Jefferies 2017 London Healthcare Conference

November 15, 2017

Jon Stonehouse, *President & Chief Executive Officer*

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BIOCRYST PHARMACEUTICALS, INC.

BioCryst's robust pipeline

	Lead optimization	Pre-clinical	Ph 1	Ph 2	Ph 3	Filed	Approved
STRATEGY: Develop oral therapies for life-threatening, rare diseases							
BCX7353 – Oral (Prophylactic HAE)							
BCX7353 – Oral Liquid Formulation (Acute HAE)							
Second generation kallikrein inhibitors (HAE & Other Indications)							
Rare disease 1							
Rare disease 2							
SUPPORTING ASSETS: Externally funded, potential for significant capital infusions							
RAPIVAB® (peramivir injection)*							
Galidesivir (broad spectrum antiviral) I.M.							
Galidesivir (broad spectrum antiviral) I.V.							

*licensed to Seqirus, Shionogi and Green Cross

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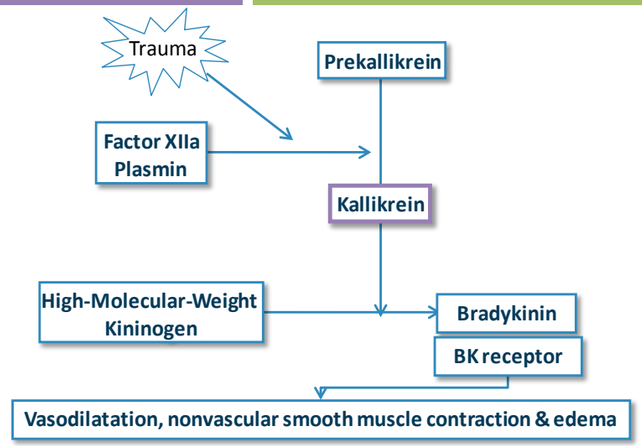
HAE first target in strategy: proven MOA and significant desire for oral therapy

Unpredictable, debilitating, potentially life-threatening swelling attacks



Contact activation pathway is unbalanced based on genetic deficiency of C1 inhibitor (C1-INH)

- Plasma-derived C1-INH (chronic and acute, infusion and injection)
- Androgen steroids (chronic, oral)
- Bradykinin inhibitor (acute, injection)
- Kallikrein inhibitor (acute, injection)

Current standard of care therapies are injected/infused

Images obtained from www.haeimages.com

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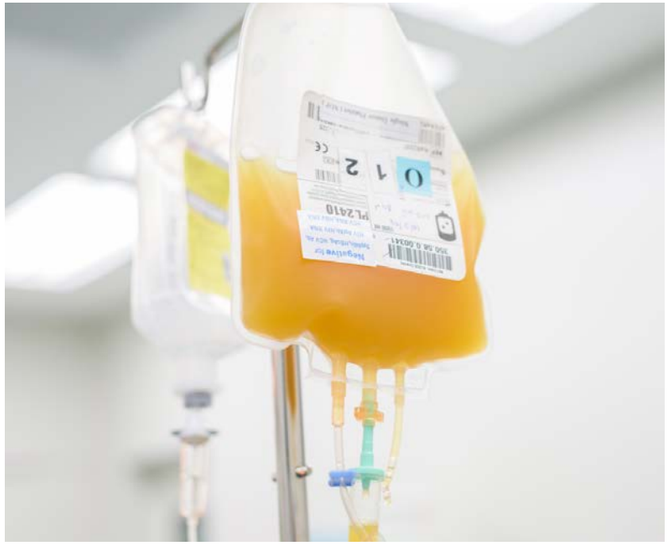
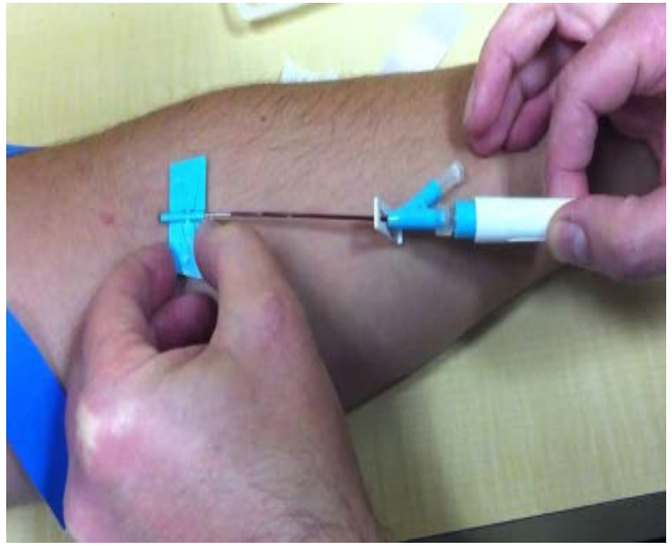
Completing the revolution in care for HAE patients





Pre-2008

"The Dark Years"

*30% mortality **

2008-2016

"The IV Era"

Improved outcomes

2017-2020

"Completing the Revolution"

High convenience/efficacy

* Source: Frank MM, Gelfand JA, Atkinson JP, Ann Intern Med. 1976;84(5):580.

