patient's plasma C1-INH level and function, as well as C4 level, were within the limits of the laboratory's normal reference range

Figure 1. Study design

METHODS (cont.)

- Attack-rate progression: Patient-reported attack rates were collected at baseline and each refill
- Baseline 30-day average was calculated based on each patient's self-reported attack rate for the 90 days prior to berotralstat initiation and dividing by 3[a,b]
  - Baseline attack rates: Median of patients' baseline 30-day average attack rates are reported
- Outcomes: Median patient-reported attack rates per month are reported for each 90-day period
  - 30-day attack rates during each 90-day period were calculated for each patient by taking the average of reported attacks for each refill interval (approximately 30 days) following their first dispense date[c]

[a]Patients who reported >10 attacks in the 90 days prior to berotralstat initiation may not have provided a firm number. [b]Not all patients reported a baseline attack rate. [c]Not all patients reported attack rates during each 90-day period.

RESULTS

Baseline characteristics

- Baseline characteristics of the dynamic cohorts for patients with HAE-C1-INH and HAE-nl-C1-INH are presented in **Table 1**

Table 1. Summary of baseline characteristics

Characteristic	Patients with HAE-C1-INH (N=402)	Patients with HAE-nl-C1-INH (N=302)
Age, years, median (range)	39 (12–92)	50 (18–87)[a]
Sex, n (%)		
Male	120 (29.9)	75 (24.8)
Female	282 (70.1)	227 (75.2)
Weight, lbs. median (range)	169 (94–420)	178.5 (94–425)
Any prior prophylactic treatment for HAE,[b] n (%)	216 (53.7)	127 (42.1)
Prior androgen use[c]	66 (16.4)	14 (4.6)
Prior IV C1-INH use	49 (12.2)	22 (7.3)
Prior SC C1-INH use	77 (19.2)	46 (15.2)
Prior lanadelumab use	99 (24.6)	85 (28.1)
30-day patient-reported baseline attack rate, n(%)		
Not reported	67 (16.7)	53 (17.5)
0 to ≤1 attack	161 (40.1)	51 (20.5)
>1 to ≤2 attacks	57 (17.0)	48 (19.3)
>2 to ≤3 attacks	32 (8.0)	28 (11.2)
>3 attacks	85 (25.4)	122 (49.0)
Latest berotralstat dose, n (%)		
110 mg	32 (8.0)	36 (11.9)
150 mg	370 (92.0)	266 (88.1)

[a]Age data were collected from 301 patients. [b]Patients who received another prophylactic treatment for HAE at any time. [c]Androgens, SC C1-INH, and lanadelumab are not mutually exclusive. "Includes danazol, stanozolol, and oxandrolone.

RESULTS (cont.)

Safety

- Adverse events were reported in 189/402 (47.0%) of patients with HAE-C1-INH and 173/302 (57.3%) of patients with HAE-nl-C1-INH

Effectiveness

- Compared to baseline, patient-reported attack rates in the dynamic cohorts of patients with and without C1-INH deficiency were reduced with berotralstat treatment (**Figure 2**)
- Similar results were observed for those patients who continued on therapy for 360 or 540 days

Figure 2. Attack rate progression in patients who received berotralstat

CONCLUSIONS

1. Long-term prophylaxis with berotralstat in the real-world clinical setting led to rapid reductions in patient-reported monthly attack rates compared to baseline in patients with and without C1-INH deficiency
2. Monthly attack rates remained consistently below baseline in patients with and without C1-INH deficiency
3. Berotralstat was generally well tolerated
4. These results suggest that berotralstat is a well tolerated, effective, and durable treatment option for long-term prophylaxis of HAE

Acknowledgments

The study was sponsored by BioCryst Pharmaceuticals, Inc. Durham, NC, USA. Medical writing and editorial assistance were provided by Porterhouse Medical and were funded by BioCryst Pharmaceuticals, Inc.

References

1. Farkas H. Expert Opin Ther Targets 2009; 23 (6): 457–470. 2. Aygoren-Pürsün E et al. World Allergy Organ J 2022; 15 (3): 100619. 3. BioCryst Pharmaceuticals Inc. ORLADEYO (berotralstat) capsules, for oral use – prescribing information. November 2023. 4. Zuraw B et al. J Allergy Clin Immunol 2021; 148 (1): 164–172.e6.

