



Bank of America Merrill Lynch Health Care Conference

May 16th, 2017

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Forward-looking statement

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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

**Oral
Drugs For
Rare
Diseases**

Help patients lead normal lives

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

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

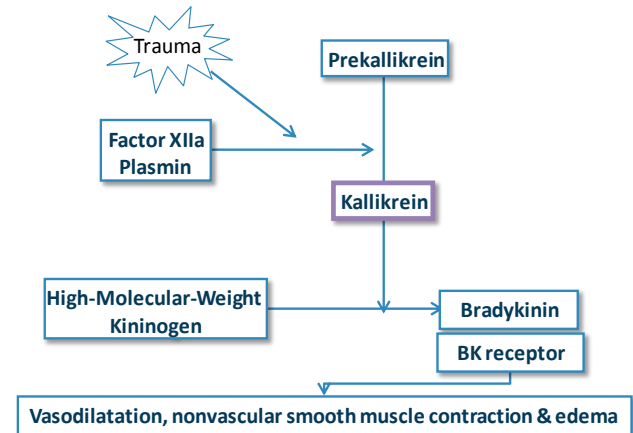


Unpredictable, debilitating, potentially life-threatening swelling attacks

- 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

Images obtained from www.haeimages.com
Market estimates based on analyst reports, earnings reports, and market data



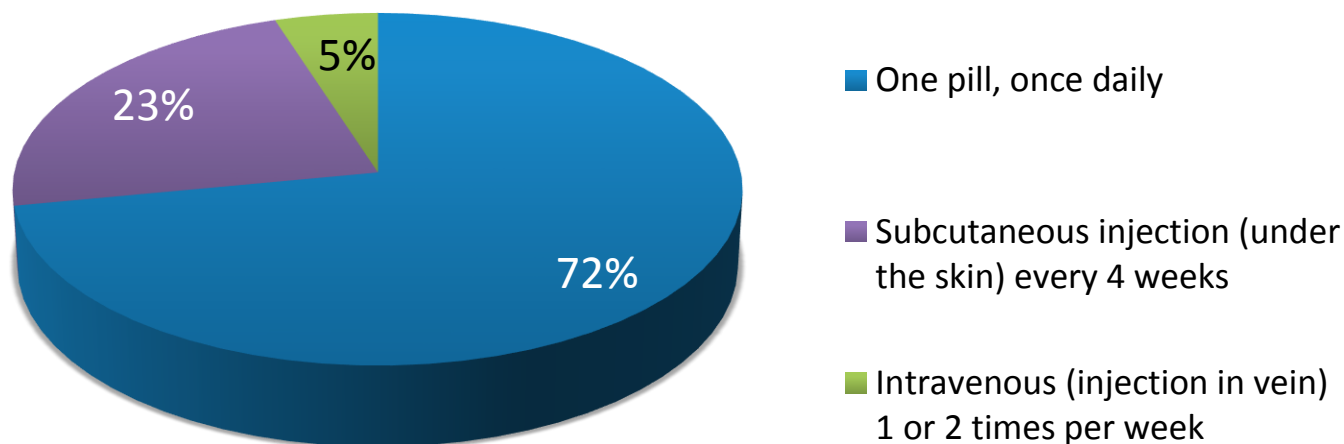
Contact activation pathway is unbalanced based on genetic deficiency of C1 inhibitor (C1-INH)

- 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

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?

APeX-1 Interim analysis: Rate of overall confirmed attacks

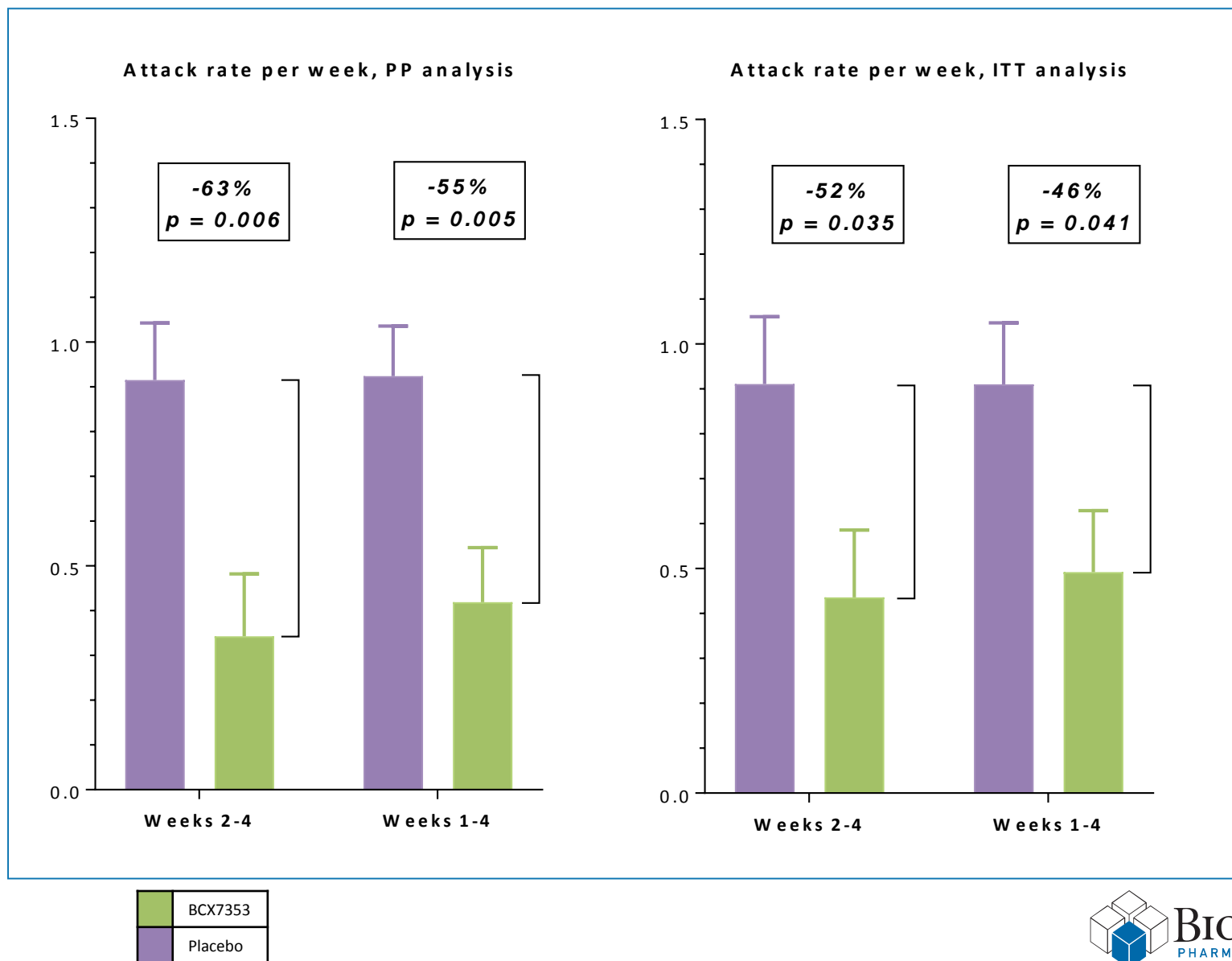
Treatment	n	LS mean ¹ Attacks per Week	Difference vs Placebo	Percentage Reduction vs Placebo	p-Value vs Placebo
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Effective dosing period (Week 2-4) – PP Population					
BCX7353 350 mg	11	0.343	-0.572	63%	0.006
Placebo	13	0.915			