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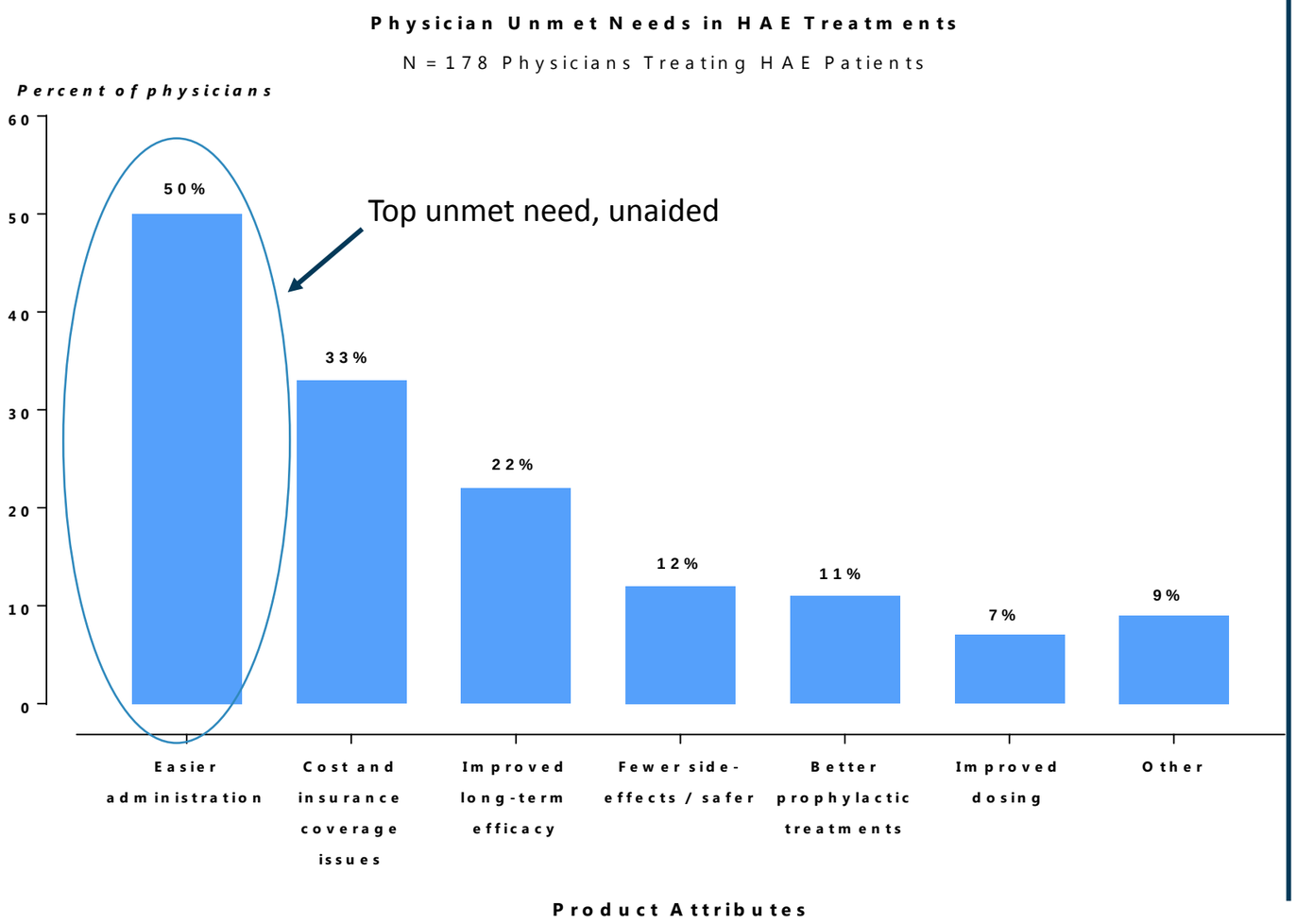
Physicians and patients agree ease of administration is a high unmet need that will drive treatment choice



FDA U.S. FOOD & DRUG ADMINISTRATION

Public Meeting on Patient-Focused Drug Development for Hereditary Angioedema

September 25, 2017

HAE Patients note '**<u>method of administration</u>**' as most important factor driving treatment choice; over access/cost, dose, and side effect profile[1]

Source: BCRX proprietary market research study, 3Q17; 1) RBC Capital Markets 'FDA Patient Event Highlights HAE Unmet Need, Challenges, Opportunities, 9/25/17

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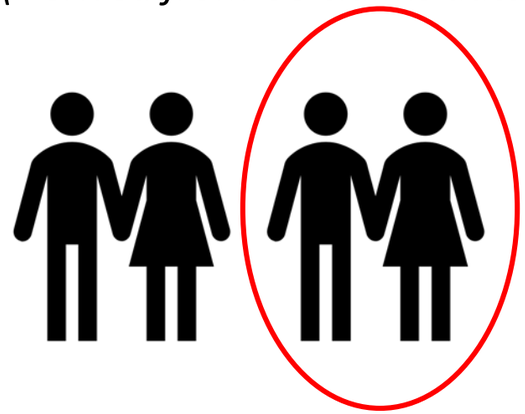
U.S. market is large with significant growth potential

~6,500 US patients
(Derived from US claims data)



50%
C1-Inhibitor
Prophylaxis

Source: BCRX proprietary market research for Prophy vs. Acute market split. Lexis-Nexis Risk Solutions- 'MarketView' Data (formerly HMS) claims data for ICD-9 & ICD-10 codes for HAE (August 2017, 12-month history). ICD10 code: D84.1 & ICD9: 277.6. Sales Data: BioCryst estimates based on Shire, CSL, Pharming public reports.