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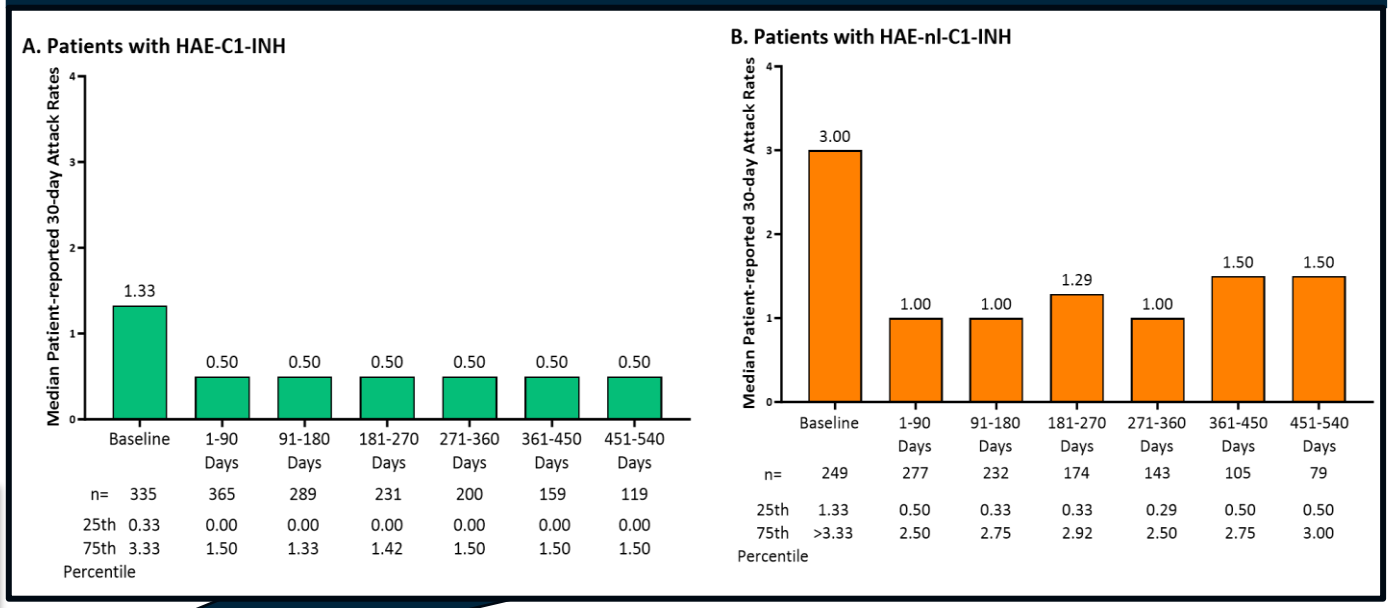
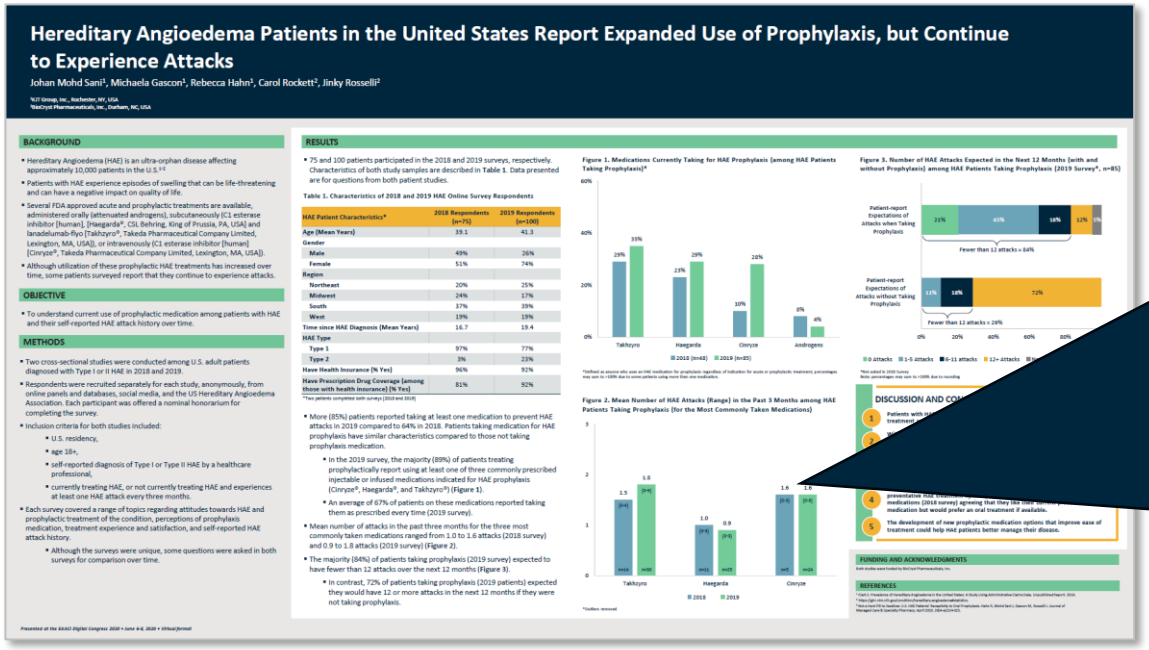
MONTHLY ATTACK RATE BY C1-INH DEFICIENCY