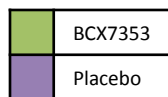
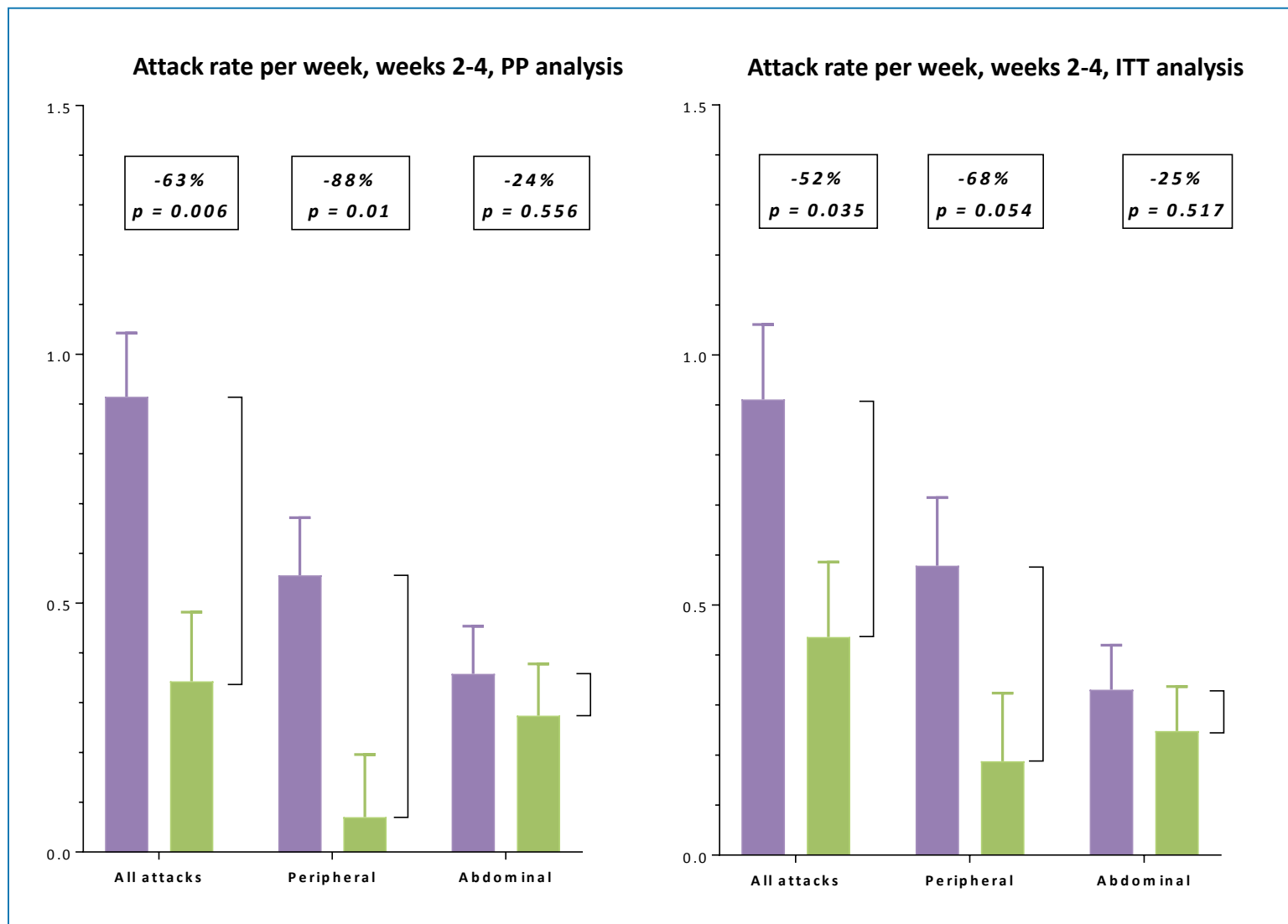
Effective dosing period (Week 2-4) – ITT Population					
BCX7353 350 mg	14	0.436	-0.474	52%	0.035
Placebo	14	0.911			

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

APeX-1 Interim analysis: Overall angioedema attack rate



APeX-1 Interim analysis: Angioedema attack rates by pre-specified anatomical location



APeX-1 Interim analysis: Angioedema attacks by anatomical category

	Peripheral		Mixed		Abdominal	
Effective dosing period (Week 2-4) – Per Protocol Population						
	Attacks	Subjects	Attacks	Subjects	Attacks	Subjects
BCX7353	2	2	2	1	7	5
Placebo	22	9	12	7	2	1
% Change vs Placebo	-91%		-83%		+250%	

Effective dosing period (Week 2-4) – ITT Population						
BCX7353	6	3	3	2	7	5
Placebo	25	10	12	7	2	1
% Change vs Placebo	-76%		-75%		+250%	

Clear imbalance in attack reduction by location. Subjects may not have been able to distinguish between BCX7353-related GI events and early signs of an abdominal attack.

