

# Clinical and Commercial Update

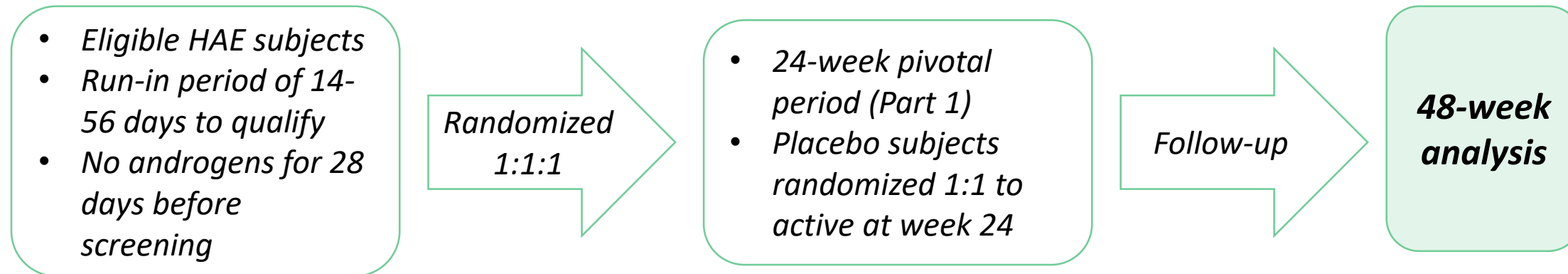
November 6, 2019



# Forward-Looking Statements

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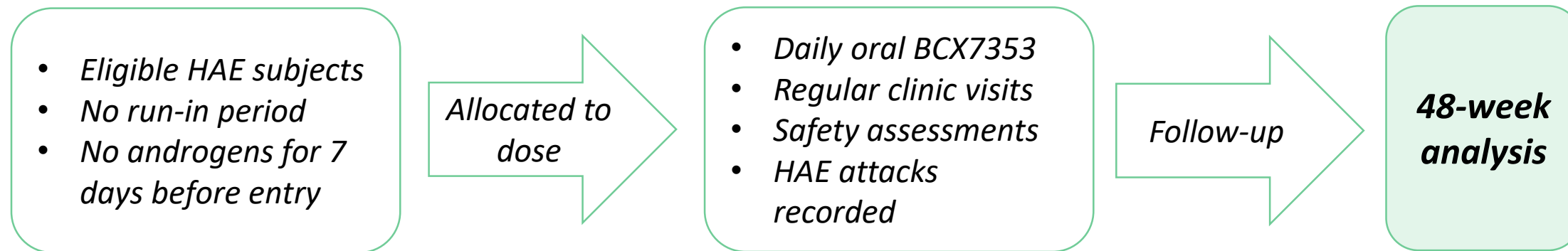
# APeX-2 Study – 48-week Analysis



Subjects Enrolled and Follow-up	BCX7353 110 mg	BCX7353 150 mg	Placebo	
Subjects enrolled [ITT Population]	41	40	40	
Subjects enrolled and dosed [Safety Population]	41	40	39	
Subjects who completed 24 weeks of study drug (Part 1)	37	37	34	
<i>Randomization to active drug at conclusion of Part 1 for placebo subjects</i>	-	-	<b>110 mg</b>	<b>150 mg</b>
<i>Placebo subjects who were randomized (110 mg:150 mg) at 24 weeks</i>	-	-	17	17
Subjects continuing on study, not yet reaching 48 weeks of study drug	3	1	1	1
Subjects who discontinued study drug between 24 and 48 weeks	9	6	2	3
Subjects who completed 48 weeks of study drug* [Completers Population]	25 (61%)	30 (75%)	14 (82%)	13 (76%)
<i>Weeks of BCX7353 treatment for Completers Population</i>	48	48	24	24
Previous Prophylactic Treatments for HAE	BCX7353 110 mg	BCX7353 150 mg	Placebo	
Androgens	19 (46%)	21 (53%)	25 (63%)	
C1-INH	16 (39%)	21 (53%)	16 (40%)	

