JMP Securities 2017 Life Sciences Conference

June 21st , 2017

Jon Stonehouse, *President & Chief Executive Officer*
Bill Sheridan, *Chief Medical Officer*

BioCryst Pharmaceuticals, Inc.

Forward-looking statement

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BioCryst PHARMACEUTICALS, INC.

BioCryst's strategy is to develop oral drugs for rare diseases

Drug discovery through structure-based design

- BCX7353 and 2nd Gens
- Lead optimization underway for two additional rare disease targets

Significant supporting capital from antiviral programs

- RAPIVAB® (peramivir injection) and Galidesivir externally funded
- Stockpiling and voucher potential

Help patients lead normal lives

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BioCryst's 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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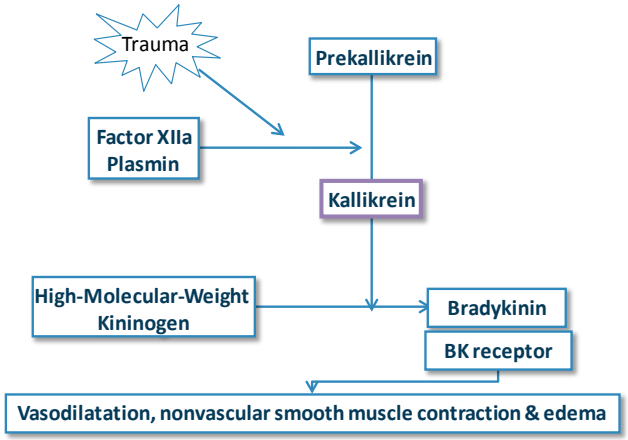
First target in strategy: Hereditary angioedema (HAE) is a high-need, high-value disease

Unpredictable, debilitating, potentially life-threatening swelling attacks



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

- Rare (estimated global prevalence of 1:50K)
- Growing US market ($1.2B, 30% growth in 2015)
- Significant global upside as paradigm shifts to attack prevention

High-value, growing market on track to exceed $2.0B globally

- Plasma-derived C1-INH (chronic and acute, infusion)
- 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
Market estimates based on analyst reports, earnings reports, and market data

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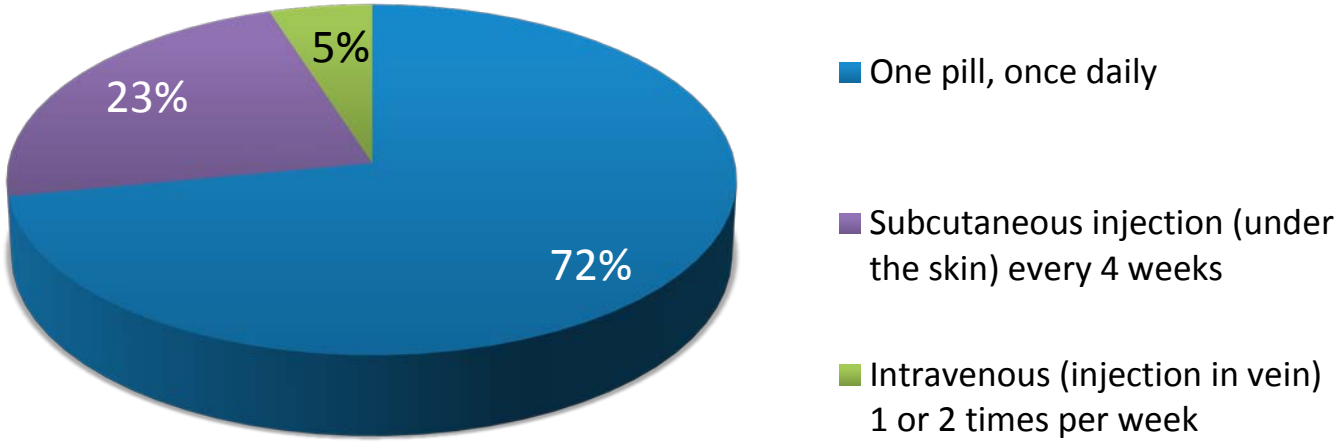
Patients with HAE overwhelmingly prefer convenient oral therapy

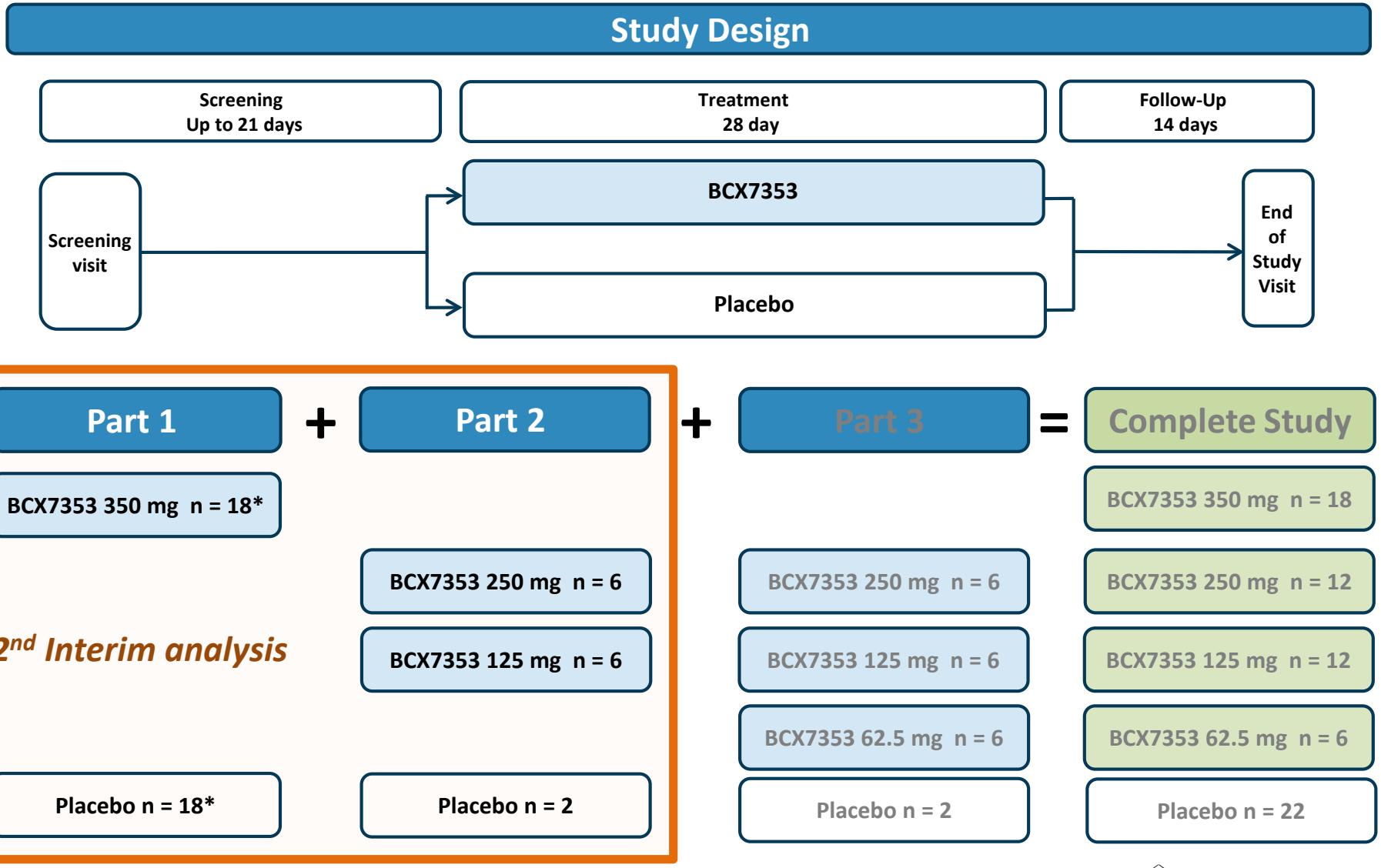
Preferred route of administration among US HAE patients currently taking prophylactic therapy (N=83)



Question: Which medication would you prefer assuming each is equally safe and effective and costs the same amount per year?

Source: BioCryst Proprietary Quantitative Market Research Study with Patients, 2016.