Post- hoc analysis

BCX7353 Phase 1 Daily dosing: Adverse events occurring in >1 subject

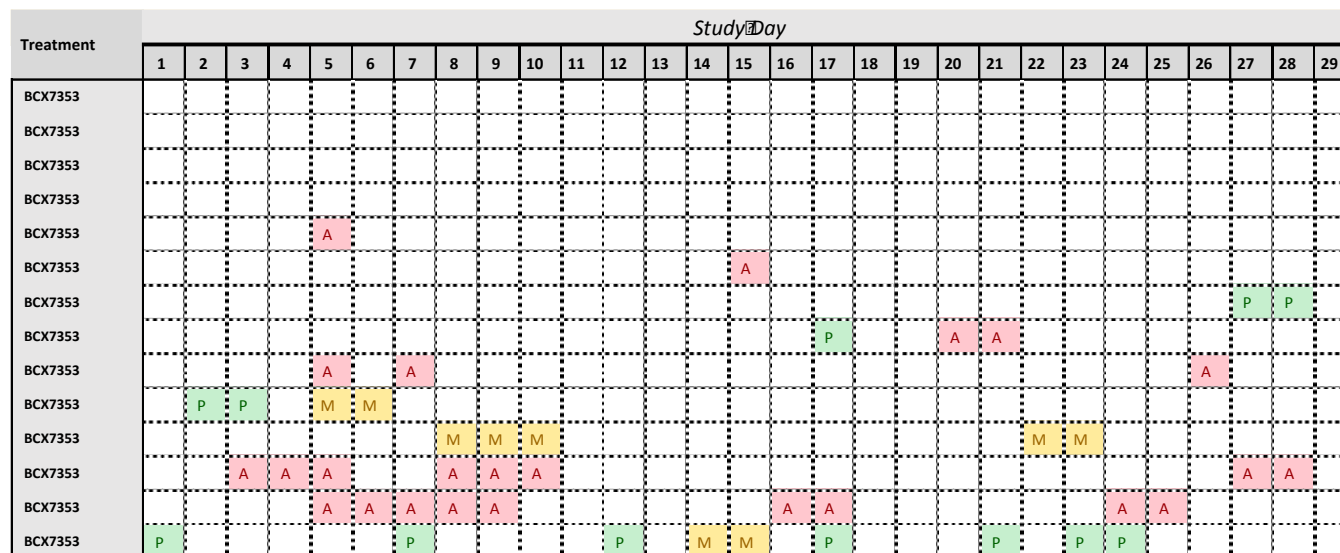
	Placebo	BCX7353			Placebo	BCX7353
<i>Dosing regimen</i>	<i>Once daily for 7 days</i>				<i>Once daily for 14 days</i>	
<i>Dose</i>	--	125mg	250mg	500 mg	--	350mg
<i>N</i>	6	10	10	10	2	10
Subjects (%) reporting an AE	2 (33.3)	2 (20.0)	2 (20.0)	7 (70.0)	2 (100.0)	8 (80.0)
Total number of AEs	2	5	6	22	2	21
Nature of AE						
Diarrhea	0	1 (10.0)	0	5 (50.0)	0	0
Flatulence	0	0	0	2 (20.0)	0	0
Abdominal pain	0	0	1 (10.0)	1 (10.0)	0	3 (30.0)
Abdominal distension	0	0	0	1 (10.0)	0	1 (10.0)
Dyspepsia	0	0	0	0	0	2 (20.0)
Epigastric discomfort	0	0	0	0	0	2 (20.0)
Nausea	0	0	0	1 (10.0)	0	1 (10.0)
Dizziness	0	1 (10.0)	0	1 (10.0)	0	1 (10.0)
Headache	0	1 (10.0)	0	1 (10.0)	0	1 (10.0)
Upper Resp Tract Infection	0	0	0	0	0	2 (20.0)

APeX-1 Interim analysis: Analysis of gastrointestinal symptoms in the subject diary

AE or symptom	Reported as AE		Reported as attack-related symptoms			
			Mixed peripheral + abdominal attack category ¹		Abdominal-only attack category ¹	
	BCX7353 (n=14)	Placebo (n=14)	BCX7353 (n=14)	Placebo (n=14)	BCX7353 (n=14)	Placebo (n=14)
Abdominal pain	1 (7.1%)	0	1 (7.1%)	5 (35.7%)	7 (50.0%)	3 (21.4%)
Nausea	1 (7.1%)	0	1 (7.1%)	5 (35.7%)	4 (28.6%)	2 (14.3%)
Vomiting	1 (7.1%)	0	0	0	1 (7.1%)	1 (7.1%)

¹ Includes all subject-reported attacks, including those rejected by expert adjudication committee. Multiple reports of the same event in the same subject are only tabulated once

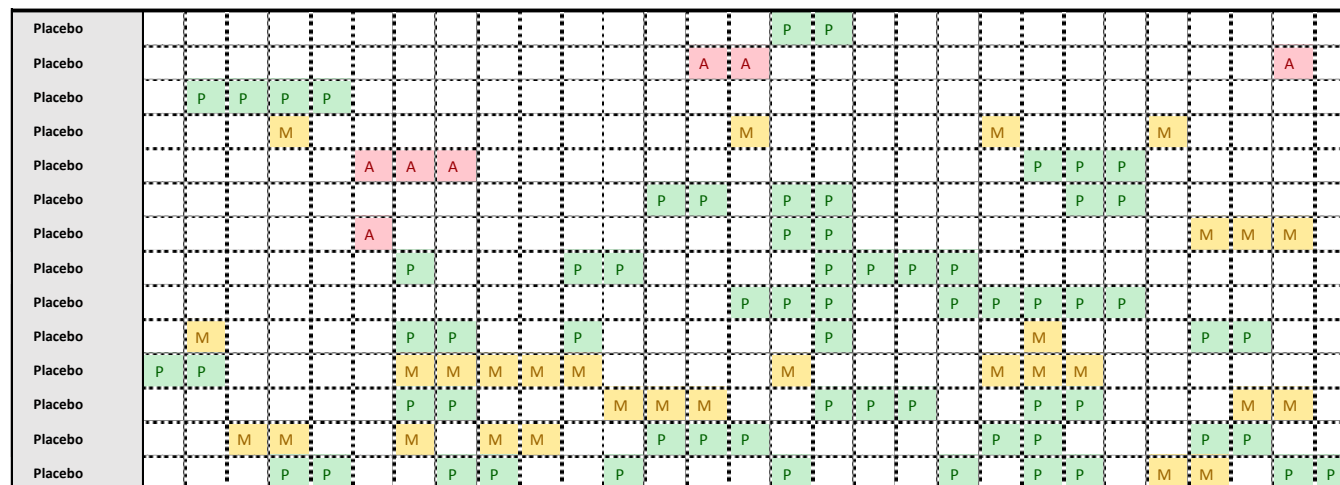
APeX-1 Interim analysis: Days with any angioedema symptoms recorded in the subject diary, by anatomical category



A Abdominal only

M Mixed

P Peripheral only



Post-hoc analysis including all days with any symptoms recorded by subjects as attack of HAE.