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Clinical evidence: APeX-1 final analysis

- Attractive and competitive product profile for the prophylaxis of HAE attacks at the 125 mg dose
  - Once-daily oral dosing
  - Competitive attack rate reductions of 73% ($p<0.001$)
  - Safety and tolerability profile similar to placebo
  - Quality of Life scores that are multiples better than the minimum clinically important difference ($p<0.001$)
- Phase 3 dose selection supported by consistent and predictable results
  - 125 mg dose is attractive based on efficacy, safety and tolerability
  - 250 mg and 350 mg doses showed dose-related AEs and drug levels far exceeded the target threshold for efficacy
  - 62.5 mg dose showed no benefit and drug levels were below the target threshold for efficacy
  - High predictability of PK and PD provides confidence in choosing 175 mg as the second dose to study in Phase 3 clinical trials

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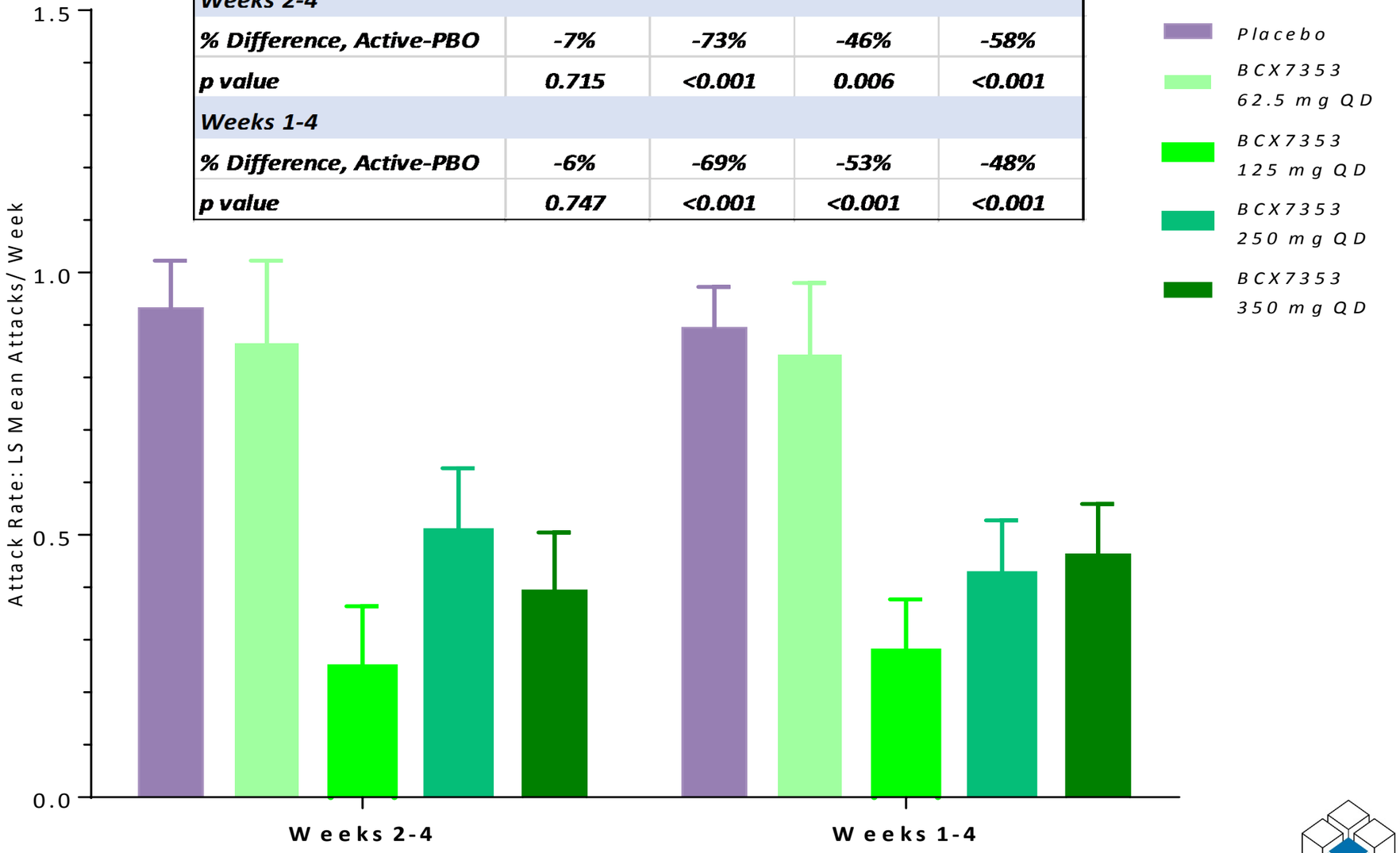
APeX-1 - Overall angioedema attack rate per week, PP population, weeks 2-4 and 1-4

	62.5 mg	125 mg	250 mg	350 mg
Weeks 2-4				
% Difference, Active-PBO	-7%	-73%	-46%	-58%
p value	0.715	<0.001	0.006	<0.001
Weeks 1-4				
% Difference, Active-PBO	-6%	-69%	-53%	-48%
p value	0.747	<0.001	<0.001	<0.001