APeX-1: Trial design



*1st Interim analysis evaluated 14 subjects in each arm in Part 1

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APeX-1 second interim analysis population

	BCX7353			
	125 mg	250 mg	350 mg	Placebo
Randomized and treated	7	6	18	20
Intent to Treat (ITT) population	7	6	18	20
Per Protocol (PP) population	6	5	14	19
Excluded from PP population				
HAE Type 1 or 2 not confirmed			1	1
<28 days of dosing with study drug	1		3	
Non compliance with diary completion		1		
Study drug compliance, mean % (SD)	98% (5)	99% (1)	98% (8)	98% (6)

Age – years, mean (SD)	48.4 (14.0)	50.7 (12.1)	43.8 (11.6)	46.5 (11.7)
Sex – female, n (%)	5 (71%)	2 (33%)	11 (61%)	12 (60%)
Prior androgen use, n (%)	2 (29%)	5 (83%)	15 (83%)	11 (55%)
Qualifying attack rate, attacks/wk mean (SD)	0.95 (0.26)	0.83 (0.52)	0.84 (0.35)	0.90 (0.46)

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Rate of overall confirmed attacks: PP population

Treatment	n	LS mean[1] Attacks per Week	Difference vs Placebo	Percentage Reduction vs Placebo	p-Value vs Placebo
Effective dosing period (Week 2-4) – PP Population					
BCX7353 combined	25	0.402	-0.543	57%	<0.001
BCX7353 125 mg	6	0.253	-0.691	73%	0.002
BCX7353 250 mg	5	0.595	-0.349	37%	0.128
BCX7353 350 mg	14	0.397	-0.548	58%	0.001
Placebo	19	0.945	-	-	-
Part 1 Interim analysis:					
BCX7353 350 mg	*11*	*0.343*	*-0.572*	*63%*	*0.006*
Placebo	*13*	*0.915*			

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

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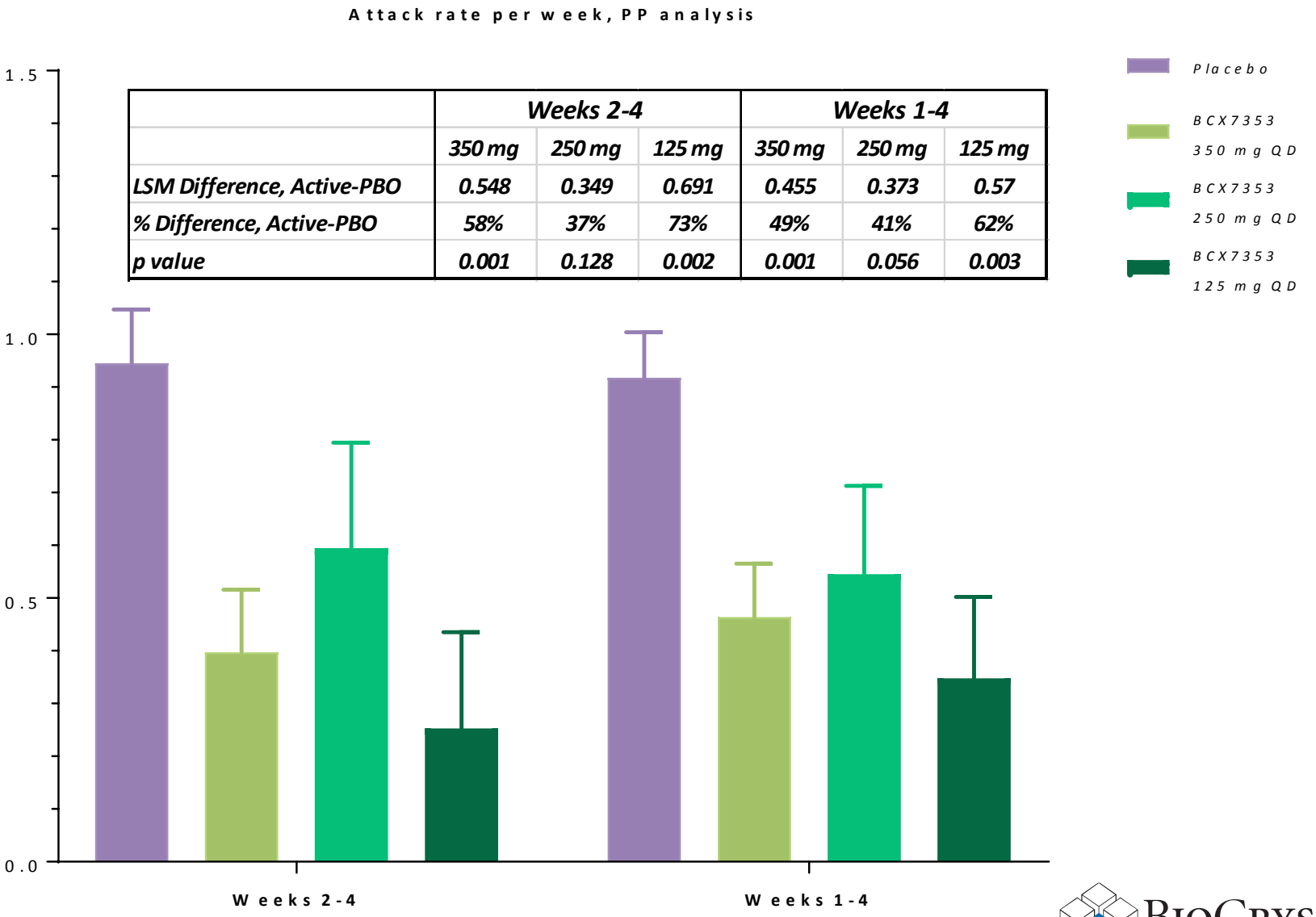
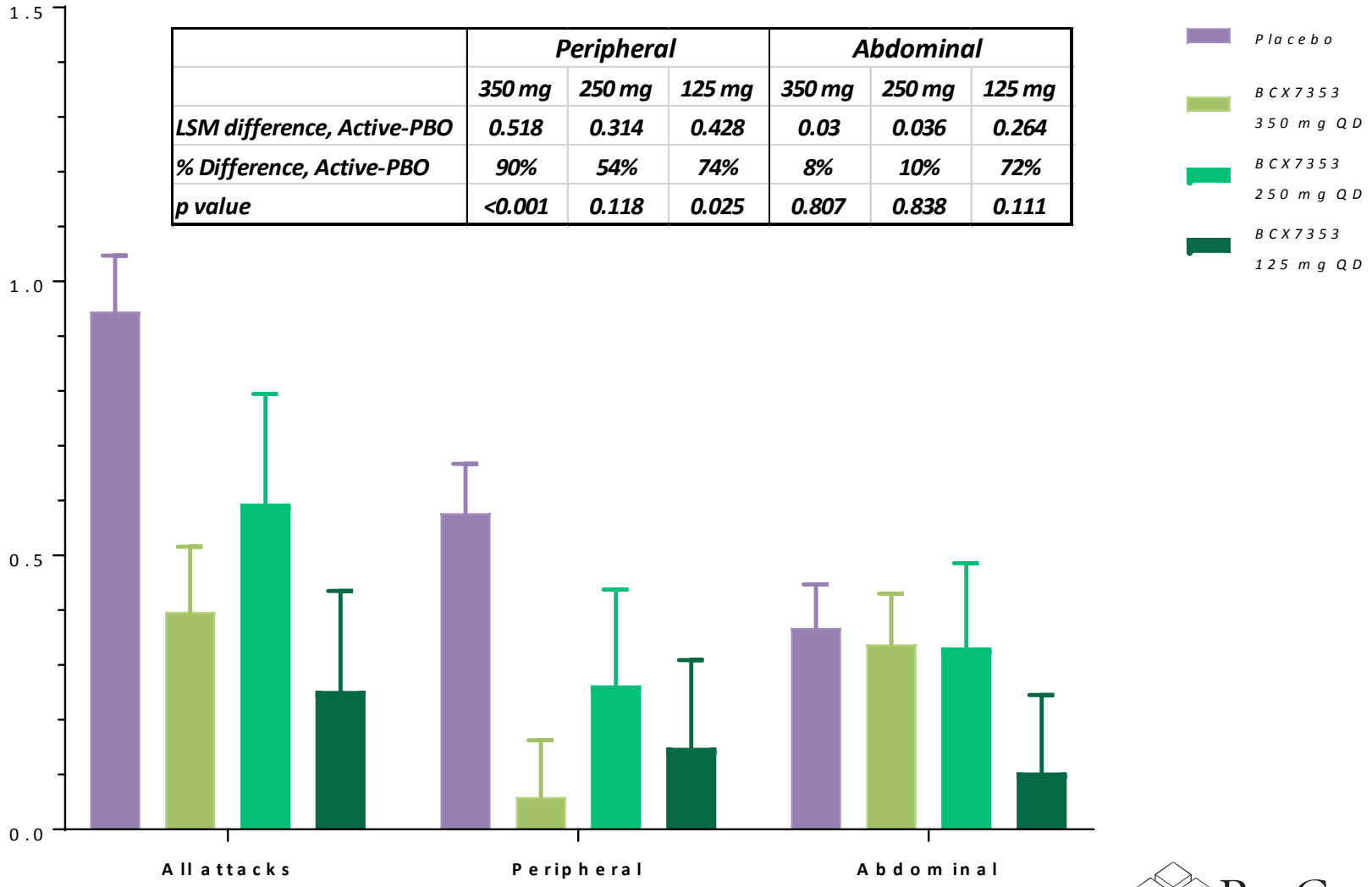
Overall attack rate, PP analysis, weeks 2-4 and 1-4