Source: Real-world effectiveness of berotralstat in HAE with and without C1-inhibitor deficiency
Presented at the AAAAI Congress 2024 • February 23–26, 2024

Historically, patients on injectable prophylaxis report low but consistent attack rates

- Patients taking injectable prophylaxis therapies have reported mean attack rates in the range of 0.3 to 0.6 per month



Hereditary Angioedema Patients in the United States Report Expanded Use of Prophylaxis, but Continue to Experience Attacks

Johan Mohd Sani[1], Michaela Gascon[1], Rebecca Hahn[1], Carol Rockett[2], Jinky Rossell[2]

[1]KJT Group, Inc., Rochester, NY, USA
[2]BioCryst Pharmaceuticals, Inc., Durham, NC, USA

BACKGROUND

- Hereditary Angioedema (HAE) is an ultra-orphan disease affecting approximately 10,000 patients in the U.S.[1-3]
- Patients with HAE experience episodes of swelling that can be life-threatening and can have a negative impact on quality of life.
- Several FDA approved acute and prophylactic treatments are available, administered orally (attenuated androgens), subcutaneously (C1 esterase inhibitor [human], [haegarda®, CSL Behring, King of Prussia, PA, USA] and lanadelumab-flyo [takhzyro®, Takeda Pharmaceutical Company Limited, Lexington, MA, USA]), or intravenously (C1 esterase inhibitor [human] [cinryze®, Takeda Pharmaceutical Company Limited, Lexington, MA, USA]).
- Although utilization of these prophylactic HAE treatments has increased over time, some patients surveyed report that they continue to experience attacks.

OBJECTIVE

- To understand current use of prophylactic medication among patients with HAE and their self-reported HAE attack history over time.

METHODS

- Two cross-sectional studies were conducted among U.S. adult patients diagnosed with Type I or II HAE in 2018 and 2019.
- Respondents were recruited separately for each study, anonymously, from online panels and databases, social media, and the US Hereditary Angioedema Association. Each participant was offered a nominal honorarium for completing the survey.
- Inclusion criteria for both studies included:
  - U.S. residency,
  - age 18+,
  - self-reported diagnosis of Type I or Type II HAE by a healthcare professional,
  - currently treating HAE, or not currently treating HAE and experiences at least one HAE attack every three months.
- Each survey covered a range of topics regarding attitudes towards HAE and prophylactic treatment of the condition, perceptions of prophylaxis medication, treatment experience and satisfaction, and self-reported HAE attack history.
  - Although the surveys were unique, some questions were asked in both surveys for comparison over time.

RESULTS

- 75 and 100 patients participated in the 2018 and 2019 surveys, respectively. Characteristics of both study samples are described in Table 1. Data presented are for questions from both patient studies.

Table 1. Characteristics of 2018 and 2019 HAE Online Survey Respondents

HAE Patient Characteristics*	2018 Respondents (n=75)	2019 Respondents (n=100)
Age (Mean Years)	39.1	41.3
Gender		
Male	49%	26%
Female	51%	74%
Region		
Northeast	20%	25%
Midwest	24%	17%
South	37%	39%
West	19%	19%
Time since HAE Diagnosis (Mean Years)	16.7	19.4
HAE Type		
Type 1	97%	77%
Type 2	3%	23%
Have Health Insurance [% Yes]	96%	92%
Have Prescription Drug Coverage (among those with health insurance) [% Yes]	81%	92%

*Two patients completed both surveys (2018 and 2019)

Figure 1. Medications Currently Taking for HAE Prophylaxis (among HAE Patients Taking Prophylaxis)*

*Defined as anyone who uses an HAE medication for prophylaxis regardless of indication for acute or prophylactic treatment; percentages may sum to >100% due to some patients using more than one medication.

