APeX-2 Topline Results Conference Call

May 21, 2019

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Agenda

- Introduction

  Jon Stonehouse – President, Chief Executive Officer

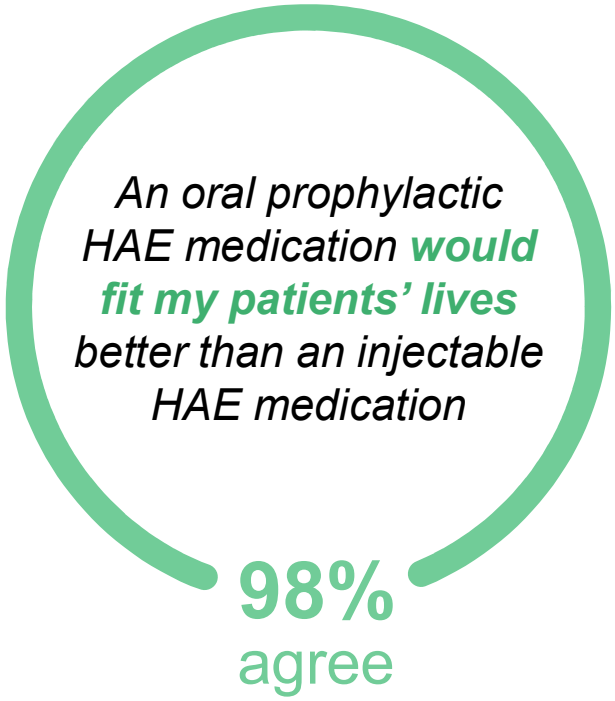
- APeX-2 Data and Key Findings

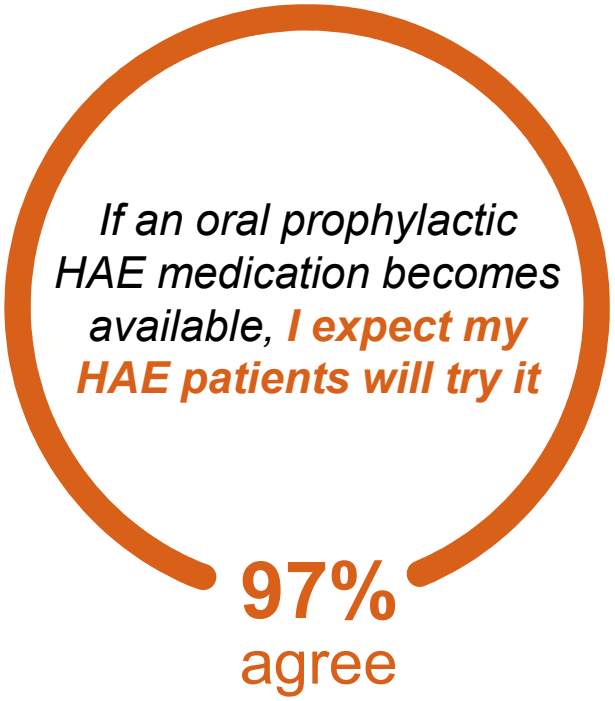
  Dr. Bill Sheridan – Chief Medical Officer