	Weeks 2-4			Weeks 1-4		
	350 mg	250 mg	125 mg	350 mg	250 mg	125 mg
LSM Difference, Active-PBO	0.548	0.349	0.691	0.455	0.373	0.57
% Difference, Active-PBO	58%	37%	73%	49%	41%	62%
p value	0.001	0.128	0.002	0.001	0.056	0.003

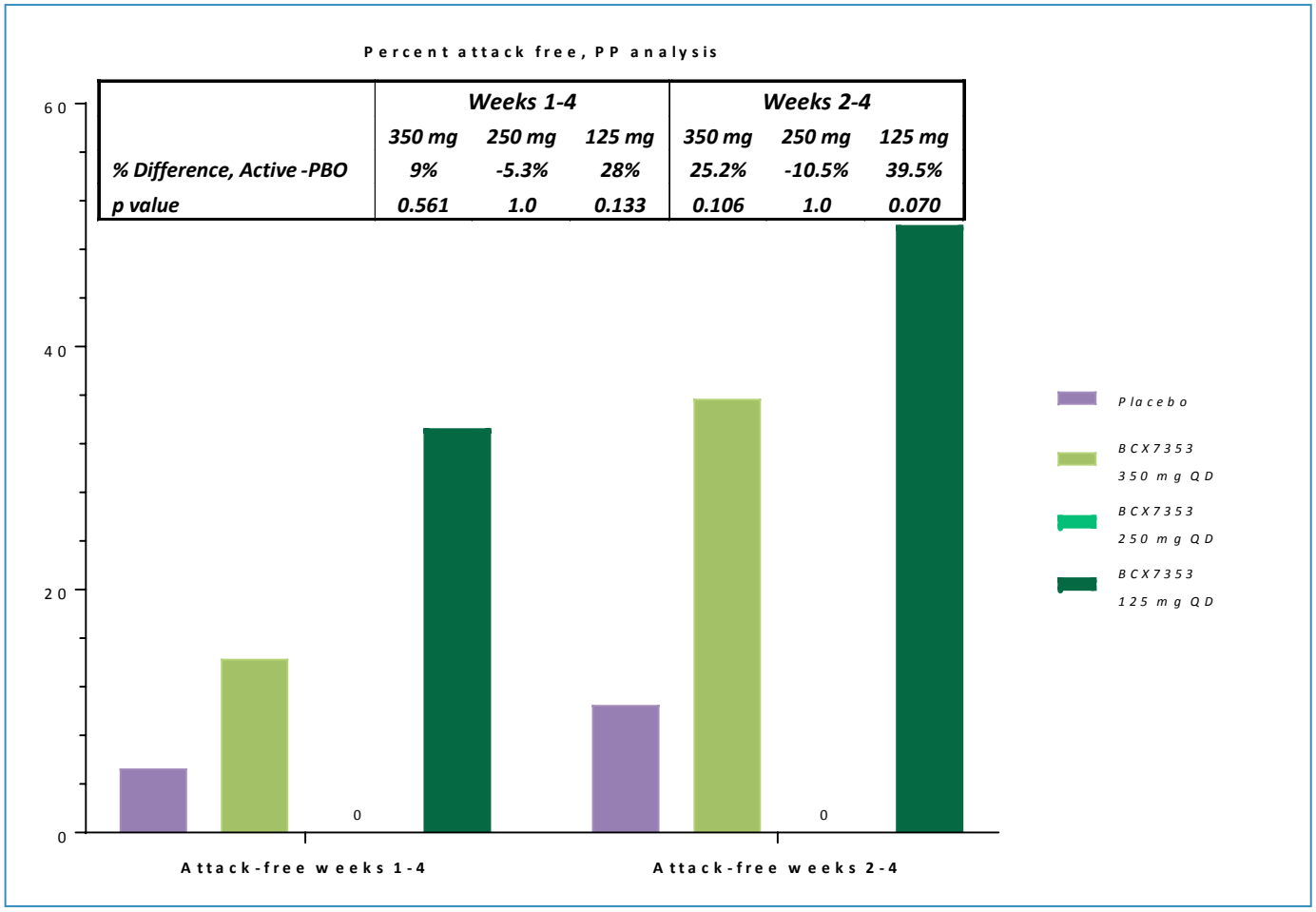


Attack rates by prespecified anatomical location, PP

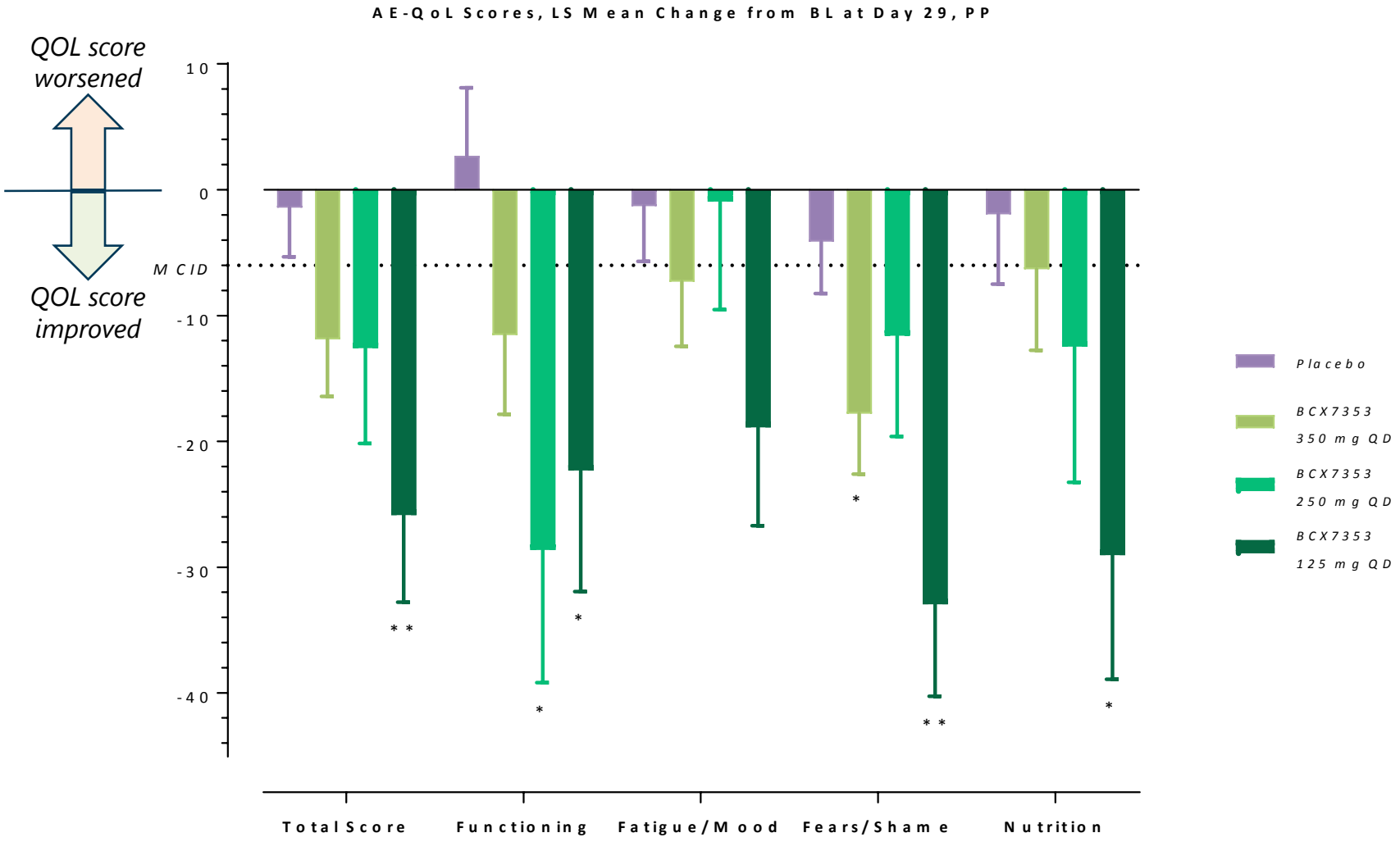
	Peripheral			Abdominal		
	350 mg	250 mg	125 mg	350 mg	250 mg	125 mg
LSM difference, Active-PBO	0.518	0.314	0.428	0.03	0.036	0.264
% Difference, Active-PBO	90%	54%	74%	8%	10%	72%
p value	<0.001	0.118	0.025	0.807	0.838	0.111