Figure 2. Mean Number of HAE Attacks (Range) in the Past 3 Months among HAE Patients Taking Prophylaxis (for the Most Commonly Taken Medications)

*Outliers removed

- More (85%) patients reported taking at least one medication to prevent HAE attacks in 2019 compared to 64% in 2018. Patients taking medication for HAE prophylaxis have similar characteristics compared to those not taking prophylaxis medication.
  - In the 2019 survey, the majority (89%) of patients treating prophylactically report using at least one of three commonly prescribed injectable or infused medications indicated for HAE prophylaxis (Cinryze®, Haegarda®, and Takhzyro®) (Figure 1).
  - An average of 67% of patients on these medications reported taking them as prescribed every time (2019 survey).
- Mean number of attacks in the past three months for the three most commonly taken medications ranged from 1.0 to 1.6 attacks (2018 survey) and 0.9 to 1.8 attacks (2019 survey) (Figure 2).
- The majority (84%) of patients taking prophylaxis (2019 survey) expected to have fewer than 12 attacks over the next 12 months (Figure 3).
  - In contrast, 72% of patients taking prophylaxis (2019 patients) expected they would have 12 or more attacks in the next 12 months if they were not taking prophylaxis.

Figure 3. Number of HAE Attacks Expected in the Next 12 Months (with and without Prophylaxis) among HAE Patients Taking Prophylaxis (2019 Survey*, n=85)

*Not asked in 2018 Survey
Note: percentages may sum to >100% due to rounding

DISCUSSION AND CONCLUSIONS

1. Patients with HAE [illegible] medications (2018 survey) agreeing that they [illegible] medication but would prefer an oral treatment if available.
4. [illegible] presentation [illegible] medications (2018 survey) agreeing that they [illegible] medication but would prefer an oral treatment if available.
5. The development of new prophylactic medication options that improve ease of treatment could help HAE patients better manage their disease.