APeX-1 - 125 mg dose provided consistent reductions in attack rate

Analysis	n	LS mean[1] Attacks per Week		Difference vs Placebo	Percentage Reduction vs Placebo	p-Value vs Placebo
		BCX7353 125 mg	Placebo			
Confirmed attacks, Weeks 2-4 PP population	13	0.248	0.932	-0.684	73%	<0.001
Confirmed attacks, Weeks 2-4 ITT population	14	0.249	0.937	-0.688	73%	<0.001
Confirmed attacks, Weeks 1-4 PP population	13	0.278	0.895	-0.617	69%	<0.001
Confirmed attacks, Weeks 1-4 ITT population	14	0.270	0.890	-0.619	70%	<0.001
Confirmed attacks requiring treatment, Weeks 2-4 PP population	13	0.221	0.807	-0.585	73%	<0.001
Confirmed attacks requiring treatment, Weeks 2-4 ITT population	14	0.224	0.771	-0.546	71%	0.002
Confirmed attacks requiring treatment, Weeks 1-4 PP population	13	0.221	0.788	-0.567	72%	<0.001
Confirmed attacks requiring treatment, Weeks 1-4 ITT population	14	0.217	0.753	-0.536	71%	<0.001

[1] Least squares mean calculated using an ANCOVA model with qualifying attack rate as covariate

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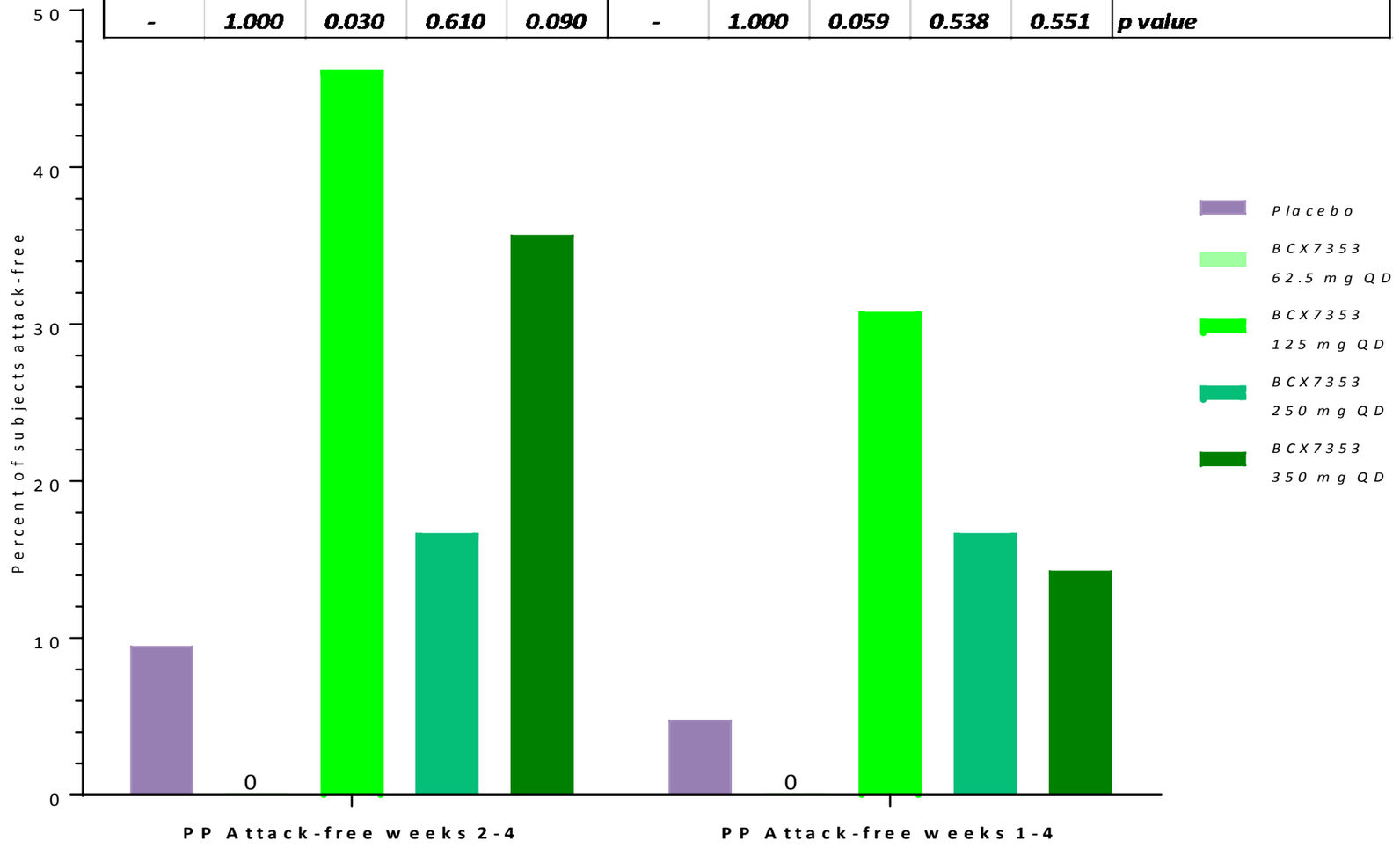
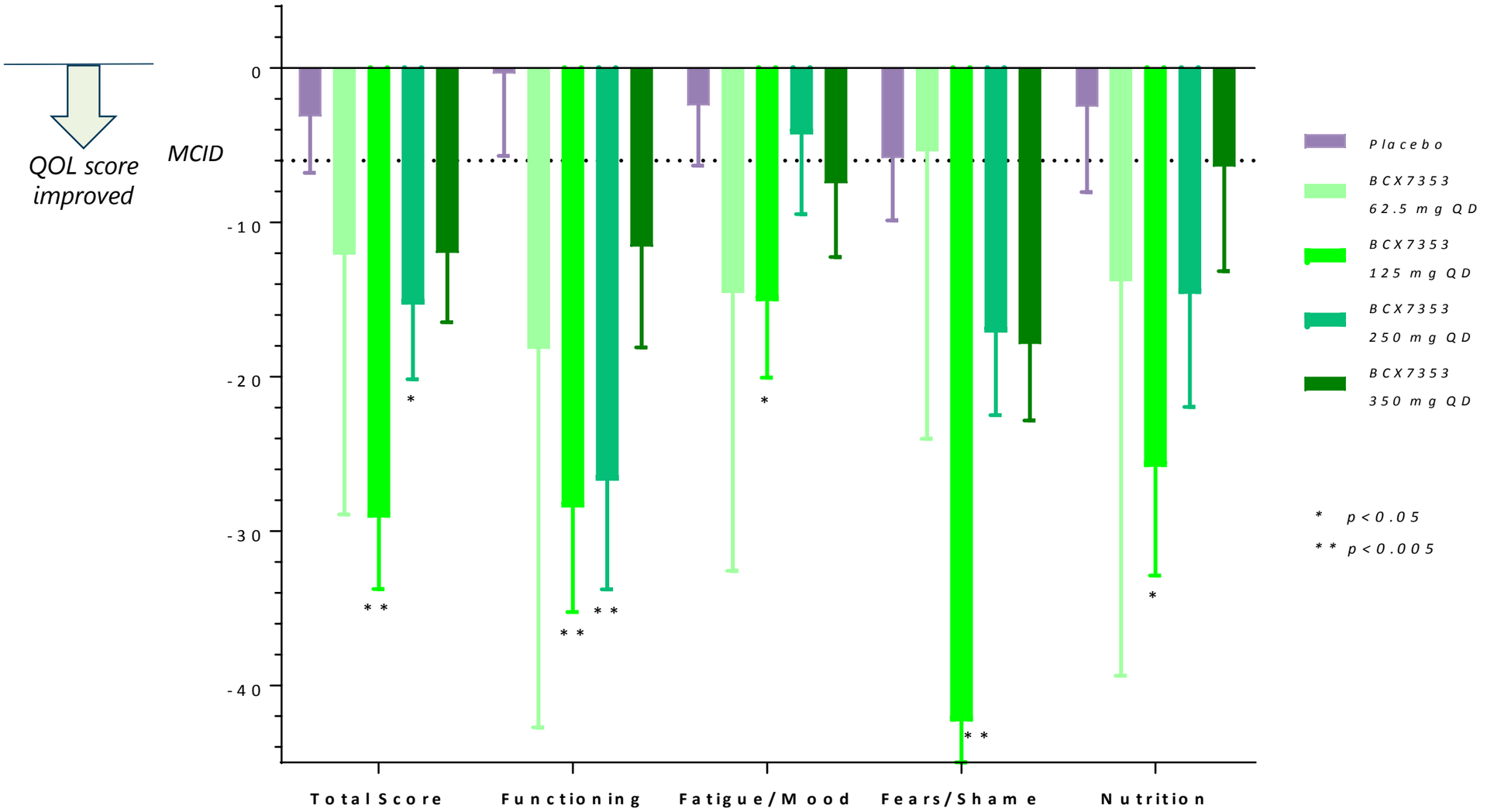
APeX-1 - Percent of subjects attack-free, PP