Percent of subjects attack-free, PP analysis

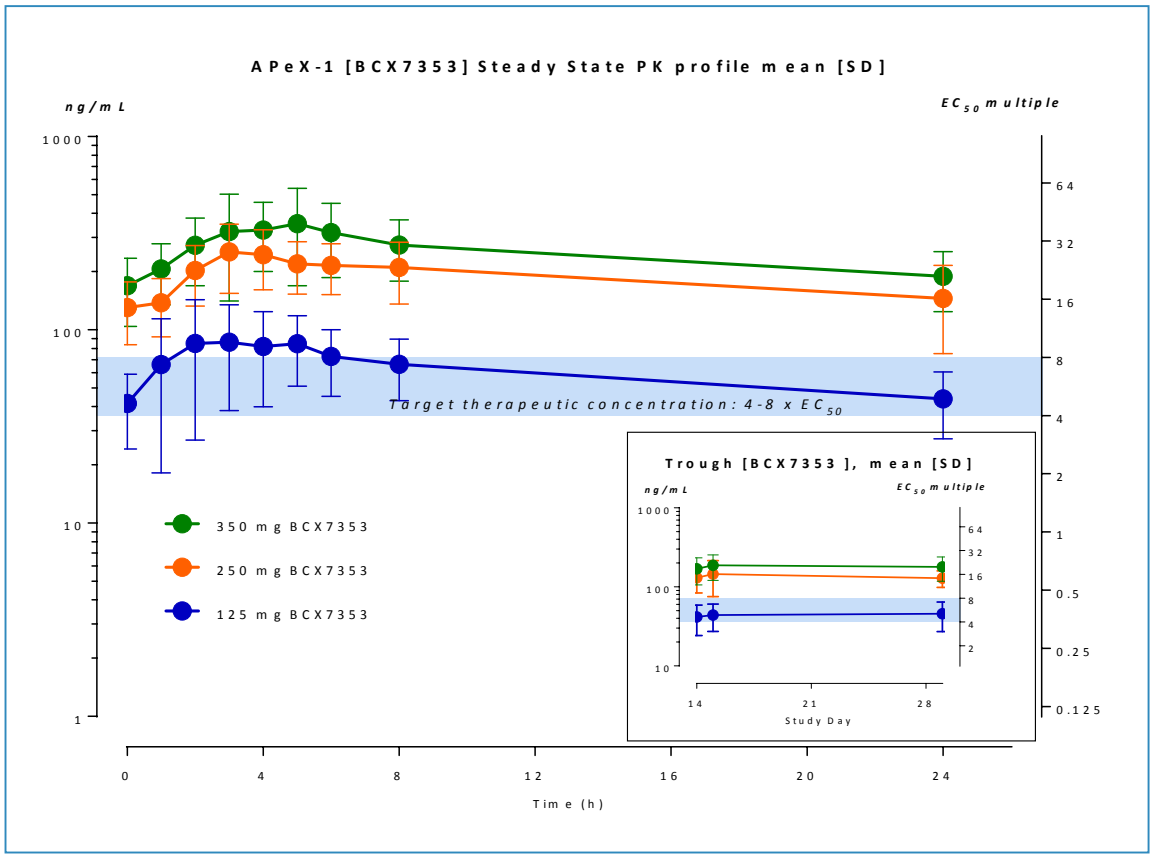


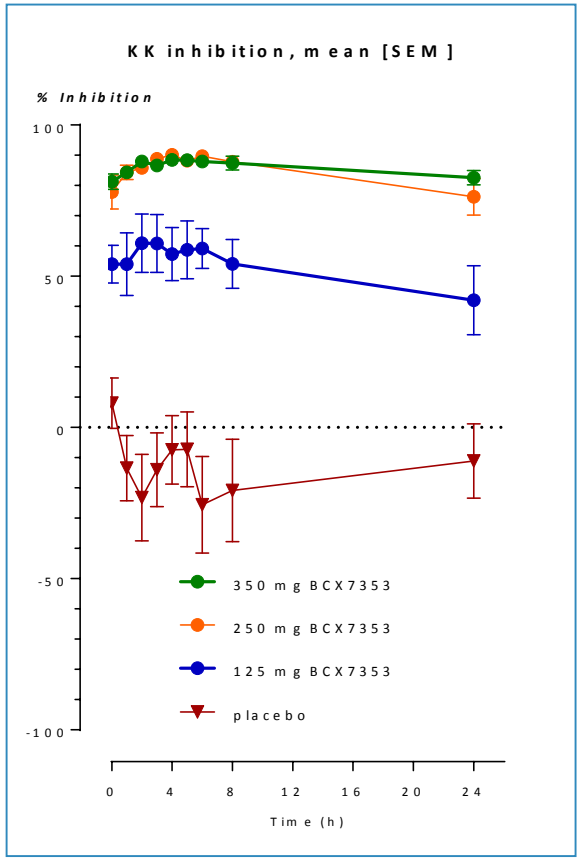
Quality of life scores, PP analysis of change from baseline



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.)*
* $p < 0.05$, ** $p < 0.01$, BCX7353 dose level compared with placebo

PK and KK inhibition profiles at steady state for BCX7353 dose levels 125 mg, 250 mg and 350 mg QD in HAE subjects in APeX-1





Steady state drug levels greatly exceeded the target therapeutic range for both 250 mg and 350 mg QD and trough levels for the 125 mg dose were generally within the target range

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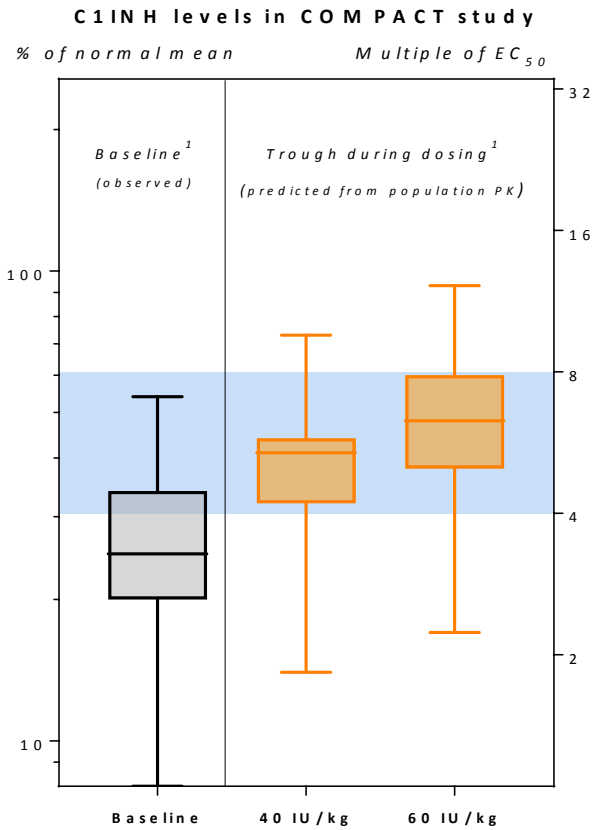
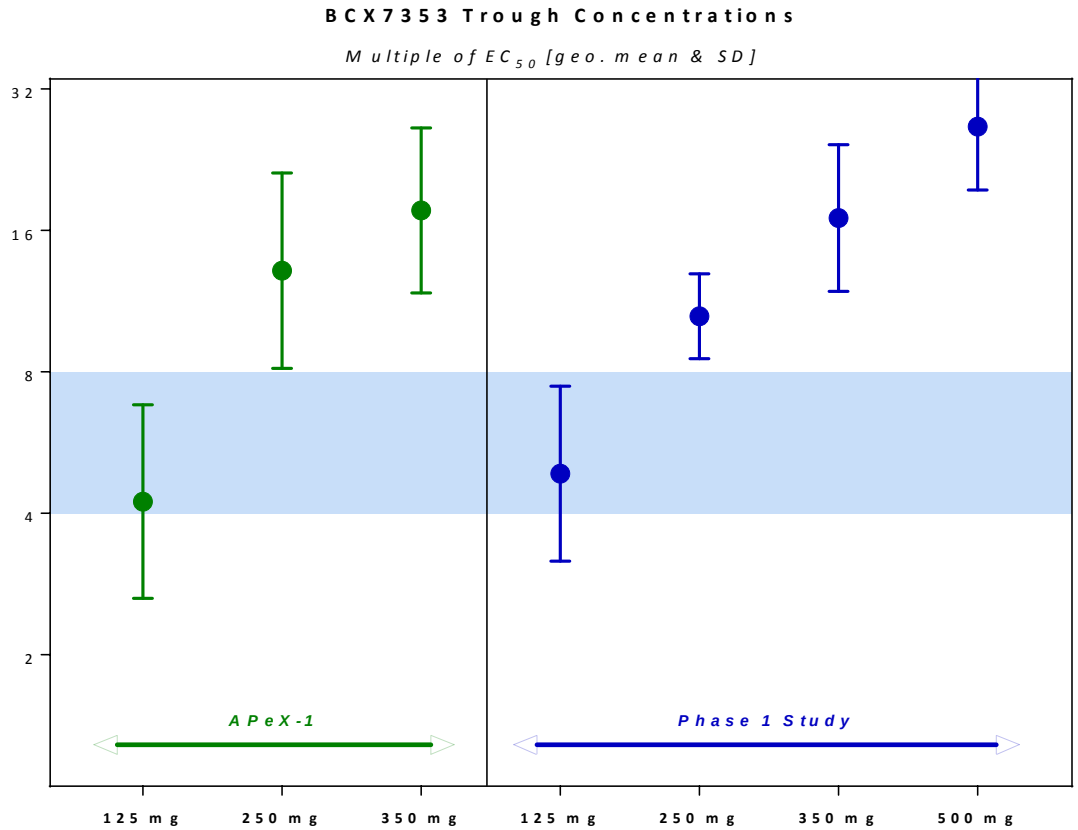
Exposure in APeX-1 and Phase 1 BCX7353 trials, and SC C1INH

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.