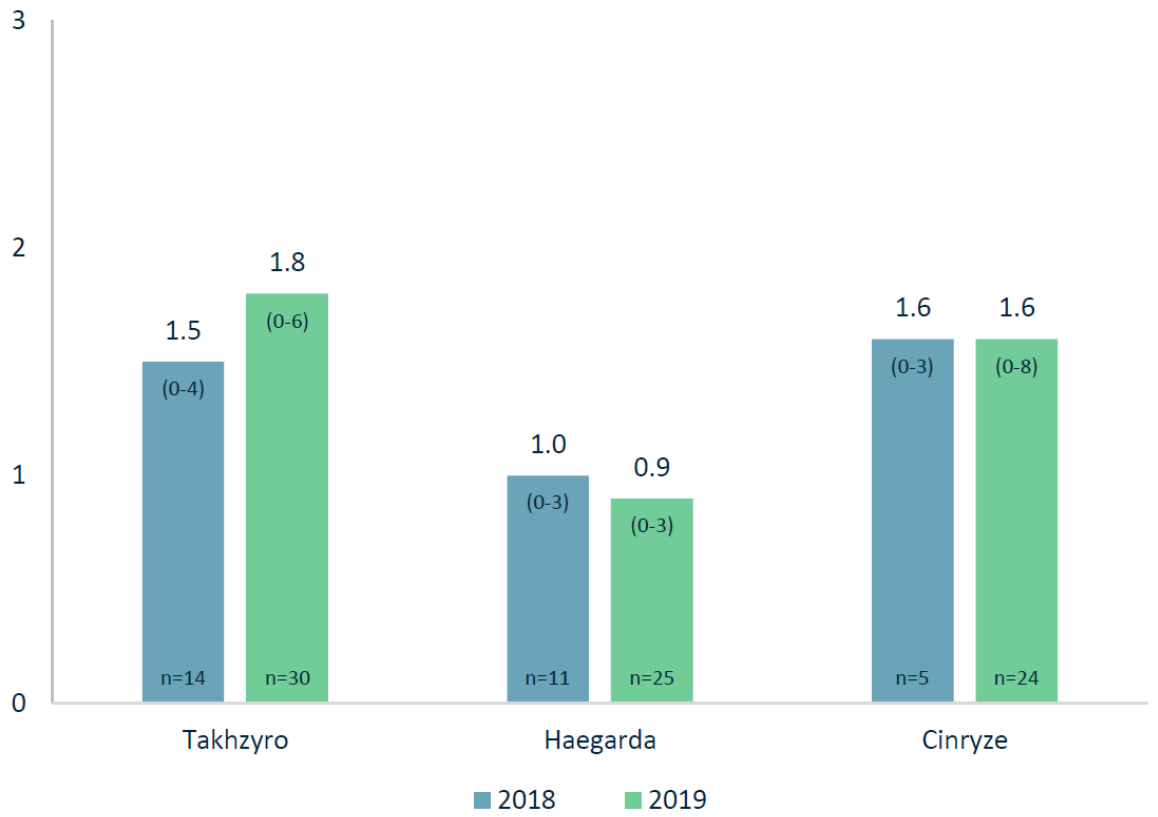
FUNDING AND ACKNOWLEDGMENTS

REFERENCES

Presented at the EAACI Digital Congress 2020 • June 6-8, 2020 • Virtual format

MEAN # OF ATTACKS IN PAST 3 MONTHS (2018-2019 PATIENT SURVEY DATA)

Figure 2. Mean Number of HAE Attacks (Range) in the Past 3 Months among HAE Patients Taking Prophylaxis (for the Most Commonly Taken Medications)



*Outliers removed

Source: Hereditary Angioedema Patients in the United States Report Expanded Use of Prophylaxis, but Continue to Experience Attacks
Presented at the EAACI Digital Congress 2020 · June 6-8, 2020

Approved label: ORLADEYO (berotralstat) safety

In APeX-2 (part 1), the most common treatment-emergent adverse reactions[a] were abdominal pain, vomiting, diarrhea, back pain, and gastroesophageal reflux disease (GERD)

Adverse reactions	Placebo (n=39)	ORLADEYO 110 mg (n=41)	ORLADEYO 150 mg (n=40)
	n (%)	n (%)	n (%)
Abdominal pain[b]	4 (10)	4 (10)	9 (23)
Vomiting	1 (3)	4 (10)	6 (15)
Diarrhea[c]	0	4 (10)	6 (15)
Back pain	1 (3)	1 (2)	4 (10)
GERD	0	4 (10)	2 (5)

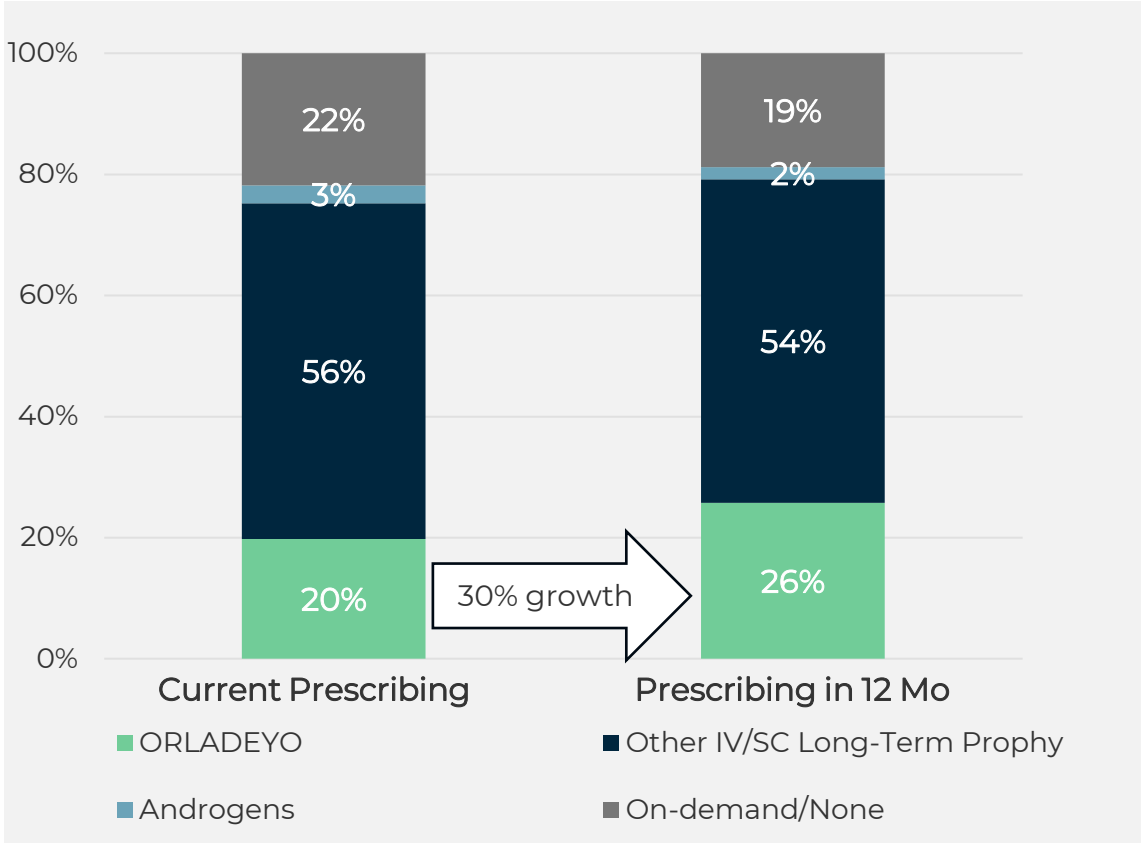
Findings from the open-label, long-term safety study, APeX-S (interim safety population, n=227), support the data observed in APeX-2 (part 1)