PBO	62.5 mg	125 mg	250 mg	350 mg	PBO	62.5 mg	125 mg	250 mg	350 mg	
Weeks 2-4					Weeks 1-4					
9.5%	0.0%	46.2%	16.7%	35.7%	4.8%	0.0%	30.8%	16.7%	14.3%	% Attack free
-	-9.5%	36.7%	7.2%	26.2%	-	-4.8%	26.0%	11.9%	9.5%	Difference, Active-PBO
-	1.000	0.030	0.610	0.090	-	1.000	0.059	0.538	0.551	p value



APeX-1 - Angioedema quality of life (AE-QoL): LS mean change from BL at day 29, PP



Difference in adjusted least square means are shown (Active treatment minus Placebo).
ANCOVA Model includes terms of treatment and adjusted qualifying attack rate.
Reductions (negative changes from BL) represent improvement in quality of life scores.
MCID, minimum clinically important difference, -6 points (*Weller, K. 2016. Allergy 71(8): 1203-1209.)*
BCX7353 dose level compared with placebo

APeX-1 - Treatment-emergent adverse event summary

Category	BCX7353 62.5 mg N = 7	BCX7353 125 mg N = 14	BCX7353 250 mg N = 14	BCX7353 350 mg N = 18	Placebo N = 22
Subjects with any TEAE[1], n (%)	4 (57)	7 (50)	11 (79)	14 (78)	15 (68.2)
Subjects with any Serious AE, n (%)	0	0	1 (7)[2]	0	0
Subjects with Drug-Related Grade 3/4 AE, n (%)	0	0	0	1 (6)	0
Subjects with AE Leading to D/C from Study Drug, n (%)	0	0	0	3 (17)	0
Non-drug-related, n (%)	0	0	0	1 (6)[3]	0
Drug-related, n (%)	0	0	0	2 (11)[4,5]	0

[1] TEAE- treatment-emergent adverse event.

[2] GI infection- investigator assessed as unrelated to study drug. Abdominal symptoms similar to several previous non-HAE-attack episodes occurring over past 3 years resulting in severe vomiting and diarrhea. Pt presented to ER and hospitalized for IV fluids and antiemetics as a precaution. No HAE acute attack meds given. Recovered and discharged the following day. Missed 1 day of dosing due to event.