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APeX-1 Second interim analysis safety summary

Category	BCX7353 125 mg n=7	BCX7353 250 mg n=6	BCX7353 350 mg n=18	Placebo n=20
Subjects with any Serious AE, n (%)	0	0	0	0
Subjects with Drug-Related Grade 3/4 AE, n (%)	0	0	1	0
Subjects with AE Leading to D/C from Study Drug, n (%)	0	0	3	0
Non- drug-related, n (%)	0	0	1[1]	0
Drug-related, n (%)	0	0	2[2]	0

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

[2] -Gastroenteritis (unrelated) with liver disorder (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
- Vomiting/ abdominal cramps concurrent with menses

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APeX-1 Second interim analysis safety summary

Category	BCX7353 125 mg n=7	BCX7353 250 mg n=6	BCX7353 350 mg n=18	Placebo n=20
Treatment-Emergent Adverse Events occurring in ≥2 subjects overall, n (%)				
Nasopharyngitis	0	0	5 (28%)	6 (30%)
Diarrhoea	0	1 (17%)	4 (22%)	2 (10%)
Abdominal pain	0	0	3 (17%)	0
Nausea	0	2 (33%)	3 (17%)	0
Fatigue	0	0	2 (11%)	1 (5%)
Flatulence	0	0	2 (11%)	0
Vomiting	0	0	2 (11%)	0
Constipation	0	0	1 (6%)	1 (5%)
Headache	2 (29%)	0	1 (6%)	3 (15%)
Constipation	0	0	1 (6%)	1 (5%)
Migraine	1 (14%)	0	1 (6%)	0
Pharyngitis	0	1 (17%)	1 (6%)	0
Clin. significant changes in clin. chem., hematology or urinalysis, n (%)	0	1[1]	1[2]	0

[1] Treatment-emergent ALT elevation to >3X ULN (ALT 4.1 x ULN, AST 1.9 x ULN). *20 years androgen use, Baseline increase in liver enzymes*

[2] 1 event previously reported: ALT elevation > 3X ULN (ALT 3.9 x ULN, AST 1.8 xULN, GGT10.7 X ULN) *Pre-existing colitis, hepatic steatosis (fatty liver), > 20 years androgen use, Baseline increase in liver enzymes*

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Conclusions

- BCX7353 once daily for 4 weeks showed clinically meaningful and statistically significant reductions in the rate of angioedema attacks
  - 125 mg dose showed a reduction of 73% in overall attack rate (p=0.002)
- Oral BCX7353 once daily over 4 weeks was generally safe and well tolerated
- A dose related improvement in GI tolerability was observed in the 250 mg and 125 mg dose groups
- No significant treatment emergent abnormalities in laboratory safety parameters were observed in the 250 mg and 125 mg dose groups
- Steady state trough drug levels (24 hours after dosing) greatly exceeded the target therapeutic range at the 250 mg and 350 mg dose levels. Trough levels for the 125 mg dose were generally within the target range
- PK profile and kallikrein inhibition levels were similar to those seen at the same dose levels in Phase 1 healthy subjects
- Completion of Part 3 will help to round out the dose response data necessary to select doses for Phase 3

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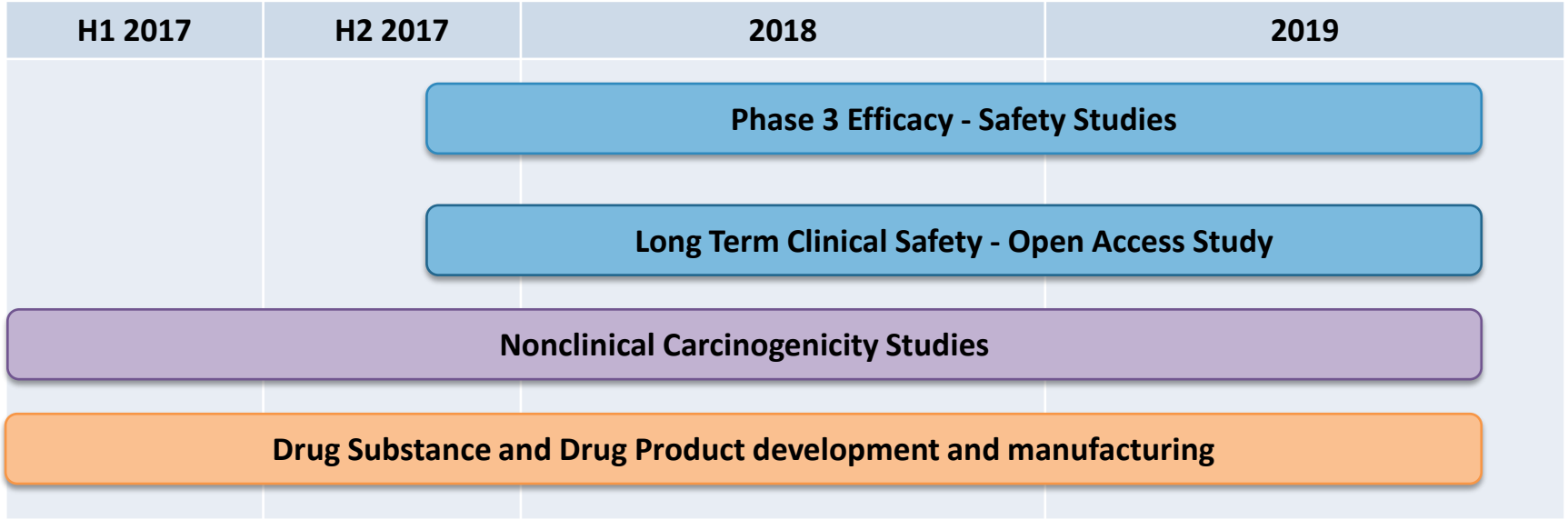
BCX7353 Remaining activities after APeX-1

Estimated timing of key activities to support NDA/MAA filing



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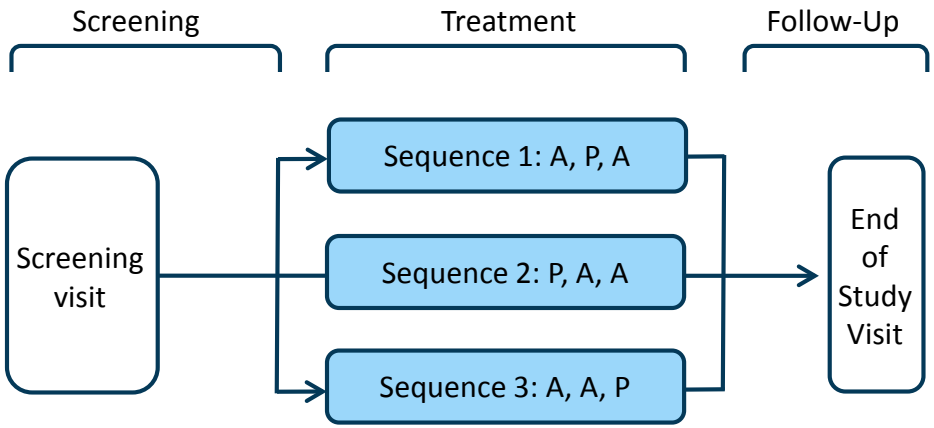
ZENITH-1 trial design



Part 1: BCX7353 Dose Level 1 n = 12 - 36 + Part 2: BCX7353 Dose Level 2 n = 12 + Part 3: BCX7353 Dose Level 3 n = 12 = Total n = 36 - 60

- The ZENITH-1 trial will evaluate the potential for an oral liquid formulation of BCX7353 to treat acute angioedema attacks
- Each subject is intended to have 3 attacks treated with blinded study drug
  - 2 with BCX7353 (A) and 1 with Placebo (P)
- Primary efficacy endpoint: proportion of subjects with improved or stable composite visual analog scale (VAS) score at 4 hours post-dose

Antiviral programs are externally funded, generating $400M to date with the potential for significant future capital infusions

Antiviral Program	Indication	Development funding	Additional capital infusions
	First and only one-dose IV treatment for influenza	Over $200M US Government funding to support development and approval	• Over $90M in milestones and royalty monetization • Over $25M in Government stockpiling (Japan/US)
Galidesivir (BCX4430)	• Ebola is lead indication • Broad-spectrum activity observed in Zika, Marburg and several other virus families	Approximately $80M US Government contract development funding	• Potential for Government stockpiling prior to FDA approval • Potentially eligible for FDA priority review voucher upon approval

Broad-spectrum activity increases attractiveness of Galidesivir for Government stockpiling