[a] ≥10% and higher than placebo.
[b] Includes abdominal pain, abdominal discomfort, abdominal tenderness, and upper abdominal pain.
[c] Includes diarrhea and frequent bowel movements.

MARKET RESEARCH: Intent to prescribe more ORLADEYO remains consistent, with prescriptions coming mostly from prophylactic switches

ALLERGIST-IMMUNOLOGISTS (A/IS) PRESCRIBING - 2023

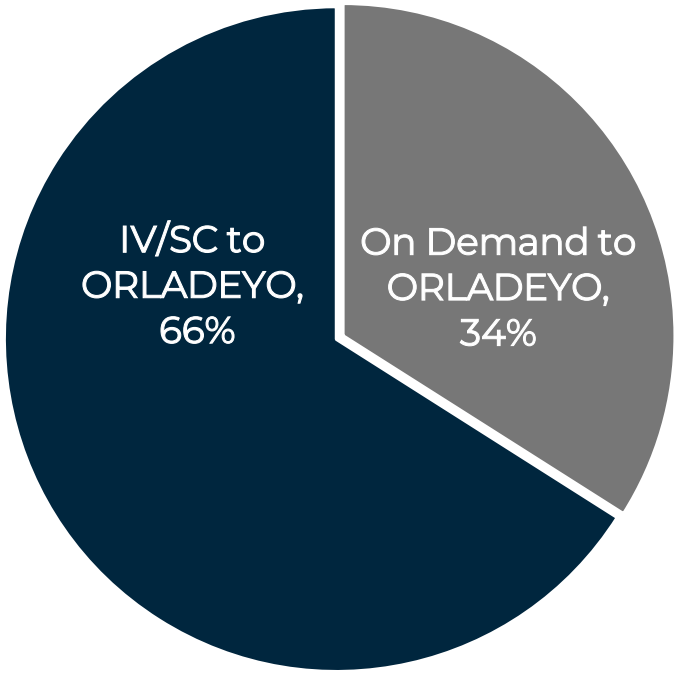
(n=154 unique A/Is managing ~1,300 HAE patients)



Future **ORLADEYO** prescribing has been <u>consistent throughout all of 2022-23</u>