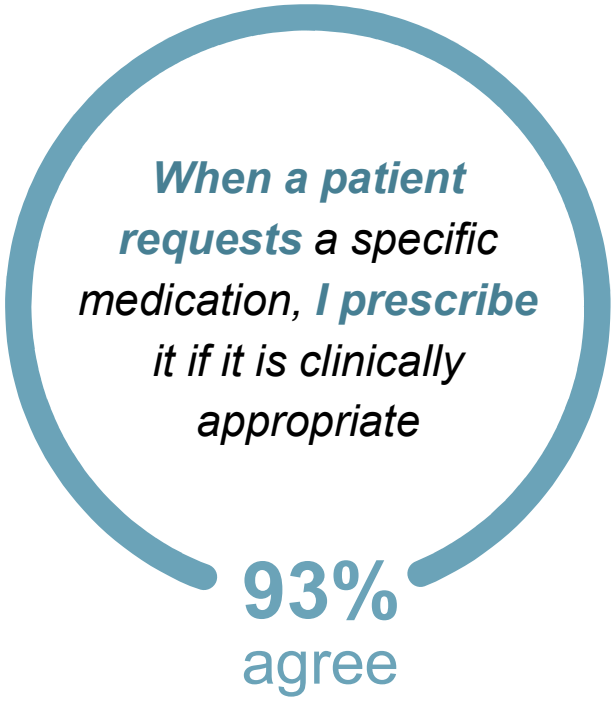
- Summary and Q&A

High Demand to Try a New Oral Therapy

US allergist survey: November 2018 (n=100)



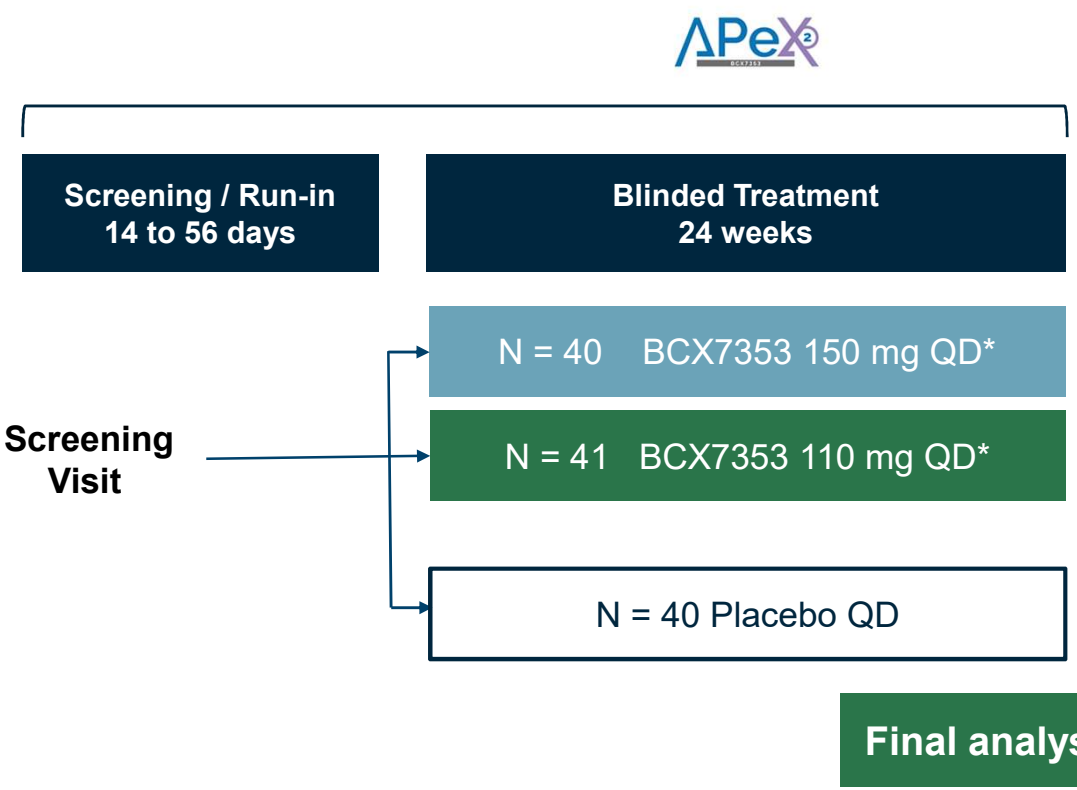




ALL QUALIFIED RESPONDENTS
Q600-609 Please read the following statement and indicate if you agree or disagree.