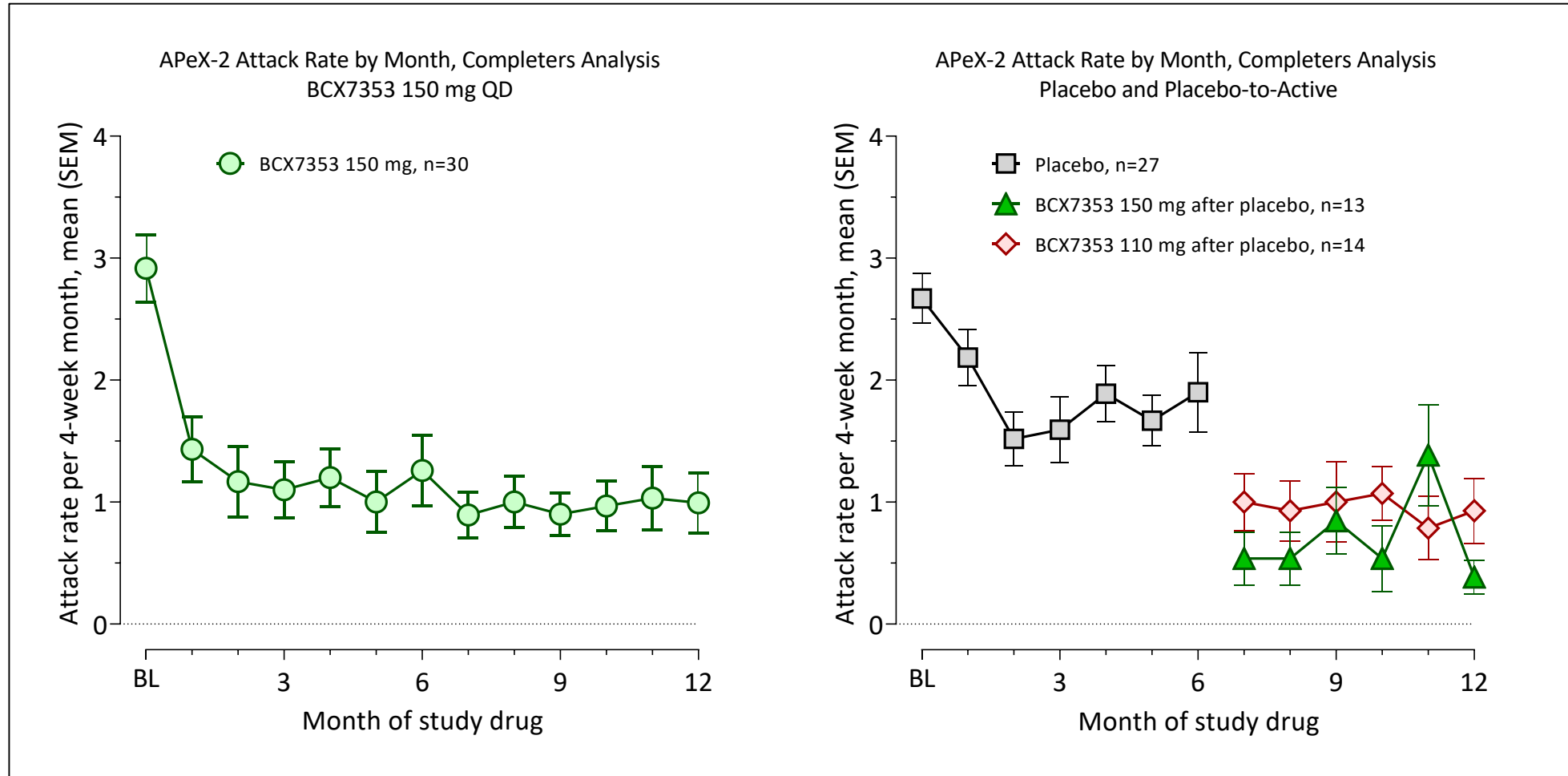
\* Study drug includes BXC7353 through the 48-week visit or placebo through the 24-week visit followed by BCX7353 through the 48-week visit (i.e., for 24 weeks).