Analysis of ITT population, adjudicated attacks.

Study subjects (rows) in each treatment group ordered by increasing number of days with symptoms.



Analysis of ITT population, adjudicated attacks.



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BCX7353 APeX-1 interim analysis: Safety summary

Category	BCX7353 (n=14)	Placebo (n=14)
Number of Subjects with any Serious AE, n (%)	0	0
Number of Subjects with Drug-Related AE of Grade 3 or Grade 4, n (%)	0	0
Number of Subjects with AE Leading to Discontinuation from Study Drug, n (%)	2 (14.3)	0
Non- drug-related, n (%) Pre-existing liver disorder (improved from baseline, but persisting)	1 (7.1)	0
Drug-related, n (%) 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)	1 (7.1)	0
Treatment-Emergent Adverse Events occurring in ≥ 2 subjects overall, n (%)		
Nasopharyngitis (common cold)	3 (21.4)	4 (28.6)
Diarrhea	4 (28.6)	2 (14.3)
Flatulence	2 (14.3)	0
Fatigue	2 (14.3)	0
Clinically significant changes in clinical chemistry, hematology or urinalysis, n (%)		
ALT elevation > 3X ULN (ALT 3.9 x ULN, AST 1.8 xULN, GGT10.7 X ULN) <i>Pre-existing colitis, hepatic steatosis (fatty liver), > 20 years androgen use until 3 years prior to study, Baseline increase in liver enzymes</i>	1 (7.1)	0

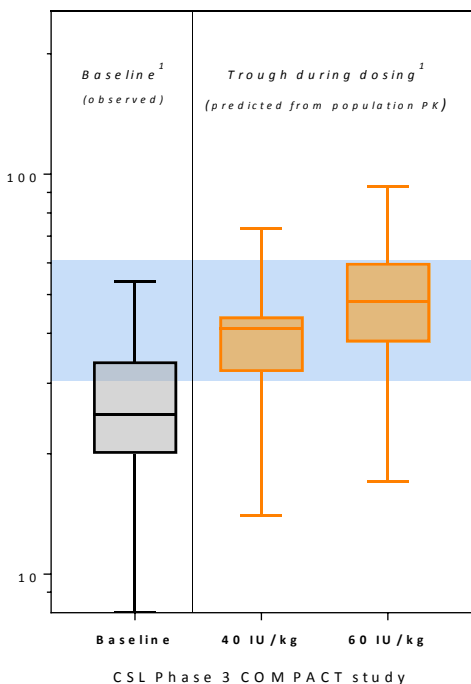
Exposure comparisons and population PK modeling support evaluation of lower doses of BCX7353

CSL-830 Phase 3 study

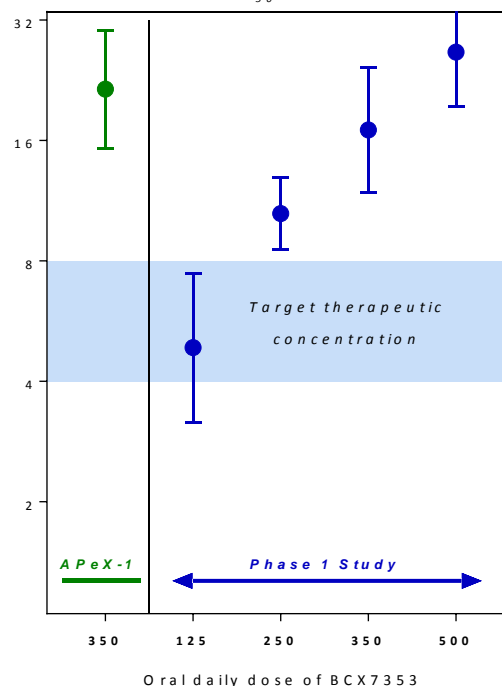
BCX7353 APeX-1 & Phase 1

PK Modeling

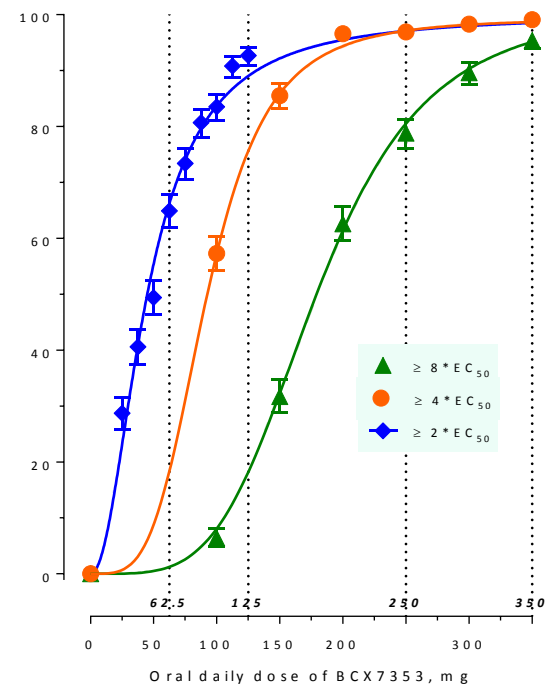
C1INH levels in COMPACT study
% of normal mean Multiple of EC_{50}



BCX7353 Trough Concentrations
Multiple of EC_{50} [geo. mean & SD]