APeX-2 Data and Key Findings

APeX-2 Study



- Primary endpoint at Week 24:
  - Rate of Investigator-confirmed HAE attacks through entire treatment period
- With N=121, study powered at 99% to detect a ≥50% reduction in attack rate over placebo

Final analysis @ Week 24

*Doses in Phase 2 APeX-1 were shown as the dihydrochloride salt:
150 mg = 175 mg dihydrochloride salt; 110 mg = 125 mg dihydrochloride salt

Study Population Characteristics

Parameter	BCX7353 110 mg	BCX7353 150 mg	Placebo
N	41	40	40
Baseline attack rate, mean (SD)	3.0 (1.4)	3.1 (1.6)	2.9 (1.1)
Baseline attack rate (stratified)			
≥ 2/month, n (%)	28 (68%)	30 (75%)	27 (68%)
< 2/month, n (%)	13 (32%)	10 (25%)	12 (30%)
Prior androgen prophylactic Rx, n (%)	19 (46%)	21 (53%)	25 (63%)
Prior C1-INH prophylactic Rx, n (%)	16 (39%)	21 (53%)	16 (40%)

Efficacy Results – Primary Endpoint

Primary endpoint: investigator-confirmed angioedema attacks, rate/month					
Arm	N*	Rate	Attack rate ratio active/placebo (95% CI)	Percent reduction from placebo (95% CI)	P value‡
BCX7353 150 mg	40	1.31	0.56 (0.41, 0.77)	44.2 (23.0, 59.5)	< 0.001
BCX7353 110 mg	41	1.65	0.70 (0.51, 0.95)	30.0 (4.6, 48.7)	0.024
Placebo	39	2.35	-	-	-

** One of 40 placebo subjects did not receive blinded study drug and did not contribute attack rate information after randomization*
‡ Statistical analysis is based on a negative binomial regression model

Responder Analyses for BCX7353 150 mg

Response outcome	Placebo n=39*	BCX7353 150 mg n=40		
	Percent	Percent	Odds ratio	P value ‡
≥ 50% reduction	25.0	57.5	3.9	0.005
≥ 70% reduction	**15.0**	**50.0**	**5.6**	**0.002**
≥ 90% reduction	7.5	22.5	3.6	0.073

** One of 40 placebo subjects did not receive blinded study drug and did not contribute attack rate information after randomization*
‡ Logistic regression model

Primary Endpoint Analysis for BCX7353 150 mg by Baseline Attack Rate Stratification Factor

Primary endpoint: subgroup analysis on stratification factor					
Arm	N*	Rate	Attack rate ratio active/placebo (95% CI)	**Percent reduction from placebo (95% CI)**	P value
Subjects with baseline attack rate < 2 per month					
Placebo	12	1.45	-	-	-
BCX7353 150 mg QD	10	0.50	0.34 (0.15, 0.76)	**65.7 (23.8, 84.5)**	0.009
Subjects with baseline attack rate ≥ 2 per month					
Placebo	27	2.92	-	-	-
BCX7353 150mg QD	30	1.76	0.60 (0.42, 0.86)	**39.9 (14.4, 57.8)**	0.005