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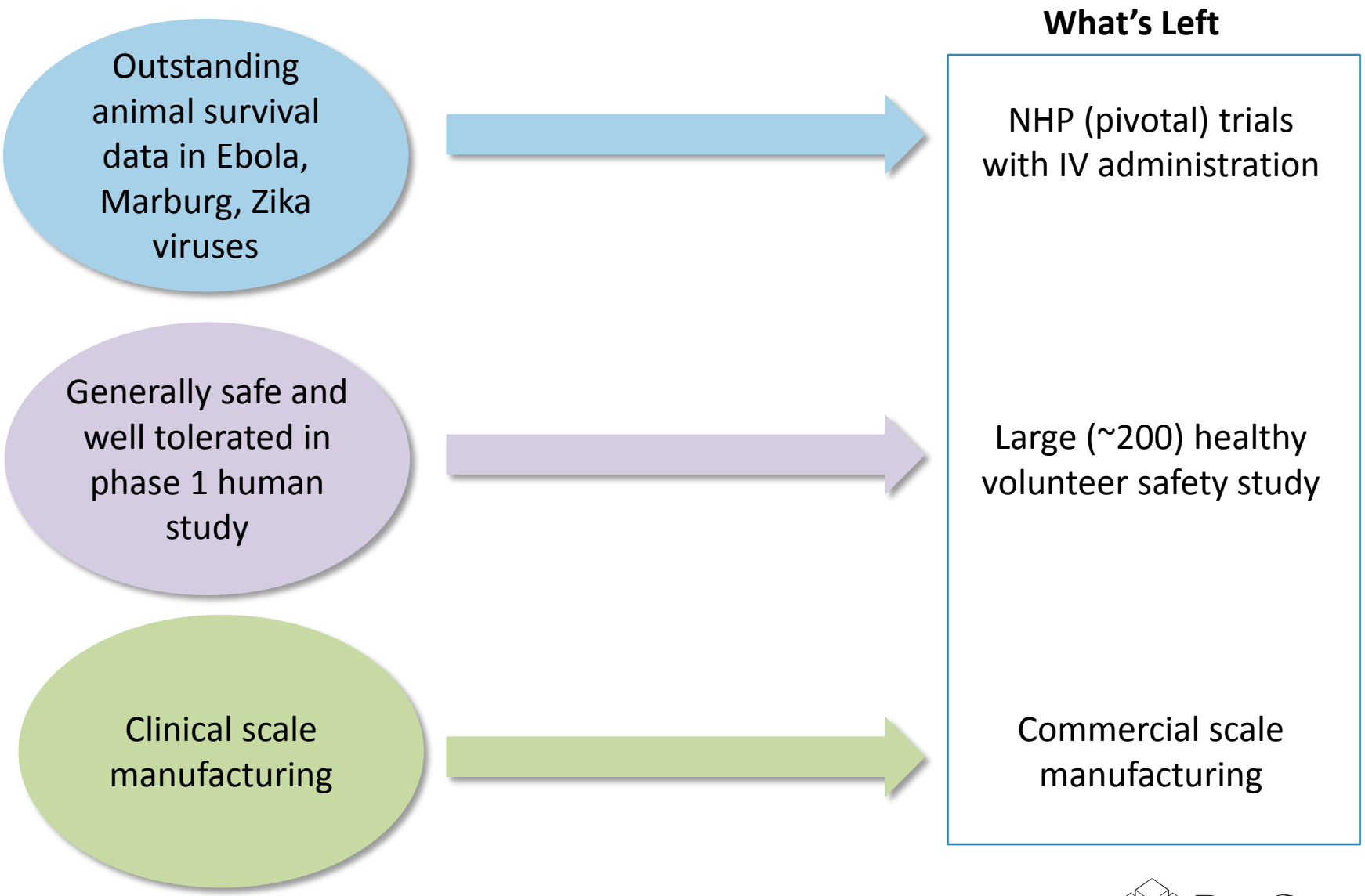
Galidesivir path to stockpiling and NDA



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Stockpiling and Voucher Comparables

Precedent highly pathogenic countermeasures

Product	Pathogen	Company	Doses	Cost
BioThrax vaccine	Anthrax	Emergent BioSolutions	29M	$691M
Raxibacumab antitoxin (CY '13)	Anthrax	GSK	60K	$193M
AbThrax antibody	Anthrax	HGS (now GSK)	65K	$326M
Botulimun antitoxin	Botulism	Cangene	200K	$427M
MVA vaccine	Smallpox	Bavarian Nordic	20M	$505M
ACAM2000 vaccine (CY '08)	Smallpox	Acambis	>72M	$425M-$660M
ST-246 antiviral	Smallpox	Siga	1.7M	$433M

Precedent voucher purchases

Disease	Drug	Seller (Buyer)	Price
Morquio A syndrome	Vimizim (elosulfase alfa)	BioMarin (Sanofi)	$67.5M
Leishmaniasis	Impavido (miltefosine)	Knight (Gilead)	$125M
High-risk neuroblastoma	Unituxin (dinutuximab)	United Therapeutics (Abbvie)	$350M
Rare bile acid synthesis disorders	Cholbam	Retrophin (Sanofi)	$245M

Stockpiling data from FY2014 Budget for Public Health and Social Services Emergency Fund, pg 116
http://www.hhs.gov/budget/fy2014/fy2014-phssef.pdf

Voucher data sourced from public reports

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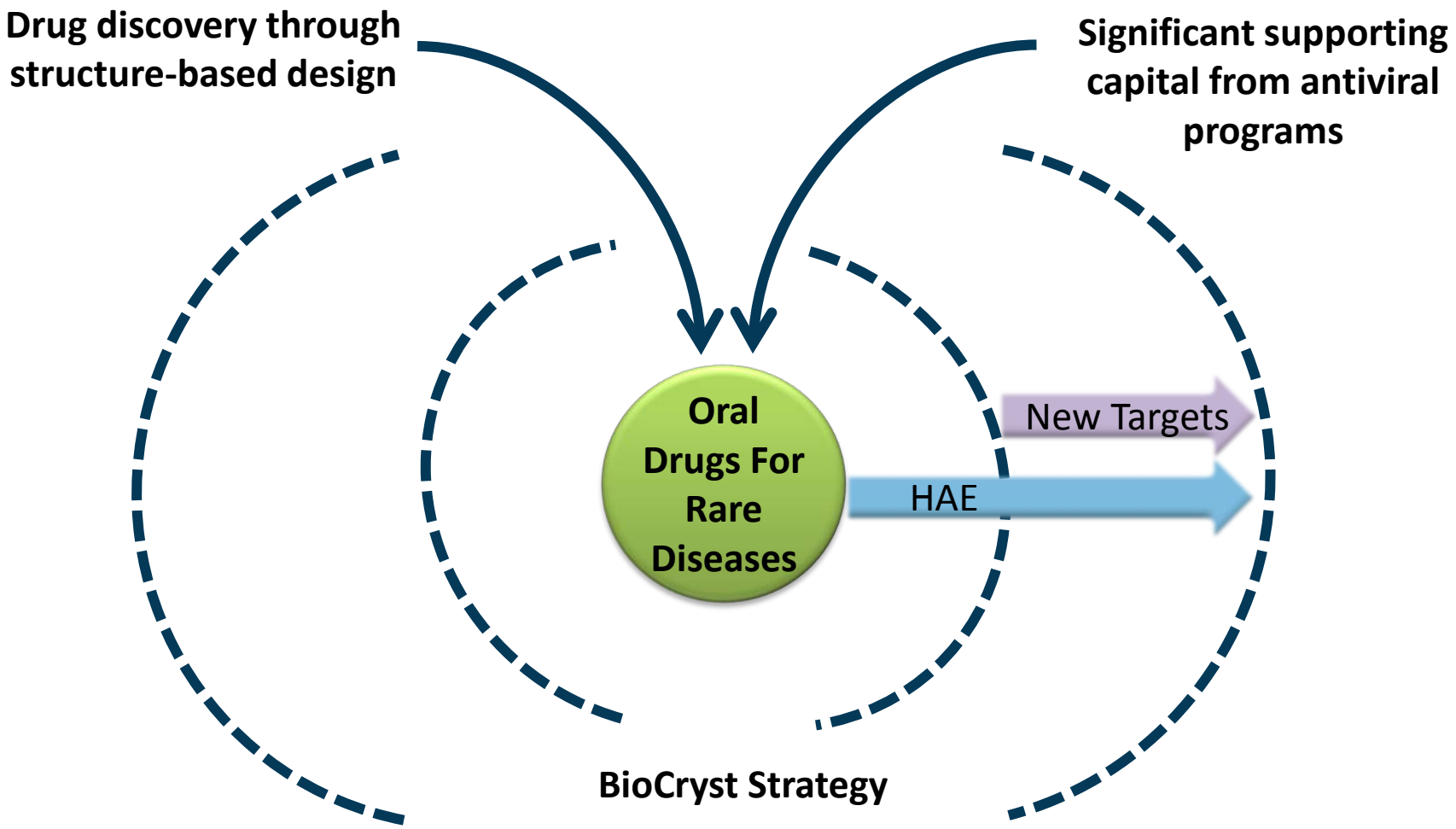
Building a company to generate expanding and sustainable value



Cash position & 2017 guidance (in millions)

Cash & investments at December 31, 2016	$65
Cash & investments at March 31, 2017	**$105**
Senior Credit Facility	$23

Guidance for 2017:

Operating cash utilization	$30 – 50
Operating expenses[#]	$53 – 73

[#] Excludes equity-based compensation.