Percent of patients predicted above target



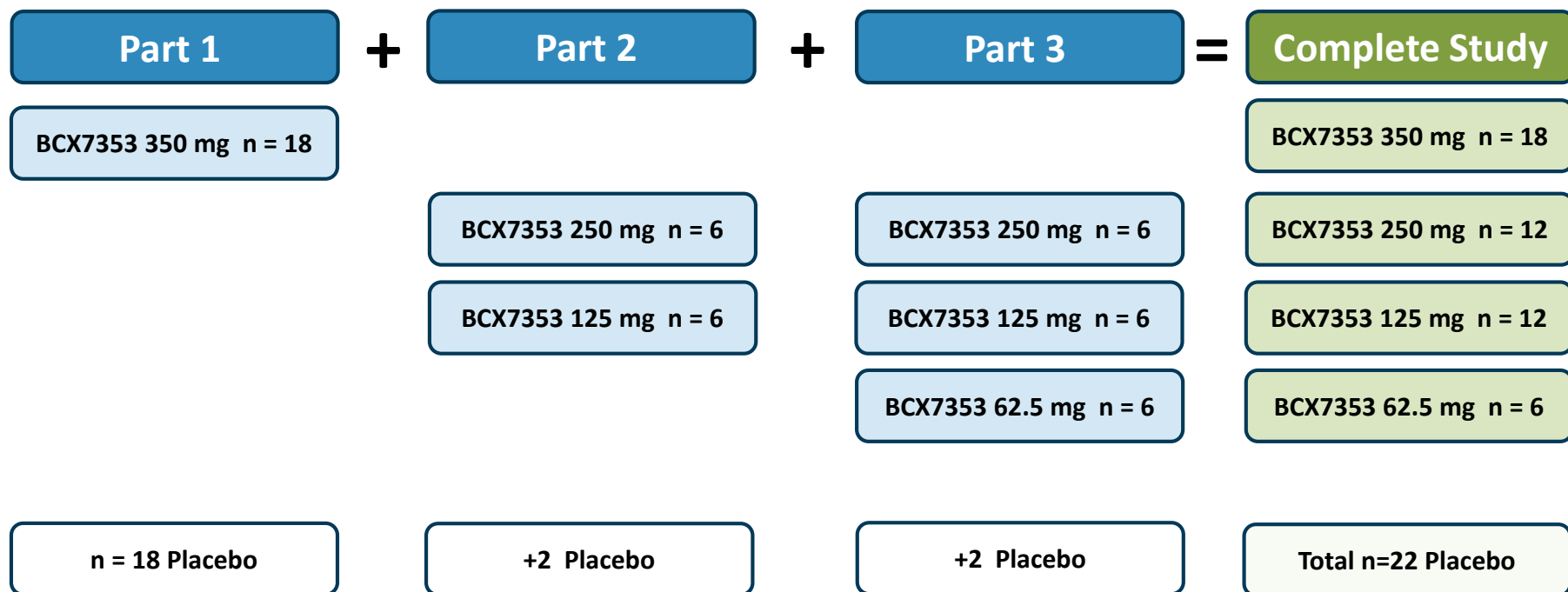
C1INH levels at baseline and after SC dosing with CSL-830¹

BCX7353 plasma concentrations at 24 hours post-dose

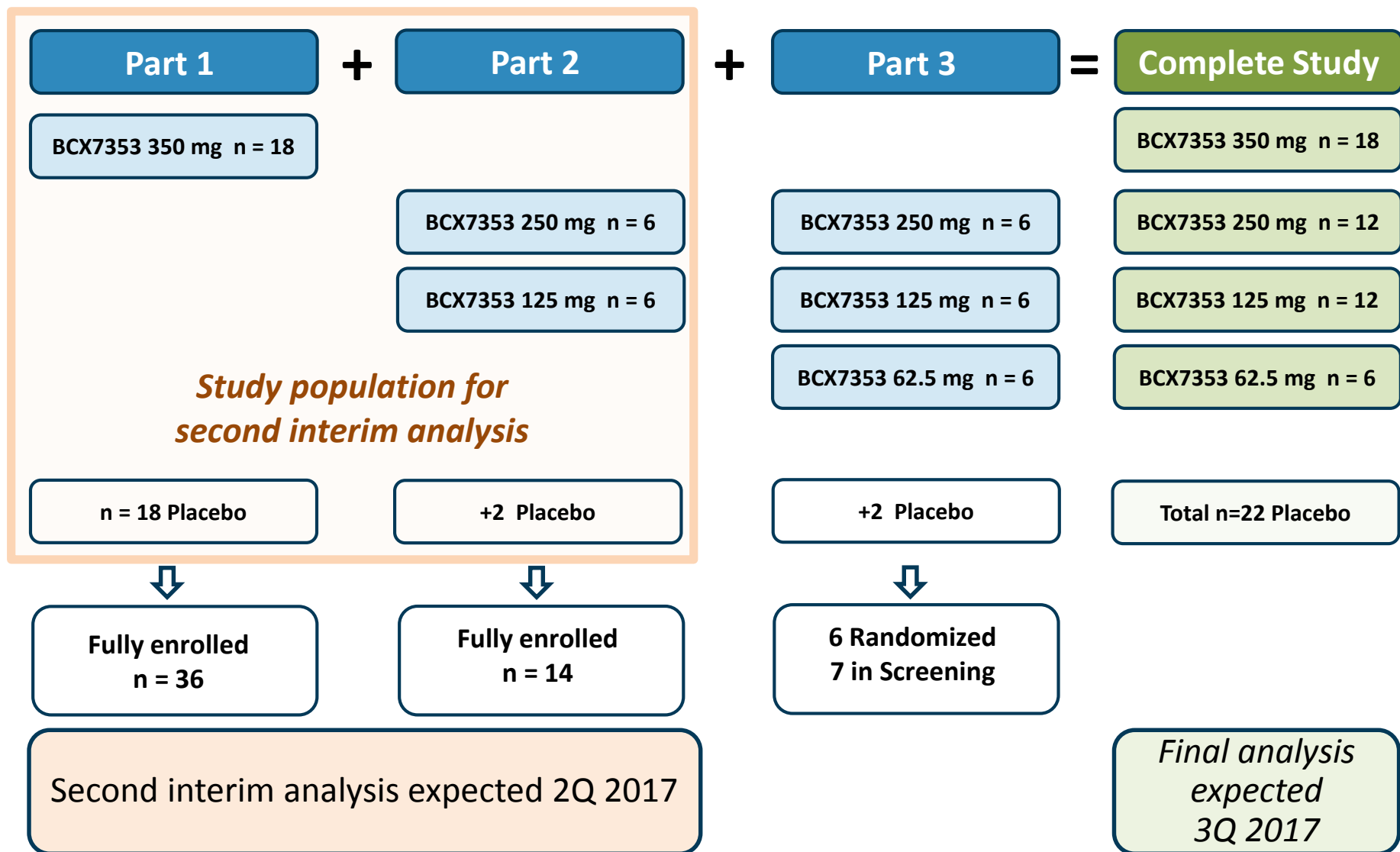
Monte Carlo simulation: 1000 subjects per data point

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.