[3] Pre-existing liver disorder (improved from baseline, but persisting). Previously reported in 1st interim analysis.

[4] n=1 Gastroenteritis with liver disorder (both assessed as related) (elevated ALT 1.9x ULN, GGT 5.4x ULN and ALP 1.6x ULN, with normal AST and bilirubin). Previously reported in 1st interim analysis.

[5] n=1 Vomiting/abdominal cramps. Previously reported in 2nd interim analysis.

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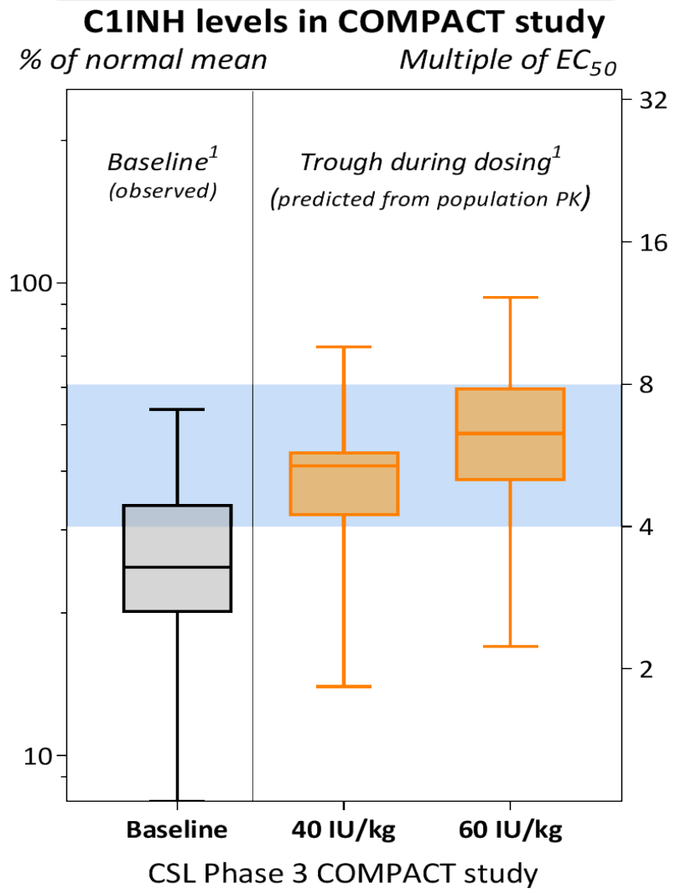
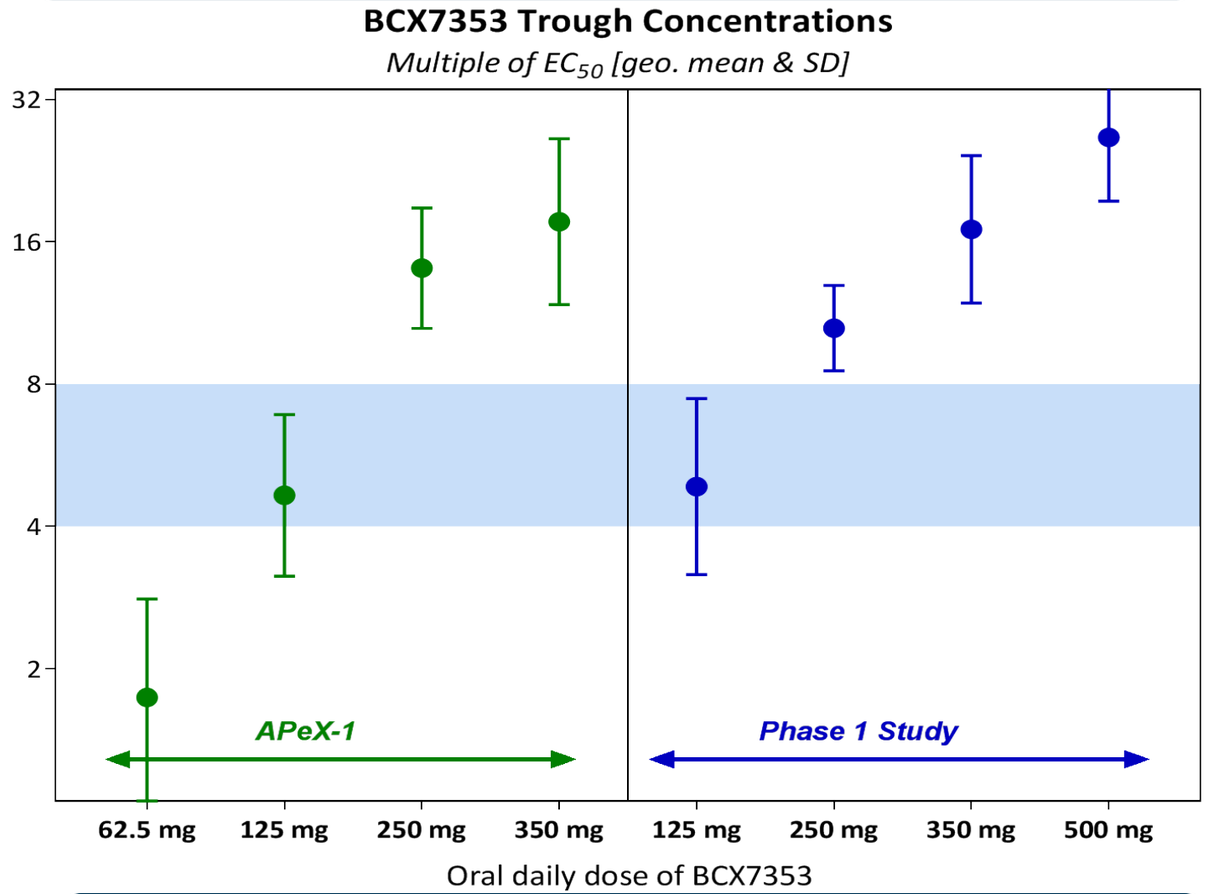
APeX-1 - Exposure comparisons of BCX7353 and SC C1INH