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BCX7353 – APeX-1
Second Interim Analysis Results
Backups

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Rate of overall confirmed attacks: ITT population

Treatment	n	LS mean[1] Attacks per Week	Difference vs Placebo	Percentage Reduction vs Placebo	p-Value vs Placebo
Effective dosing period (Week 2-4) – ITT Population					
BCX7353 combined	31	0.457	-0.481	51%	0.002
BCX7353 125 mg	7	0.249	-0.689	73%	0.004
BCX7353 250 mg	6	0.526	-0.411	44%	0.090
BCX7353 350 mg	18	0.515	-0.423	45%	0.014
Placebo	20	0.938	-	-	-
Part 1 Interim analysis:					
BCX7353 350 mg	*14*	*0.436*	*-0.474*	*52%*	*0.035*
Placebo	*14*	*0.911*			

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

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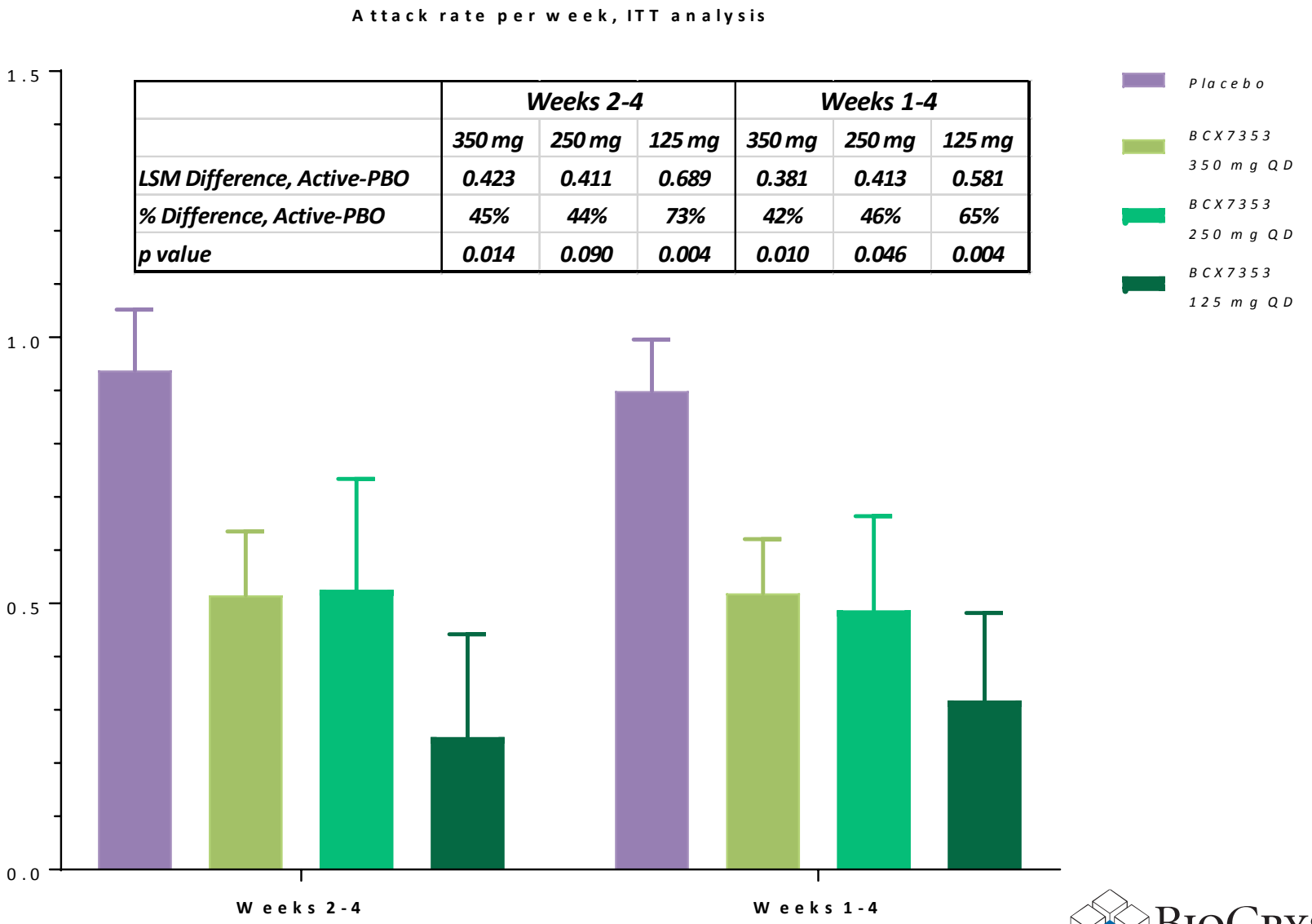
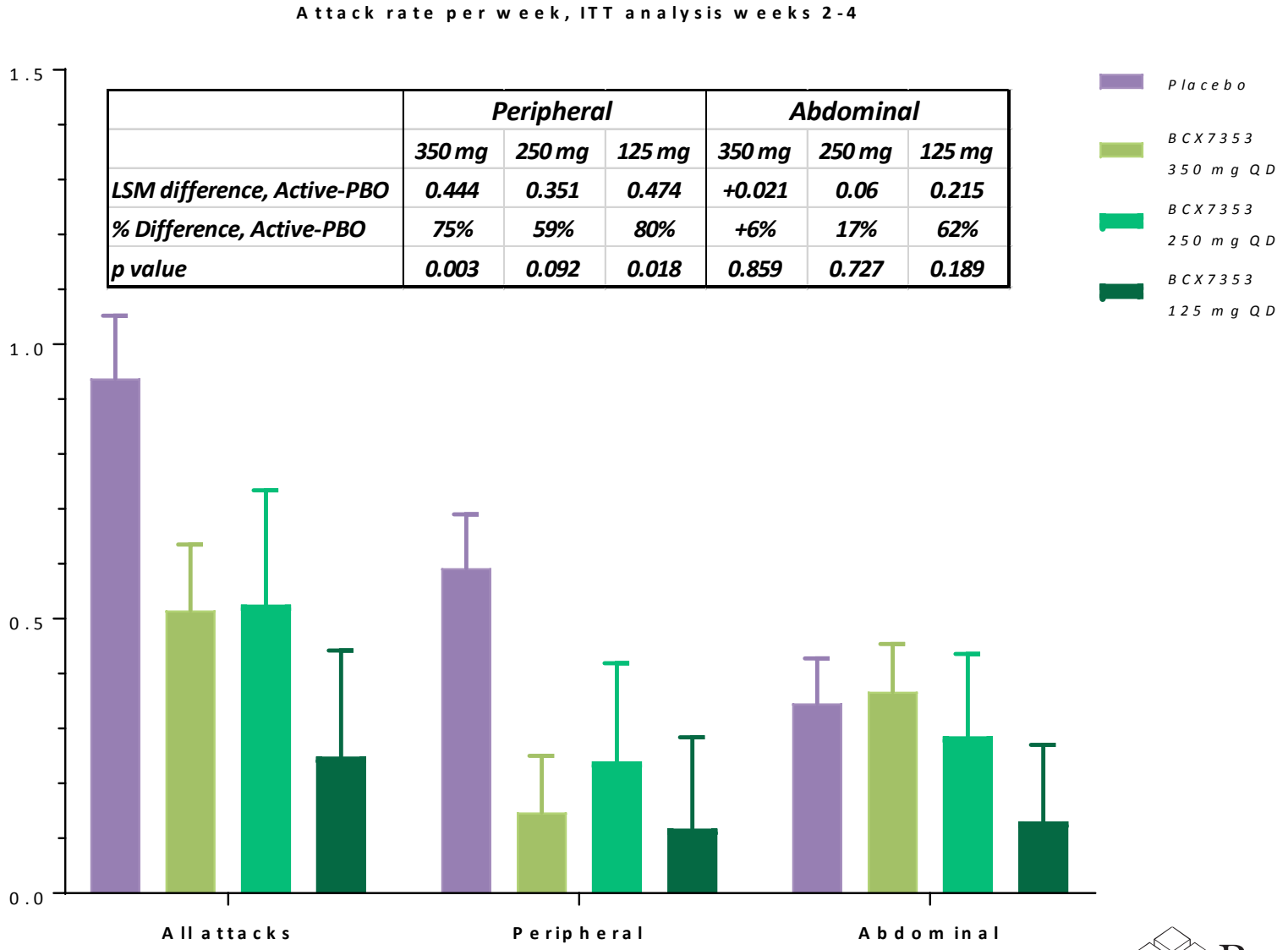
Overall attack rate, ITT analysis, weeks 2-4 and 1-4