APeX-1



APeX-1: update



APeX-1 trial second interim analysis plan

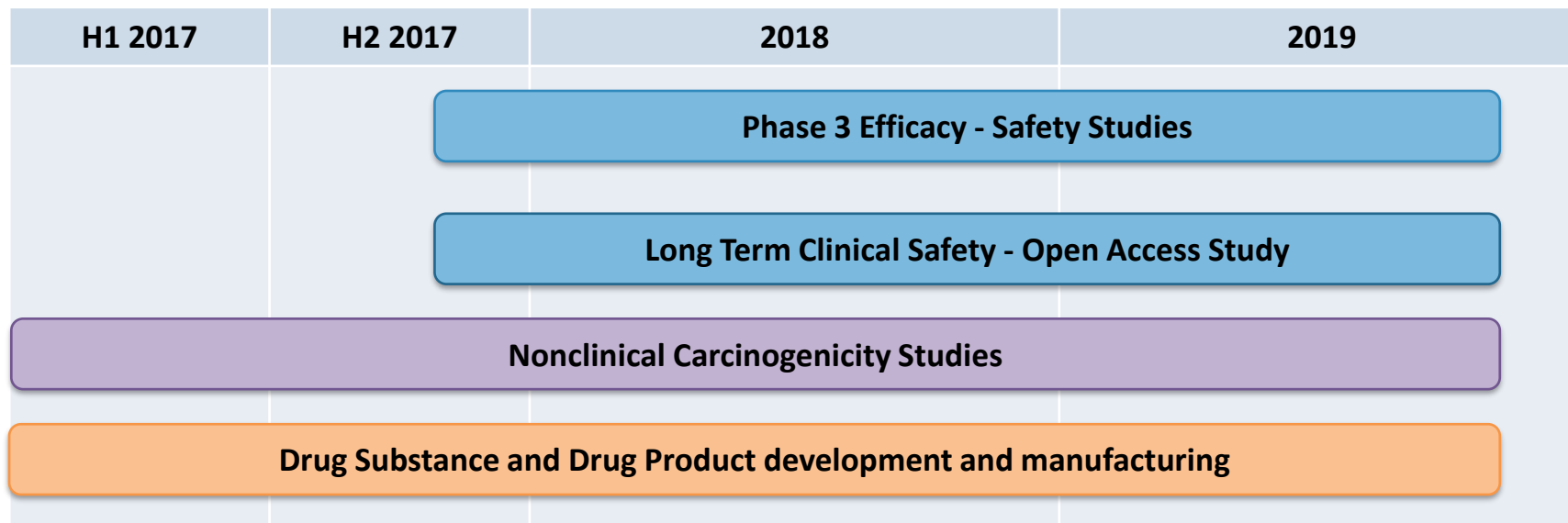
- Scope of analyses will be similar to the first interim analysis:
 - Subject demographics
 - Efficacy
 - Safety
 - PK
 - Kallikrein inhibition

<i>Planned N</i>	Placebo	125 mg QD BCX7353	250 mg QD BCX7353	350 mg QD BCX7353	All BCX7353
	20	6	6	18	30

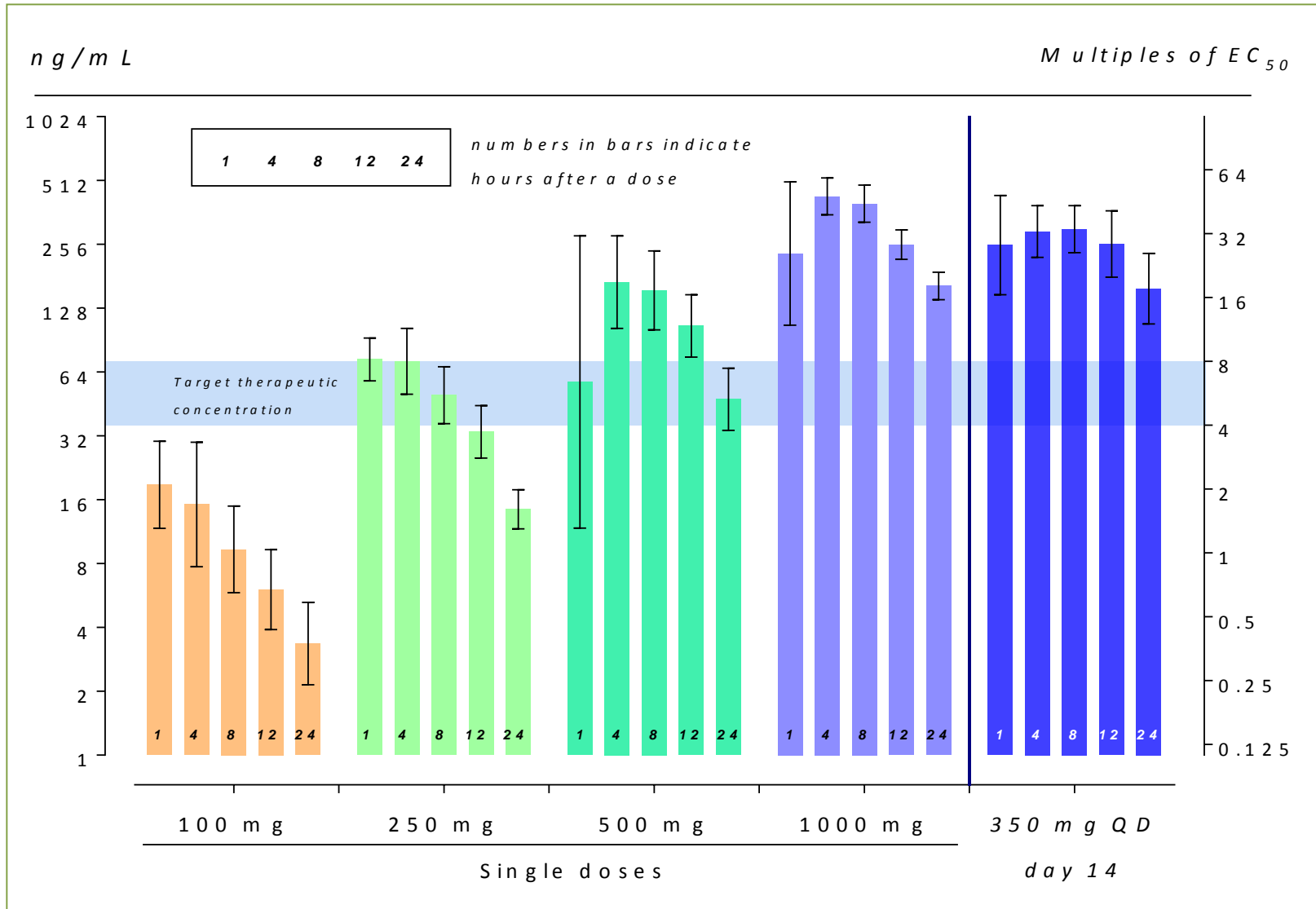
<i>Comparisons of interest</i>	PBO vs all BCX7353
	PBO vs 350 mg QD
	PBO vs 250 mg QD
	PBO vs 125 mg
	Dose comparison