# APeX-S Study – 48-week Analysis

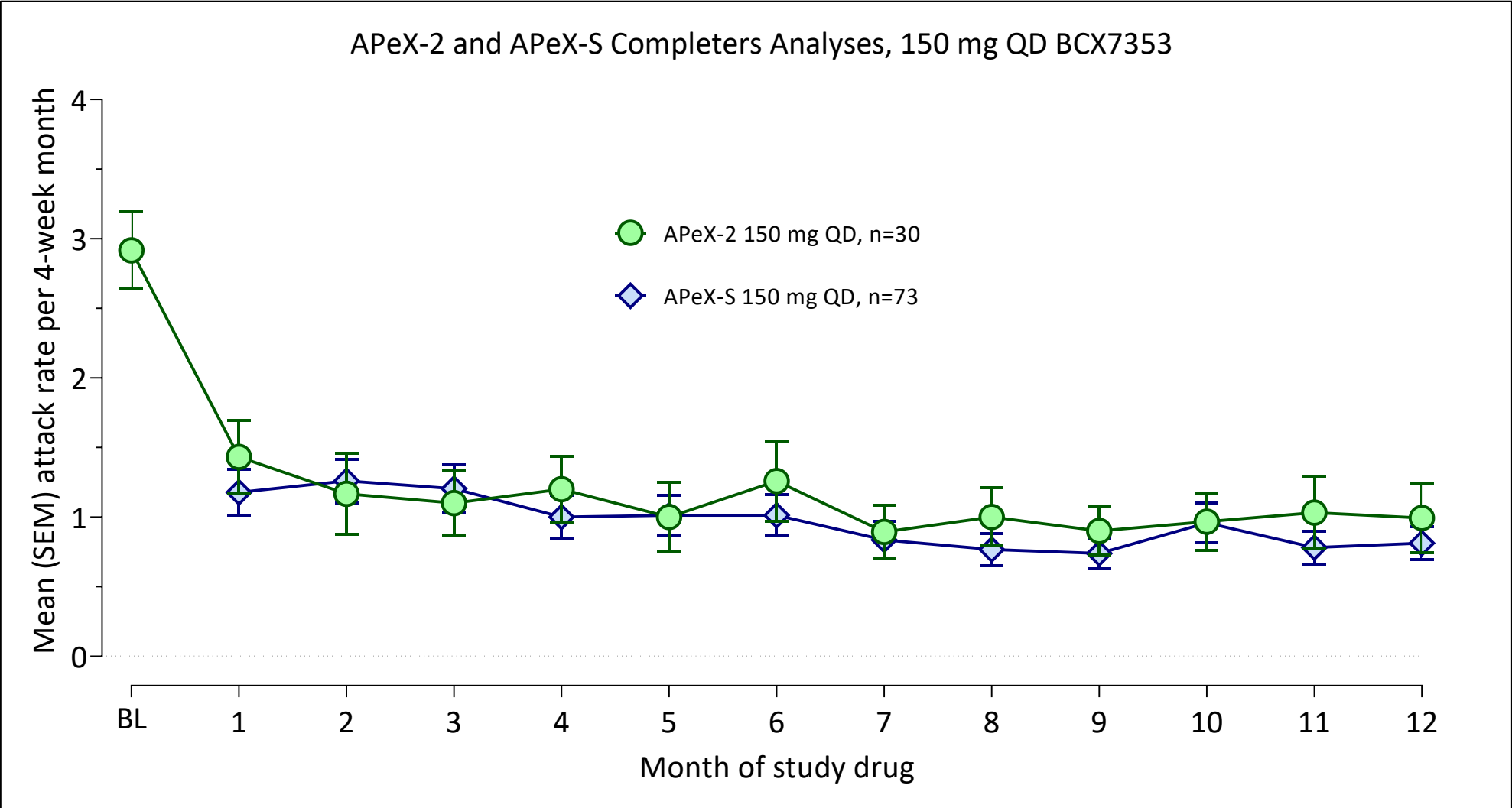


<b>Subjects Enrolled and Follow-up</b>	<b>BCX7353 110 mg</b>	<b>BCX7353 150 mg</b>
Subjects enrolled [Safety Population]	100	127
Subjects continuing on study, not yet reaching 48 weeks of study drug	44	23
Subjects who discontinued study drug before 48 weeks	26	31
Subjects who completed 48 weeks of BCX7353 [Completers Population]	30	73
<b>Past Prophylactic Treatment of HAE</b>	<b>BCX7353 110 mg</b>	<b>BCX7353 150 mg</b>
Androgens	69 (69%)	84 (66%)
C1-INH	22 (22%)	32 (25%)

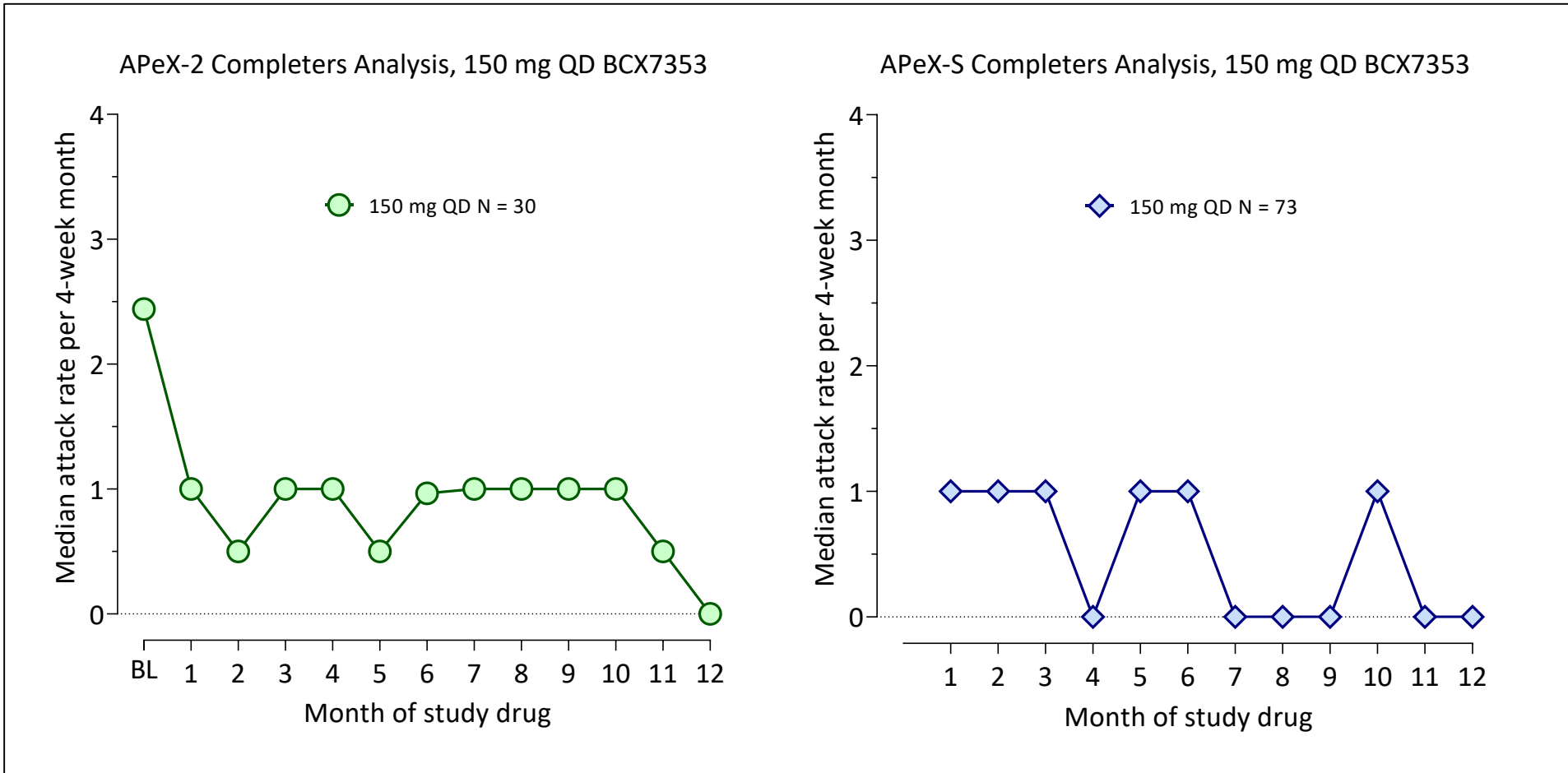
# Rapid and Sustained HAE Attack Rate Reduction: Analyses for APeX-2: 150 mg and Placebo 48-week Completers