	Weeks 2-4			Weeks 1-4		
	350 mg	250 mg	125 mg	350 mg	250 mg	125 mg
LSM Difference, Active-PBO	0.423	0.411	0.689	0.381	0.413	0.581
% Difference, Active-PBO	45%	44%	73%	42%	46%	65%
p value	0.014	0.090	0.004	0.010	0.046	0.004

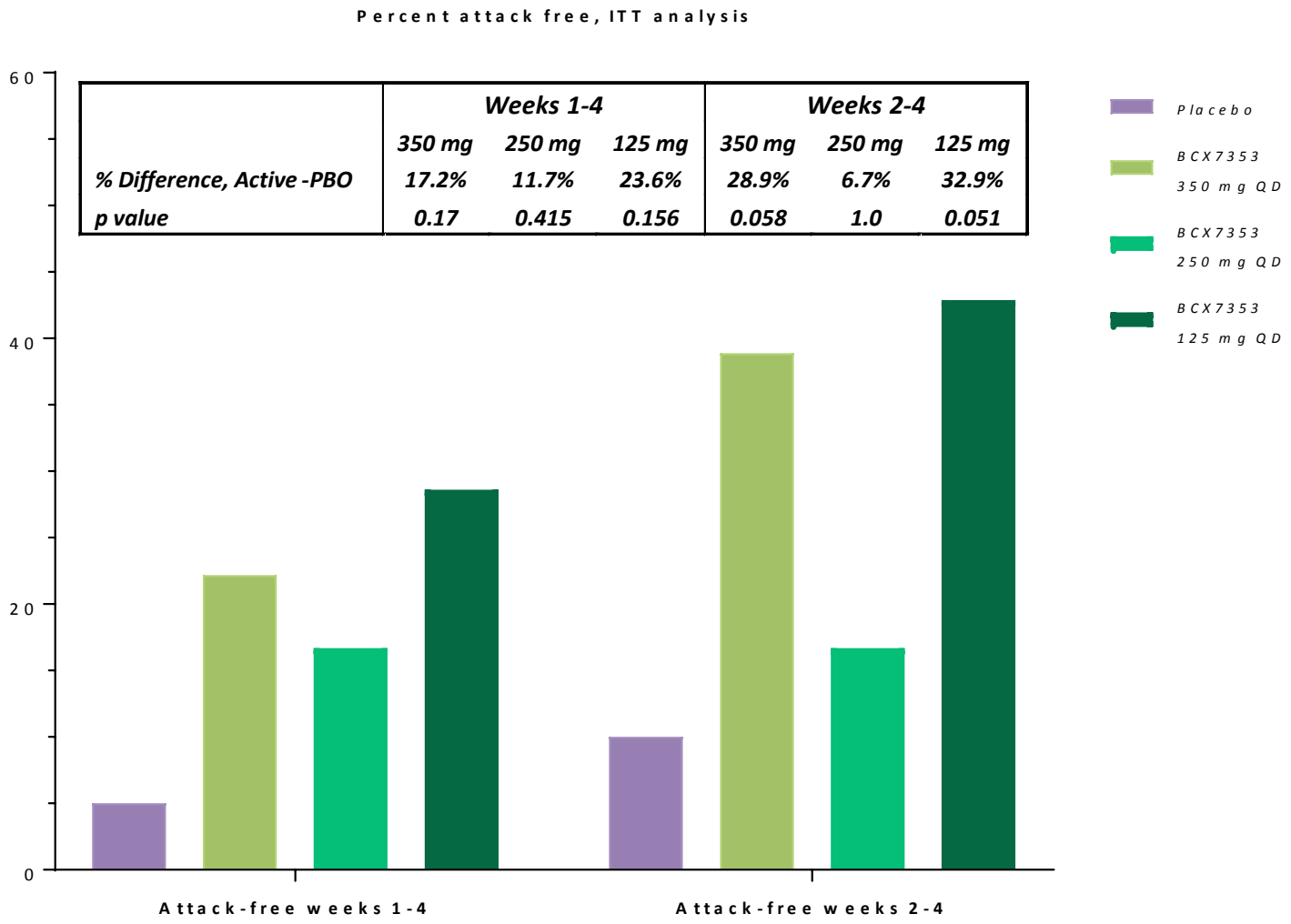


Attack rates by prespecified anatomical location, ITT

	Peripheral			Abdominal		
	350 mg	250 mg	125 mg	350 mg	250 mg	125 mg
LSM difference, Active-PBO	0.444	0.351	0.474	+0.021	0.06	0.215
% Difference, Active-PBO	75%	59%	80%	+6%	17%	62%
p value	0.003	0.092	0.018	0.859	0.727	0.189

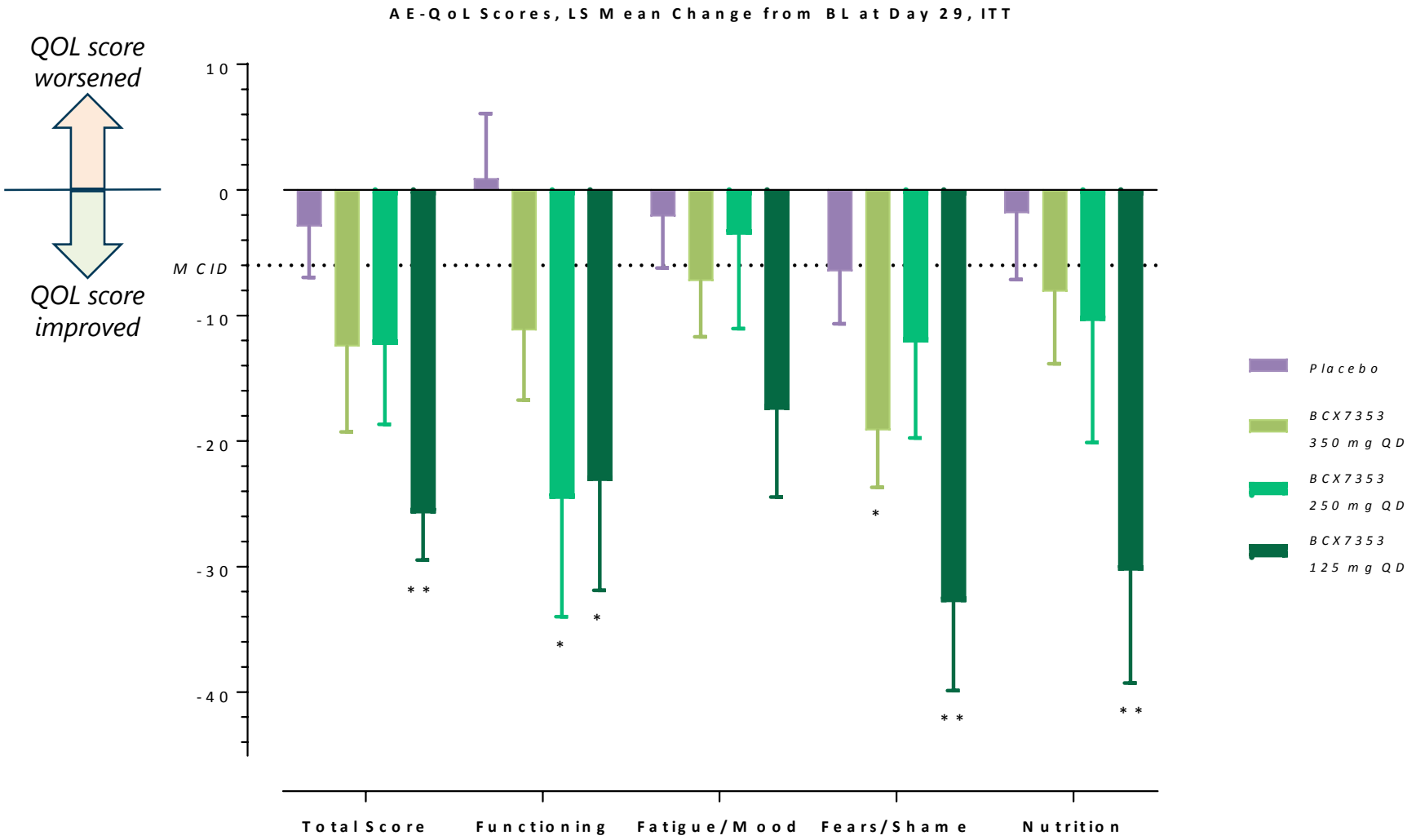


Percent of subjects attack-free, ITT analysis



	Weeks 1-4			Weeks 2-4		
	350 mg	250 mg	125 mg	350 mg	250 mg	125 mg
% Difference, Active -PBO	17.2%	11.7%	23.6%	28.9%	6.7%	32.9%
p value	0.17	0.415	0.156	0.058	1.0	0.051

Quality of life scores, ITT analysis of change from baseline



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.)*
* $p < 0.05$, ** $p < 0.01$, BCX7353 dose level compared with placebo