SOURCE OF FUTURE NEW ORLADEYO PRESCRIPTIONS - 2024

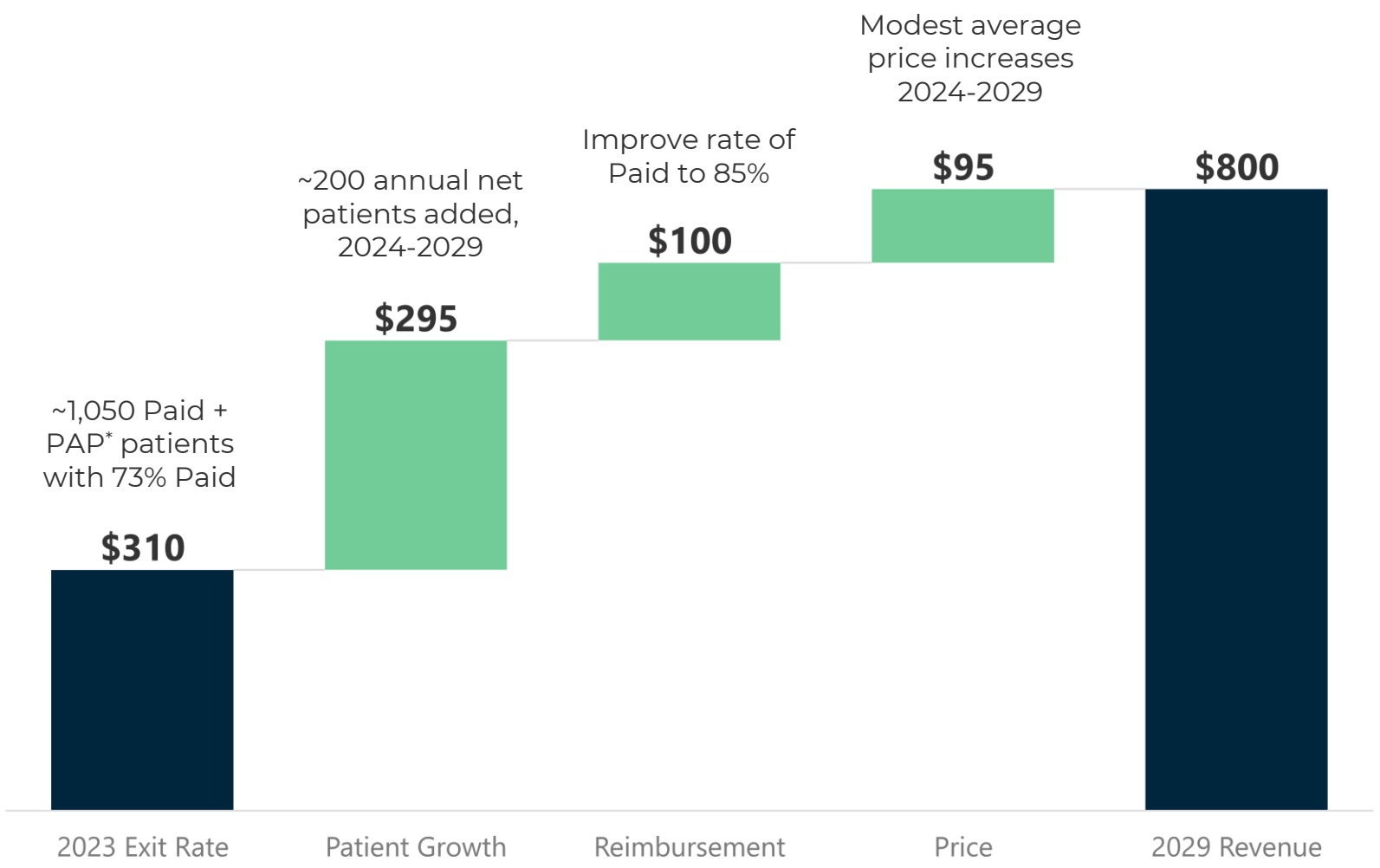
(n=154 unique A/Is managing ~1,300 HAE patients)



A/Is expect future new ORLADEYO prescriptions to come ~**2/3 from** prophylaxis switches and ~**1/3 from On-Demand Only**

Source: BioCryst Internal Market Research Studies (Conducted Feb 2023, May 2023, Sep 2023, and Dec 2023)

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

Finance summary

(FIGURES IN MILLIONS)

Q4 2023 CASH POSITION	
Cash, cash equivalents, restricted cash & investments at December 31, 2022	$444
Cash, cash equivalents, restricted cash & investments at December 31, 2023	$391
Senior credit facility[A]	$314

2024 FY GUIDANCE	
ORLADEYO revenue	$380-400
Operating expenses (excluding non-cash comp)	$365-375

A – From Pharmakon Advisors, $300M drawn at issuance in Q2 2023. The $314M balance above represents $300M initial issuance plus PIK interest to-date (eligible to PIK 50% per quarter for first six quarters).

Fourth Quarter and Full Year 2023 Results Call

Corporate Update & Financial Results

February 26, 2024