# Consistent Mean Attack Rates in APeX-2 and APeX-S



# Median Attack Rates in 48-week Completers: *Zero Attacks per Month in 6 of 12 Months in APeX-S*



# Safety and Tolerability Confirmed in Integrated 48-week Analysis

Integrated Safety Summary – APeX-2 and APeX-S	BCX7353 110 mg	BCX7353 150 mg	Placebo
<b>Subjects enrolled and dosed [Safety Population]</b>	<b>N = 158</b>	<b>N = 184</b>	<b>N = 39</b>
<b>Subject Incidence of SAEs or Discontinuations due to AEs</b>			
Drug-Related Serious AEs	2 (1.3%) <sup>1, 2</sup>	1 (0.5%) <sup>3</sup>	0
AEs Leading to Discontinuation of Study Drug			
Abdominal GI AEs <sup>4</sup>	4 (2.5%)	7 (3.8%)	0
Abnormal Liver Function Test	3 (1.9%)	6 (3.3%)	0
Other AEs	4 (2.5%) <sup>5</sup>	5 (2.7%)	1 (2.6%)
<b>Subject Incidence of Most Common GI Abdominal AEs Reported as Drug-Related<sup>6</sup></b>			
Gastrointestinal Disorders System Organ Class	62 (39.2%)	65 (35.3%)	11 (28.2%)
Nausea	10 (6.3%)	15 (8.2%)	6 (15.4%)
Abdominal pain	14 (8.9%)	16 (8.7%)	0
Diarrhea	10 (6.3%)	15 (8.2%)	0
Flatulence	4 (2.5%)	11 (6.0%)	1 (2.6%)
Abdominal pain upper	9 (5.7%)	7 (3.8%)	1 (2.6%)
Dyspepsia	8 (5.1%)	10 (5.4%)	2 (5.1%)
Abdominal discomfort	7 (4.4%)	6 (3.3%)	2 (5.1%)
Abdominal distension	5 (3.2%)	8 (4.3%)	2 (5.1%)
Vomiting	4 (2.5%)	7 (3.8%)	0
<p>1: Gastroenteritis and hepatic enzyme increased in the same subject, events resolved after stopping study drug (ApeX-S)</p> <p>2: Abdominal pain, event resolved after interrupting study drug (ApeX-S)</p> <p>3: LFT abnormal, event resolved after stopping study drug (ApeX-S)</p> <p>4: GI abdominal-related AEs were any AEs with a PT within the MedDRA 19.1 hierarchy under the high level group terms of GI signs and symptoms or GI motility and defecation conditions</p> <p>5: One subject in this category had an infection and abnormal LFTs and is also counted in that row</p> <p>6: For GI abdominal AEs occurring with a rate of at least 3% of BCX7353-treated subjects</p>			



# Data Supports 4-week Washout Period for Current Androgen Users

APeX-S Analysis	BCX7353 110 mg				BCX7353 150 mg			
	<i>All Subjects</i>	<i>Discontinued ≤ 1 month before BCX7353</i>	<i>Discontinued &gt; 1 month before BCX7353</i>	<i>Never used</i>	<i>All Subjects</i>	<i>Discontinued ≤ 1 month before BCX7353</i>	<i>Discontinued &gt; 1 month before BCX7353</i>	<i>Never used</i>
<i>Prior Androgen Rx Status</i>								
<i>Number of Subjects</i>	<i>N = 100</i>	<i>N = 26</i>	<i>N = 43</i>	<i>N = 31</i>	<i>N = 127</i>	<i>N = 23</i>	<i>N = 61</i>	<i>N = 43</i>
Subject Incidence of Post-Baseline ALT > 3xULN	9 (9.0%)	8 (30.8%)	1 (2.3%)	0	6 (4.7%)	6 (26.1%)	0	0

## Assessment:

- *There is a strong association of recent discontinuation of androgens with post-baseline elevations in ALT*

A photograph of a woman in a pink tank top and jeans holding a young child in a white shirt and dark pants over a body of water. The child is being held horizontally, as if about to be thrown or is being held up. The background shows a shoreline with trees and a clear sky. The image has a blue tint and is overlaid with a grid of white and blue circles in the top right corner.

# BCX7353 Market Research Update

# Robust Market Research Since APeX-2



## Market Sizing

- US prevalence study using administrative claims data

## US HAE Patients

- 100 quantitative, 25-minute online surveys
- 26 individual, 60- to 75-minute qualitative interviews

## US Physicians

- 175 quantitative, 20-minute online surveys
- 43 individual, 60- to 75-minute qualitative interviews

## US Payors

- 16 interviews with medical and pharmacy directors from insurance plans and PBMs covering >100 million lives

# Administrative Claims Analysis Estimates US HAE Population at ~10,000 Patients with ~7,500 Diagnosed & Treated

## Data Source:

Administrative claims from Symphony Integrated Dataverse (IDV) from 2017-2019 for >270 million US patients

### HAE Patient cohorts

1. Diagnosed and treated with HAE-specific medication
2. Diagnosed but not treated with HAE-specific medication
3. Treated with HAE-specific medication but not diagnosed

### Claims Variables

- Recurring claims with HAE ICD-9/10 diagnosis codes
- Complement function and/or level tests
- Recurring claims for HAE-specific medications

### National projections\*

- 1. ~7,500 patients diagnosed and treated**
2. ~1,700 patients diagnosed but not treated
3. ~600 patients treated but not diagnosed



# Large, Quantitative Market Research Studies with US Patients and HAE-treating Physicians in July 2019 with 24-week APeX-2 Profile

**100 HAE Patients**

- 25-minute online survey
- Age 18+, diagnosed with Type I or II HAE
- Currently treating HAE or not currently treating and has 1+ attack every 3 months
- 50% recruited from HAEA patient organization
- 50% recruited via social media and online panels