BCX7353 Remaining activities after APeX-1

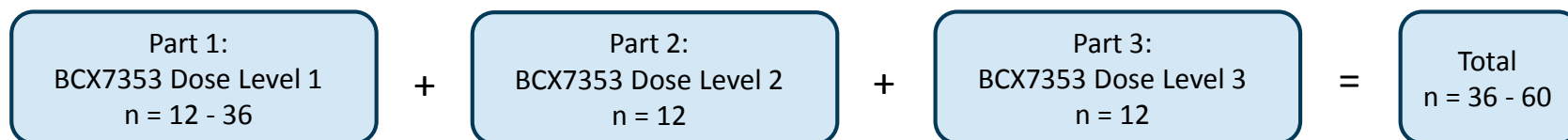
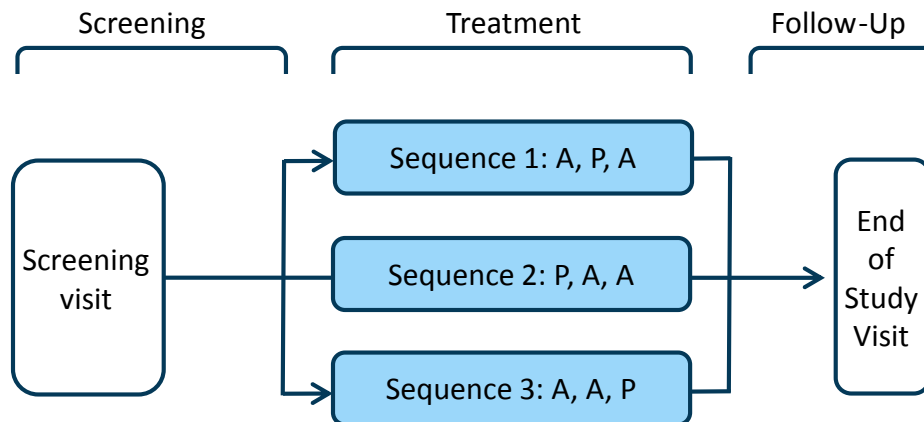
Estimated timing of key activities to support NDA/MAA filing



Phase 1 PK data support evaluation of BCX7353 for treatment of angioedema attacks




ZENITH-1 trial design



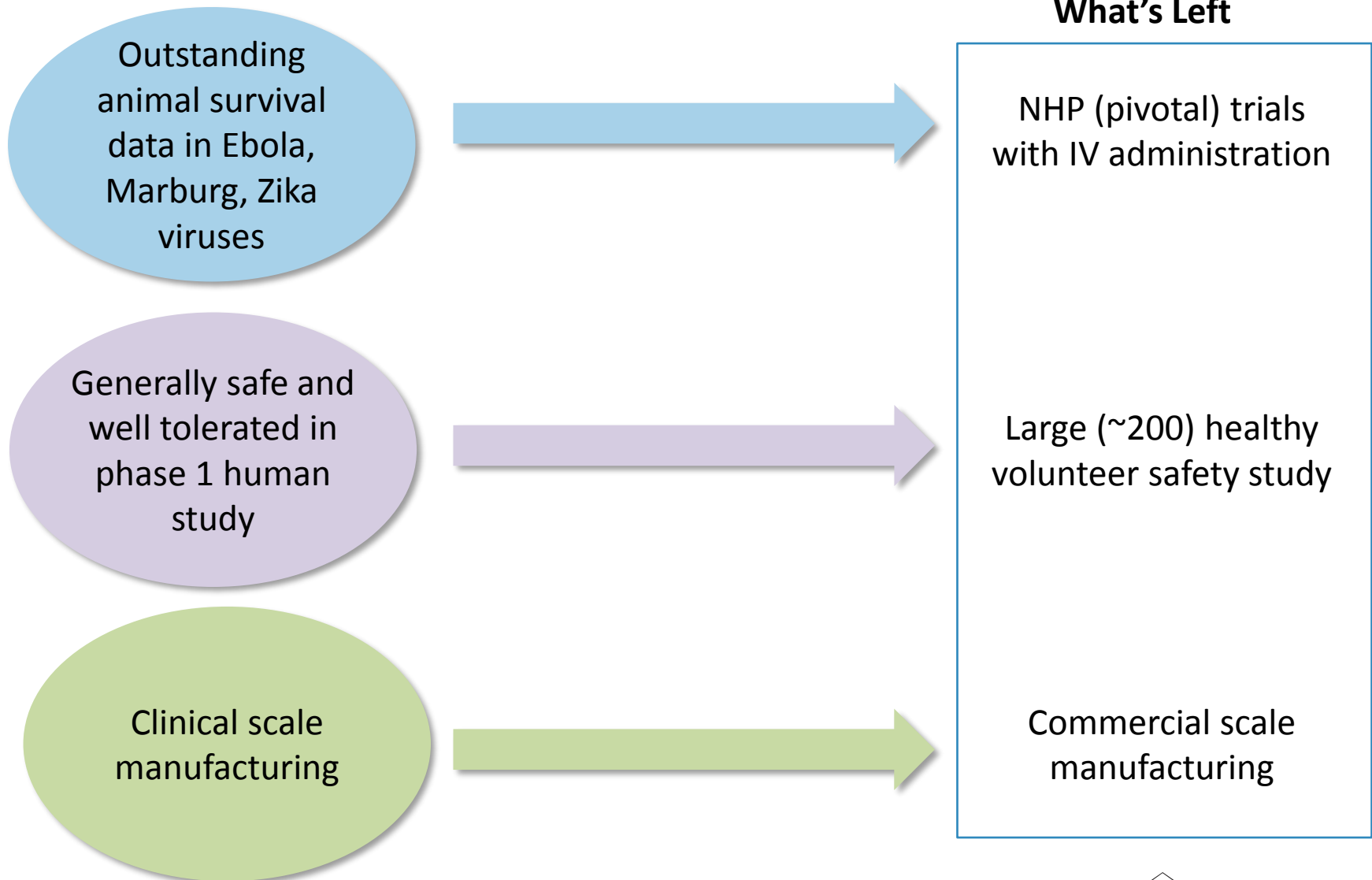
- The ZENITH-1 trial will evaluate the potential for an oral liquid formulation of BCX7353 to treat acute angioedema attacks
- Each subject is intended to have 3 attacks treated with blinded study drug
 - 2 with BCX7353 (A) and 1 with Placebo (P)
- Primary efficacy endpoint: proportion of subjects with 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	<ul style="list-style-type: none"> • Over \$90M in milestones and royalty monetization • Over \$25M in Government stockpiling (Japan/US)
Galidesivir (BCX4430)	<ul style="list-style-type: none"> • Ebola is lead indication • Broad-spectrum activity observed in Zika, Marburg and several other virus families 	Approximately \$80M US Government contract development funding	<ul style="list-style-type: none"> • 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