CSL-830 Phase 3 study



C1INH levels at baseline and after SC dosing with CSL-830[1]

BCX7353 APeX-1 & Phase 1



BCX7353 plasma concentrations at 24 hours post-dose

[1] Longhurst, H. et al. N Engl J Med 376, 1131-1140 (2017). Box plots represent median, 25th and 75th percentiles, minimum and maximum values. CSL-830 and BCX7353 data are from distinct clinical trials and no head to head study has been conducted.

Predictable PK supports 175 mg as second dose in Phase 3

Dose, mg QD	% >4 x EC_{50}		% > 6 x EC_{50}		% > 8 x EC_{50}	
	Predicted	**Actual**	Predicted	Actual	Predicted	Actual
62.5	**--**	***0***	--	*0*	--	*0*
125	**70**	***64***	38	*43*	17	*0*
175	**93**		80		58	
200	97		88		73	
225	98		93		83	
250	**100**	***100***	97	*100*	93	*100*

- Given the predictable PK of BCX7353, simulations are helpful in selecting an intermediate dose of BCX7353 to study that is between 125 mg and 250 mg.
- These simulations suggest a relatively small increase in dose above 125 mg should achieve a significant increase in the proportion of subjects achieving trough levels above the therapeutic target.
- These simulations suggest 175 mg dose should maintain trough drug levels > 4 x EC_{50} in > 90% of patients.
- Doses ≥ 200 mg offer little additional increment in proportions achieving target level.