**175 HAE-Treating Physicians**

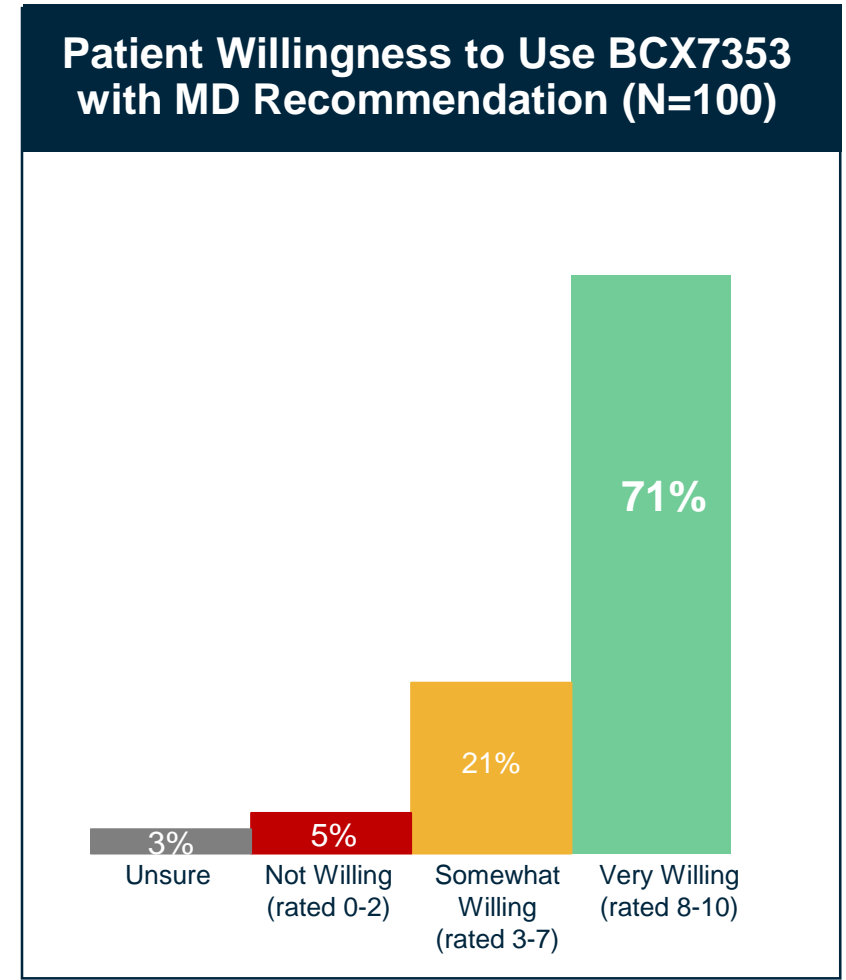
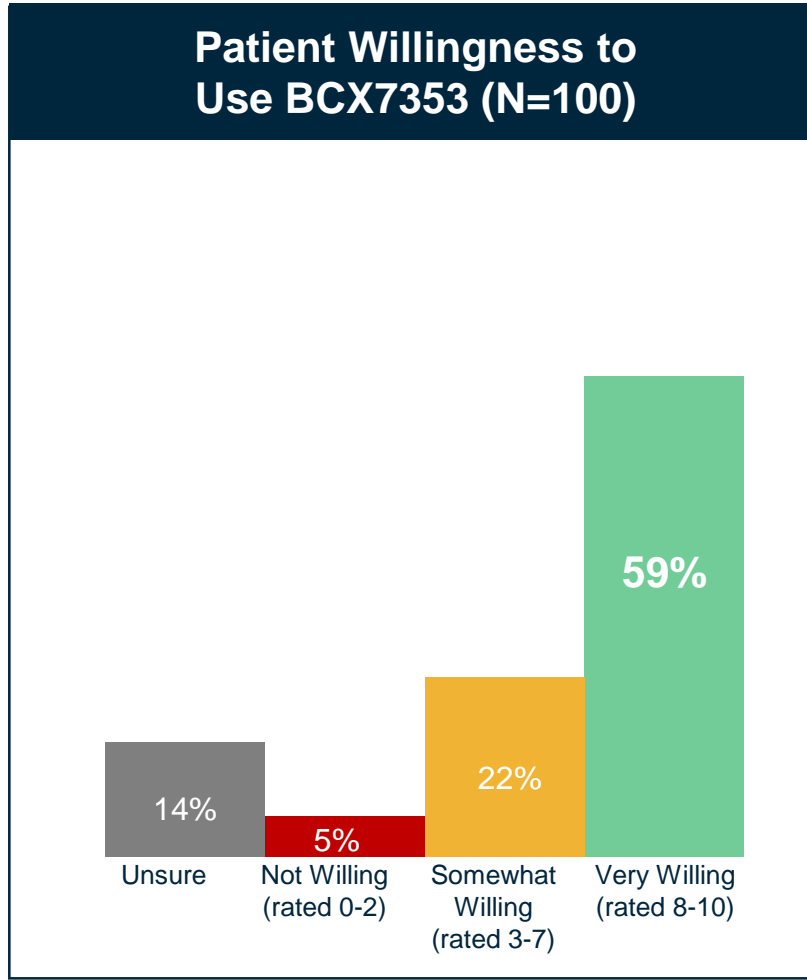
- 20-minute online survey
- Allergist/Immunologist (n=100)
- Other specialty (n=75)
- Actively treats 2+ Type I or II HAE patients per year
- Study average = 7.6 patients/year
- Recruited via email and online panels

Physicians in this study treat 1,300 HAE patients representing over 10% of US HAE patients

# Respondents Viewed a Blinded Profile of BCX7353 Based on 24-week Results from APeX-2

<b>Indication</b>	<u>Prophylactic</u> treatment of HAE for patients 12 years and above
<b>Dosage</b>	Take 1 capsule by mouth once per day
<b>Clinical trial design</b>	Patients who were experiencing an average of 3 HAE attacks per month took Treatment X or a placebo (an inactive drug often used in clinical trials) for 6 months
<b>Efficacy</b>	<p>Patients taking Treatment X had 44% fewer HAE attacks overall than patients taking a placebo during the 6-month clinical trial</p> <p>Half (50%) of patients taking Treatment X reduced their number of HAE attacks by 70% or more between the beginning and end of the trial</p> <p>About 1 in 4 patients (23%) taking Treatment X reduced their number of HAE attacks by 90% or more between beginning and end of the trial</p>
<b>Safety and tolerability</b>	<p>Adverse events from Treatment X were generally mild and similar to placebo</p> <p>The most common side effects experienced more often with Treatment X were short episodes of mild diarrhea or vomiting experienced by about 10% of patients</p>

# Strong HAE Patient Demand for BCX7353: 59% of Patients Expressed High Willingness to use BCX7353 Rises to 71% with Physician Recommendation