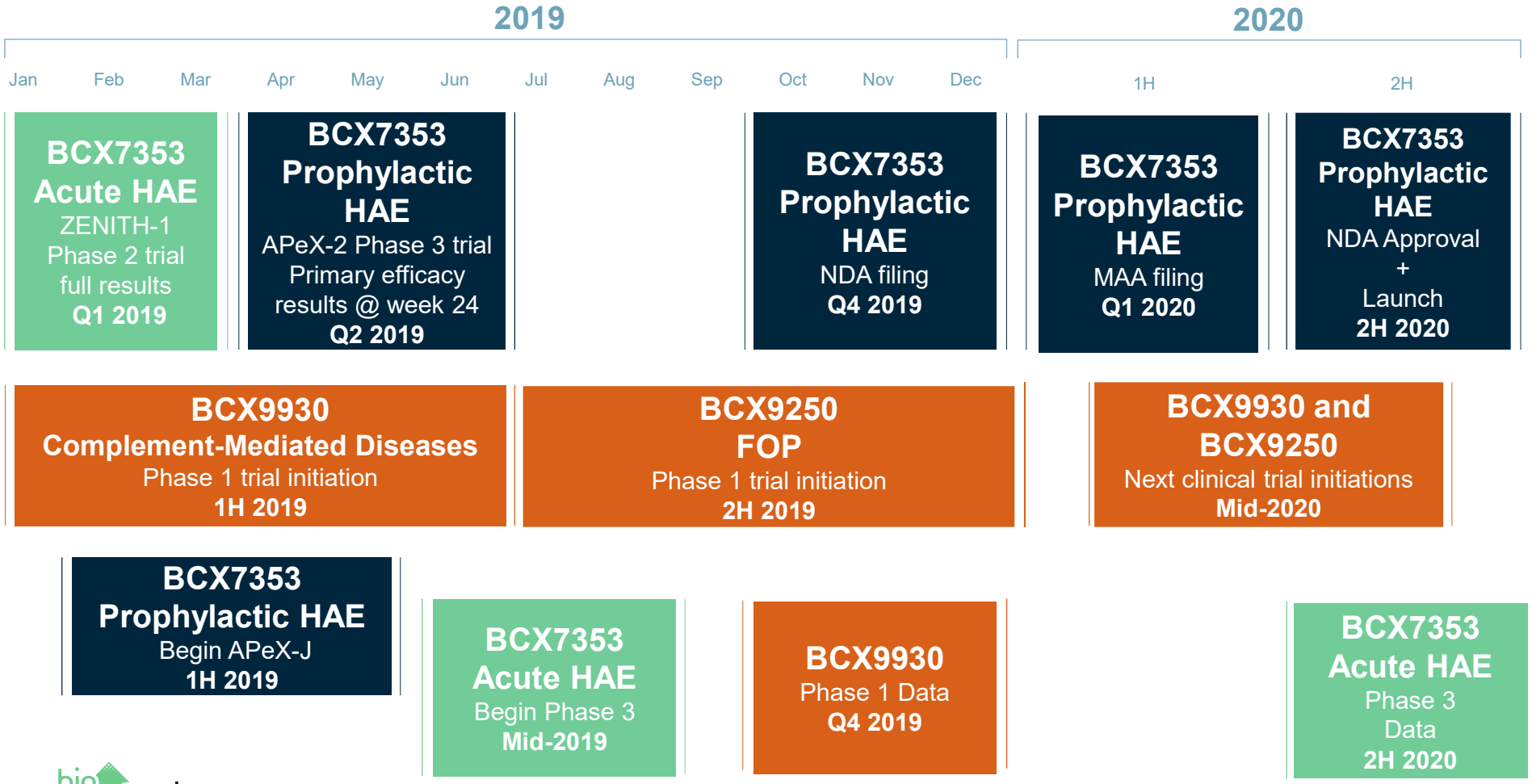
** One of 40 placebo subjects did not receive blinded study drug and did not contribute attack rate information after randomization*

Overall Safety Summary

Treatment-emergent (TE) Adverse Events (AEs) or Discontinuations (DCs) due to TEAEs	BCX7353 110 mg	BCX7353 150 mg	Placebo
	N = 41	N = 40	N = 39*
Drug-Related TE Serious AEs	0	0	0
Most common Drug-Related AEs			
Nausea	4 (9.8%)	3 (7.5%)	6 (15.4%)
Dyspepsia	4 (9.8%)	3 (7.5%)	2 (5.1%)
Diarrhea	3 (7.3%)	4 (10.0%)	0
DCs due to TEAEs	3 (7.3%)	1 (2.5%)	1 (2.6%)
DCs due to Abdominal-Related GI TEAEs	1 (2.4%)	0	0
DCs due to Abnormal Liver Function Test	0	1 (2.5%)	0

** One of 40 placebo subjects did not receive blinded study drug and did not contribute attack rate information after randomization*

Many Anticipated Milestones in 2019 - 2020



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