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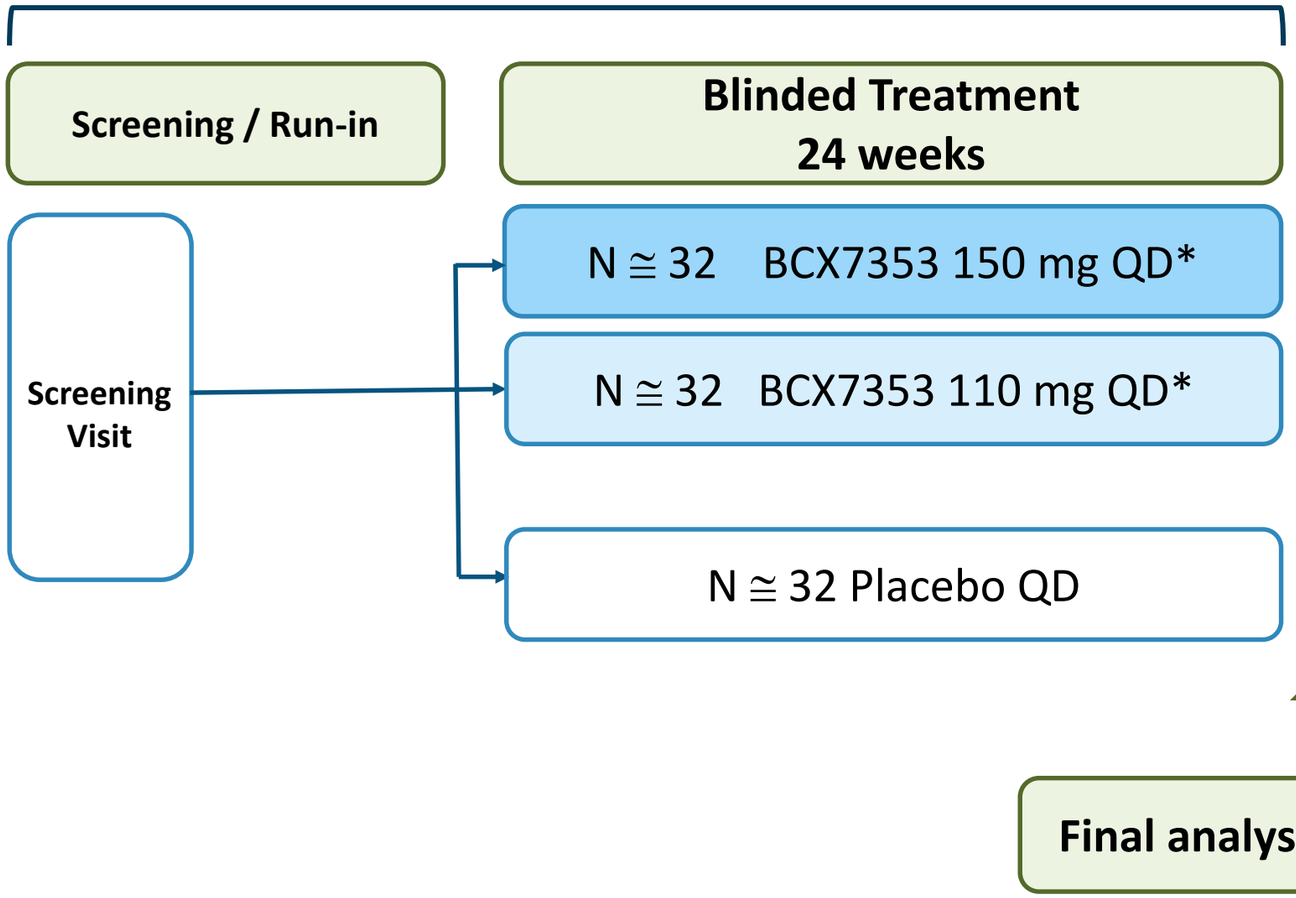
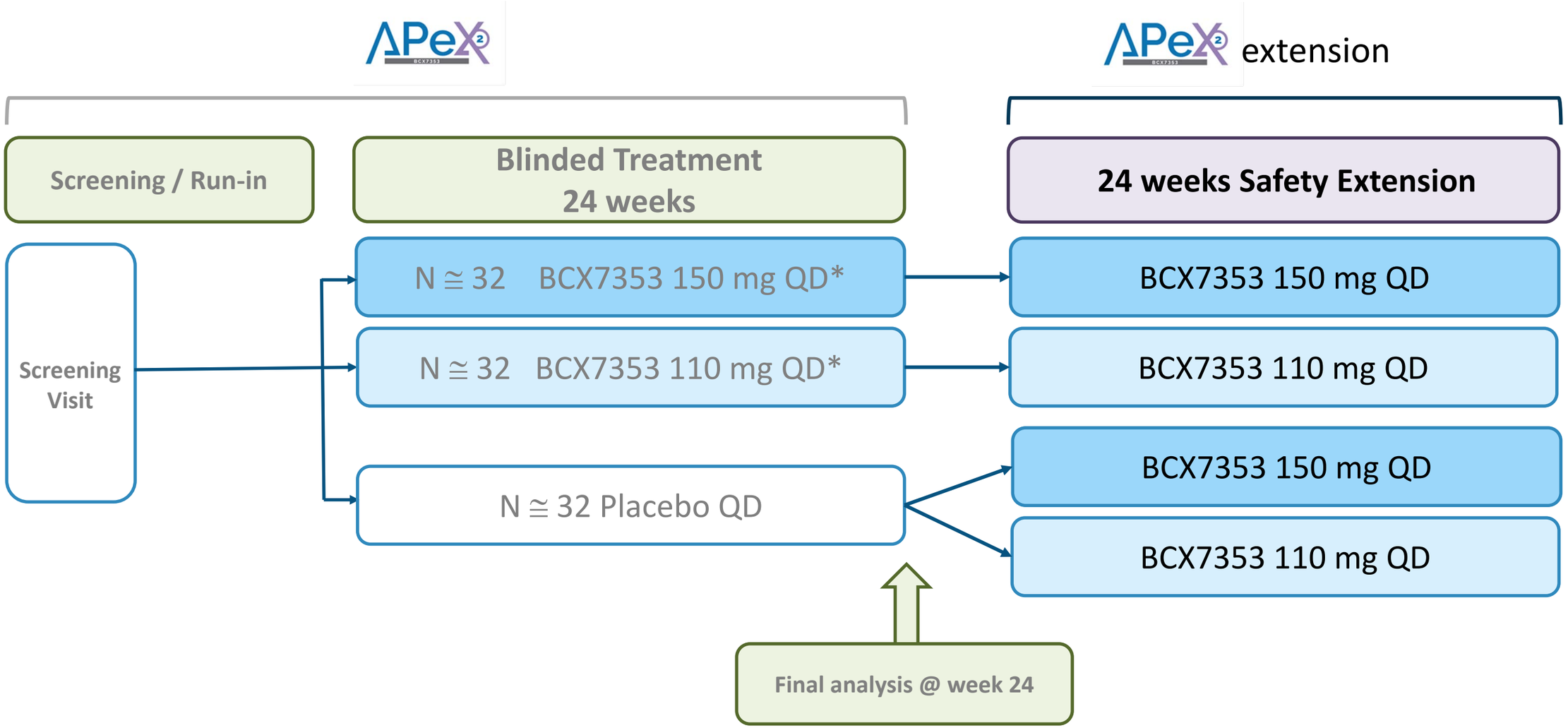
APeX-2 phase 3 trial design



- *Primary endpoint at Week 24:*
  - *Rate of Investigator-confirmed HAE attacks through entire treatment period*
- *Study powered at >90% to detect a ≥50% reduction in attack rate over placebo*

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

APeX-2 phase 3 trial design – safety extension



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

APeX-S long-term safety study design

48 weeks treatment

N ≅ 80 BCX7353 150 mg QD

N ≅ 80 BCX7353 110 mg QD

Analyses as needed for regulatory submissions

- *Endpoints:*
  - *Long term safety of BCX7353*
  - *Durability of response*
  - *Quality of Life*
- *N = approximately 200 subjects through 12 months in total from*
  - *APeX-2 safety extension*
  - *APeX-S*
- *APeX-1 subjects eligible*

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

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Cash position and 2017 guidance (in millions)

Cash & investments at December 31, 2016	$65
Cash & investments at September 30, 2017	**$169**
Senior Credit Facility	$23

Guidance for 2017:

Operating cash utilization	$30 – 50@
Operating expenses#	$53 – 73@

Excludes equity-based compensation.
@ We currently forecast our actual results to be in the upper-half of our 2017 Guidance.

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Building a company to generate expanding and sustainable value