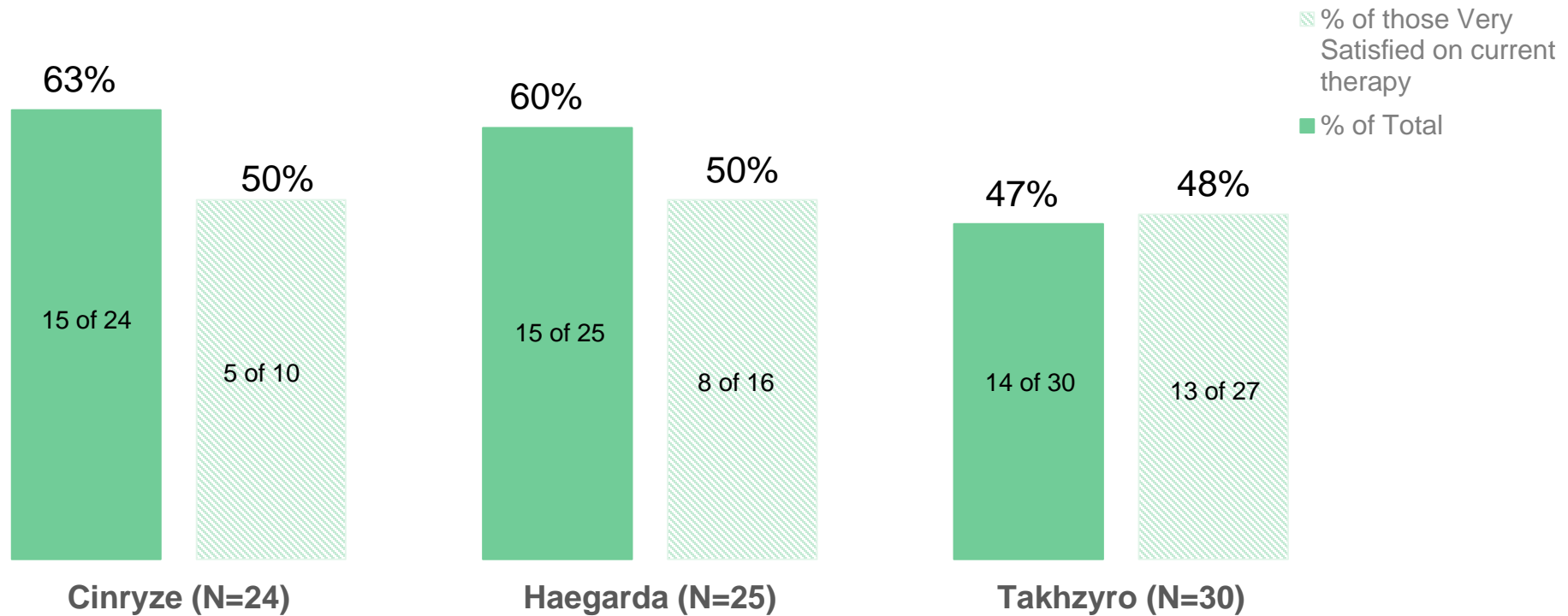
All Qualified HAE Patients (n=100)  
Rated on a scale where a "0" indicates "Not at all willing", and a "10" indicates "Extremely willing"



# Prophylaxis Patients are Very Willing to Use BCX7353—Even Those Very Satisfied with their Current Injectable Prophylactic Treatment



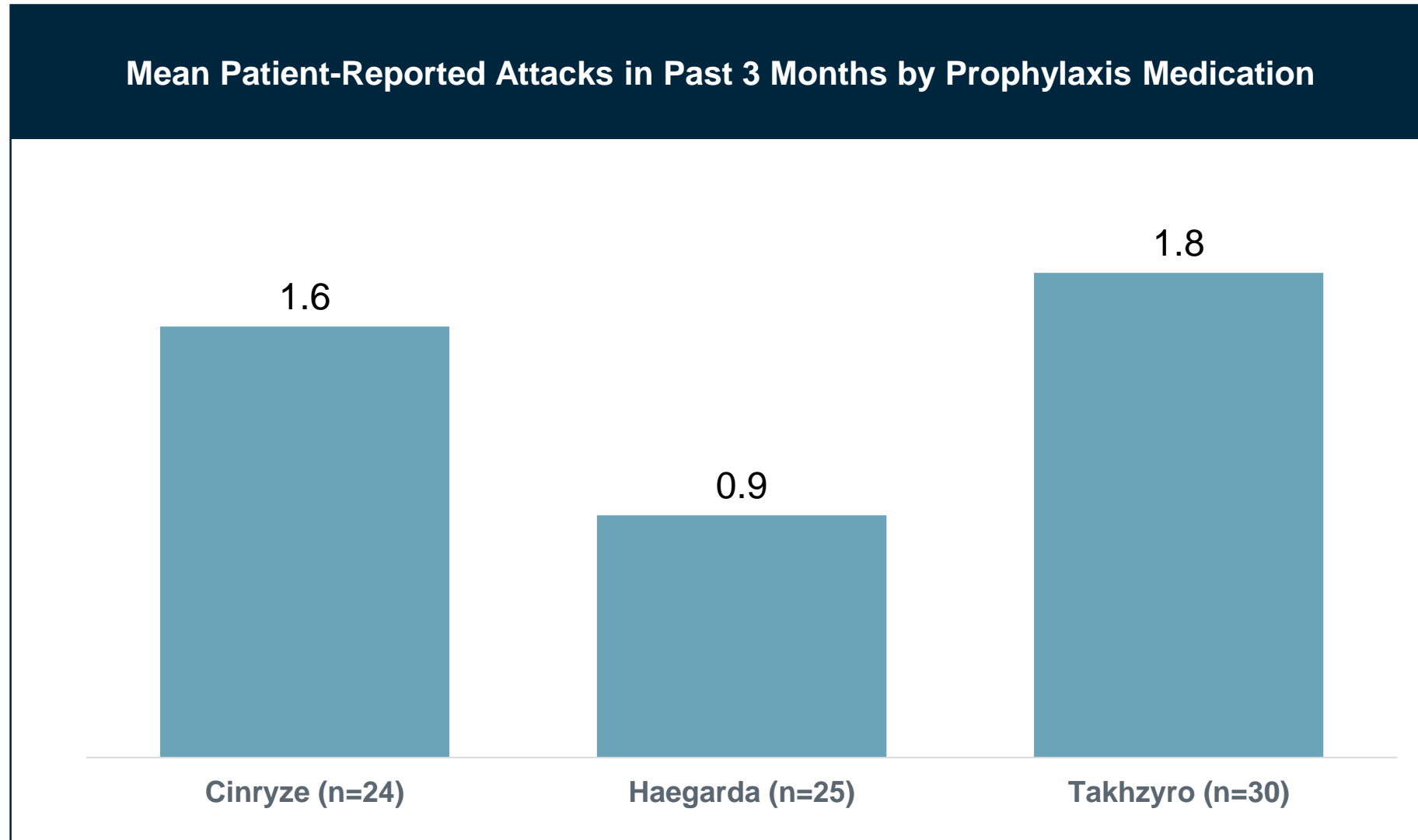
## Prophylaxis Patients VERY WILLING to Use BCX7353