Galidesivir path to stockpiling and NDA



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
Botulinum 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

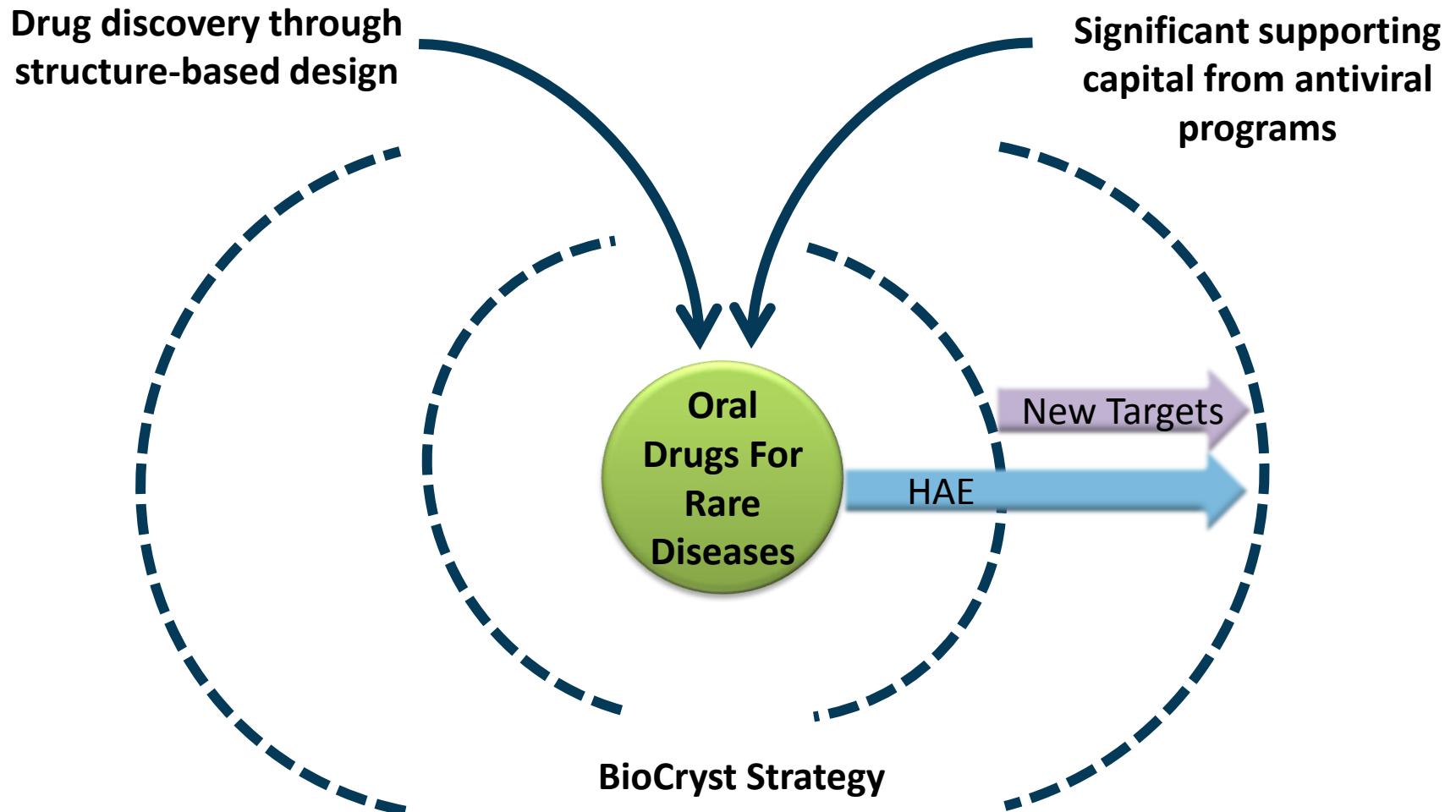
Disease	Drug	Seller (Buyer)	Price
Morquio A syndrome	Vimizim (elosulfase alfa)	BioMarin (Sanofi)	\$67.5M
Leishmaniasis	Impavido (miltefosine)	Knight (Gilead)	\$125M
High-risk neuroblastoma	Unituxin (dinutuximab)	United Therapeutics (Abbvie)	\$350M
Rare bile acid synthesis disorders	Cholbam	Retrophin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

Stockpiling data from FY2014 Budget for Public Health and Social Services Emergency Fund, pg 116

<http://www.hhs.gov/budget/fy2014/fy2014-phsgef.pdf>

Voucher data sourced from public reports

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.