All Current Prophylaxis Users- "Very Willing" & "Very Satisfied" = Top 3 Box (rated 8,9,10 on 10 point scale)  
 Willingness rated on a scale where a "0" indicates "Not at all willing", and a "10" indicates "Extremely willing"  
 Satisfaction with current treatment rated on a scale where a "0" indicates "Not at all satisfied", and a "10" indicates "Extremely satisfied"



# Patients Report Breakthrough Attacks with Injectable/Infused Treatments



*Currently Taking Medication Prophylactically*

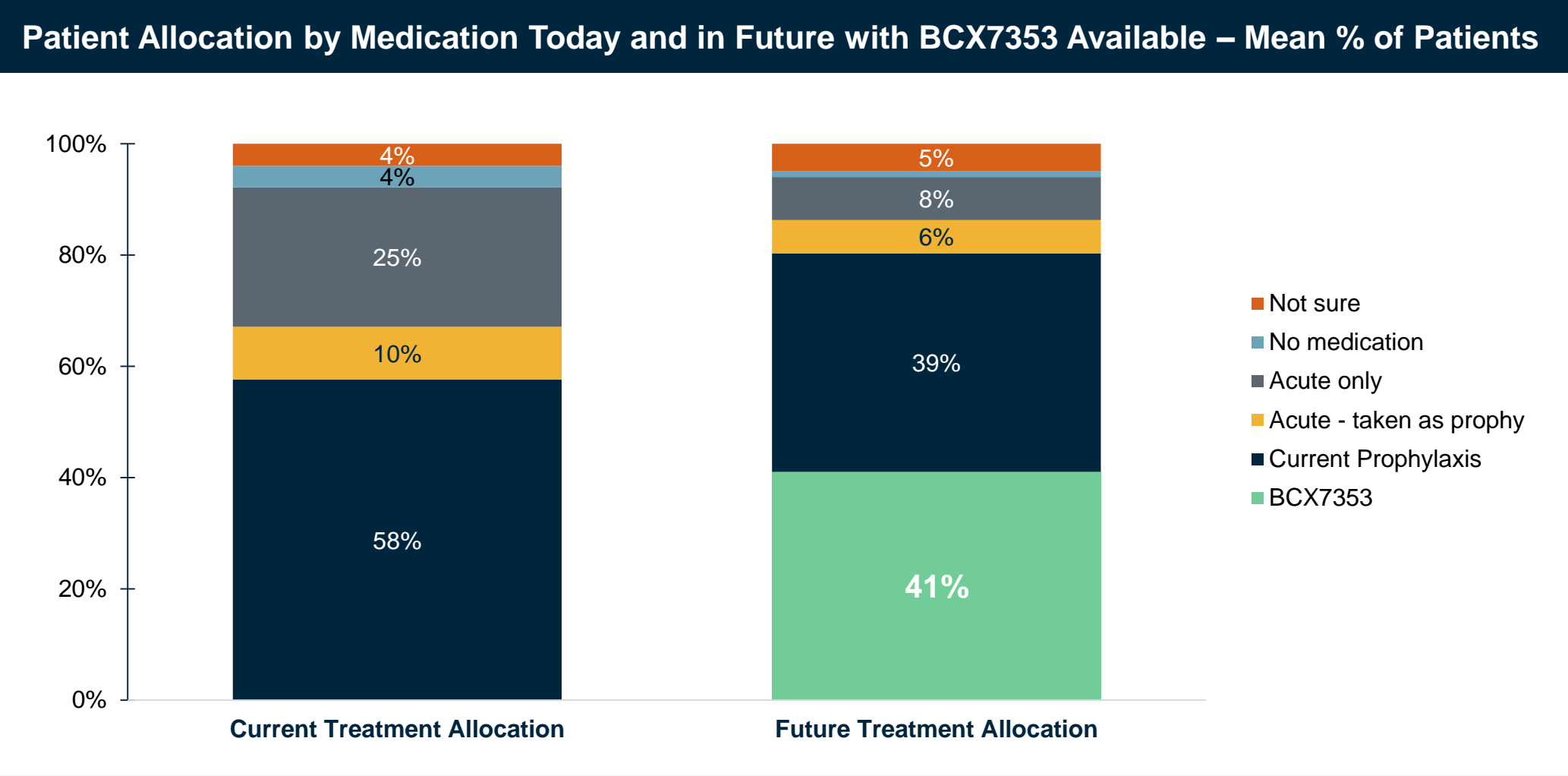
# Patients Are Coping With Their Injectable Therapies

## *They Want the Ease that Only BCX7353 can Offer*

Time saving	<i>“Make HAE less a part of my life...less time consuming...getting on with my day. A lot of times I am so rushed and <b>I don’t have the time to do simple things</b>, like do laundry or go grocery shopping. Being able to do this quickly...this is one thing that will be a lot simpler and help with my overall day.”</i>
Less to think about and coordinate	<i>“<b>You can focus on doing other things</b> that are more important in life. I’d rather spend my time doing other things...going out with friends, spending time with my grandson.”</i>
Less hassle— inconvenience	<i>“<b>Less of a hassle for me</b>; I work full time, I have two kids and I have a stressful, difficult life...anything I can do to prevent attacks in an easier way is less of a burden on me. Injections can be burdensome, injection site reactions, pain and swelling, dizziness.”</i>
Less burden	<i>“With medications, you have to make sure that they’re kept in a fridge, you have to make sure that when you take them to travel, that they don’t get too hot, you have to bring ice packs, you have to bring coolers. The fact that you don’t have to do that with this, again, just makes it easier. You <b>don’t have to worry about keeping it a certain temperature.</b>”</i>
Not painful	<i>“<b>Less painful, not that using needles is all that painful, but it would be less painful.</b> Probably less work behind it. Even if it’s just drawing the medication out of a vial, it’s still some work that you have to do. I’d almost say that it’s safer because you’re not injecting something into your blood, or on your skin. You’re swallowing a pill.”</i>
Better routine	<i>“<b>Most people have a morning routine</b>, whether it’s vitamins or taking other medication...so it would be easier than remembering every two weeks. Not worrying about the shipment, keeping it refrigerated, bent needles, the prep, etc.”</i>

# Physicians Expect to Prescribe BCX7353 for Over 40% of HAE Patients

## 80% of HAE Patients Expected to be on Some Form of Prophylaxis

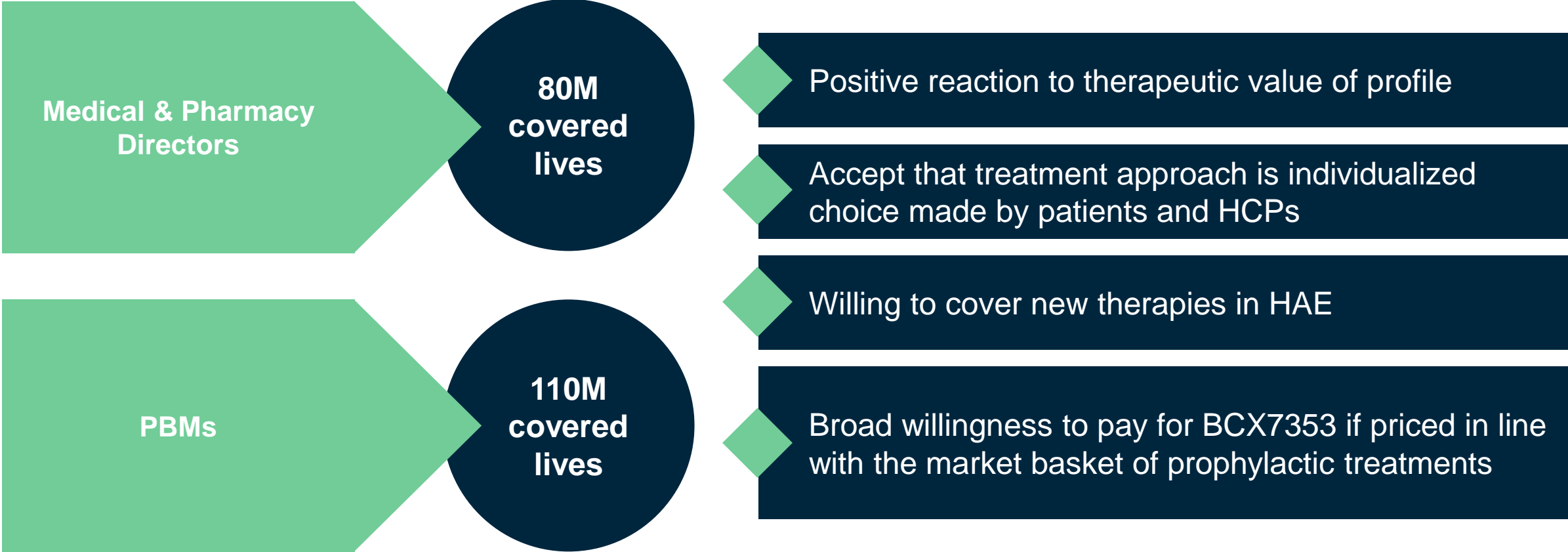


All Qualified Respondents (n=175)



Source: BioCryst Proprietary Market Research Study, 2019. Respondents were shown a blinded profile (Treatment X) of BCX7353, 24-week results of APeX-2, Physicians were asked to perform a patient allocation.

# US Payors Anticipate Providing Coverage for BCX7353



# Preparing for a Successful Commercial Launch



Building out critical launch elements based on our detailed market understanding

- Marketing strategy, messages and tactics
- Sales force structure and targeting
- Market access strategies



Deployed competitively-sized MSL team to call on top-tier HAE treaters



Attracting commercial leadership with extensive record of success in rare disease



Developing a best-in-class patient services and hub program

# Data Supports Global Peak Market Opportunity >\$500M



**Clinical Data**

Consistent, clinically meaningful benefit demonstrated through 48 weeks

Safe and generally well-tolerated

**Prevalence**

~10,000 (US) HAE Patients

~7,500 diagnosed and treated

**Treatment Paradigm**

Physicians expect shift to ~80% prophylaxis

**Strong Demand for BCX7353 Product Profile and Benefit**

Overall, 60-70% of patients very willing to use  
Physicians intending to prescribe to >40% of patients  
Payers acknowledge therapeutic value and broad willingness to pay