Ladenburg Thalmann 2017 Healthcare Conference

September 26, 2017

Jon Stonehouse, *President & Chief Executive Officer*

BioCryst Pharmaceuticals, Inc.

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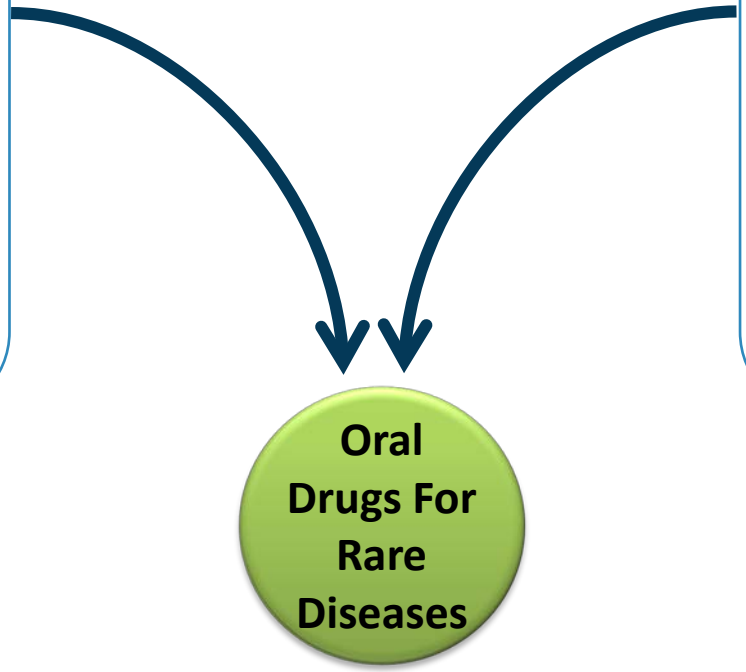
BioCryst's strategy is to develop oral drugs for rare diseases

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



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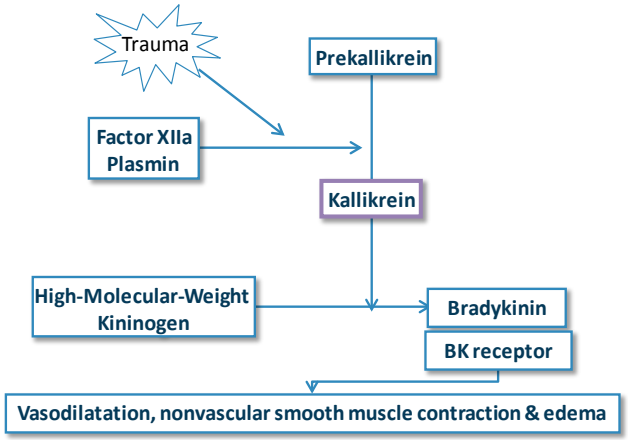
First target in strategy: Hereditary angioedema (HAE) is a high-need, high-value disease



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: ~$1.44B, 20% growth over 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 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
Market estimates based on analyst reports, earnings reports, and market data

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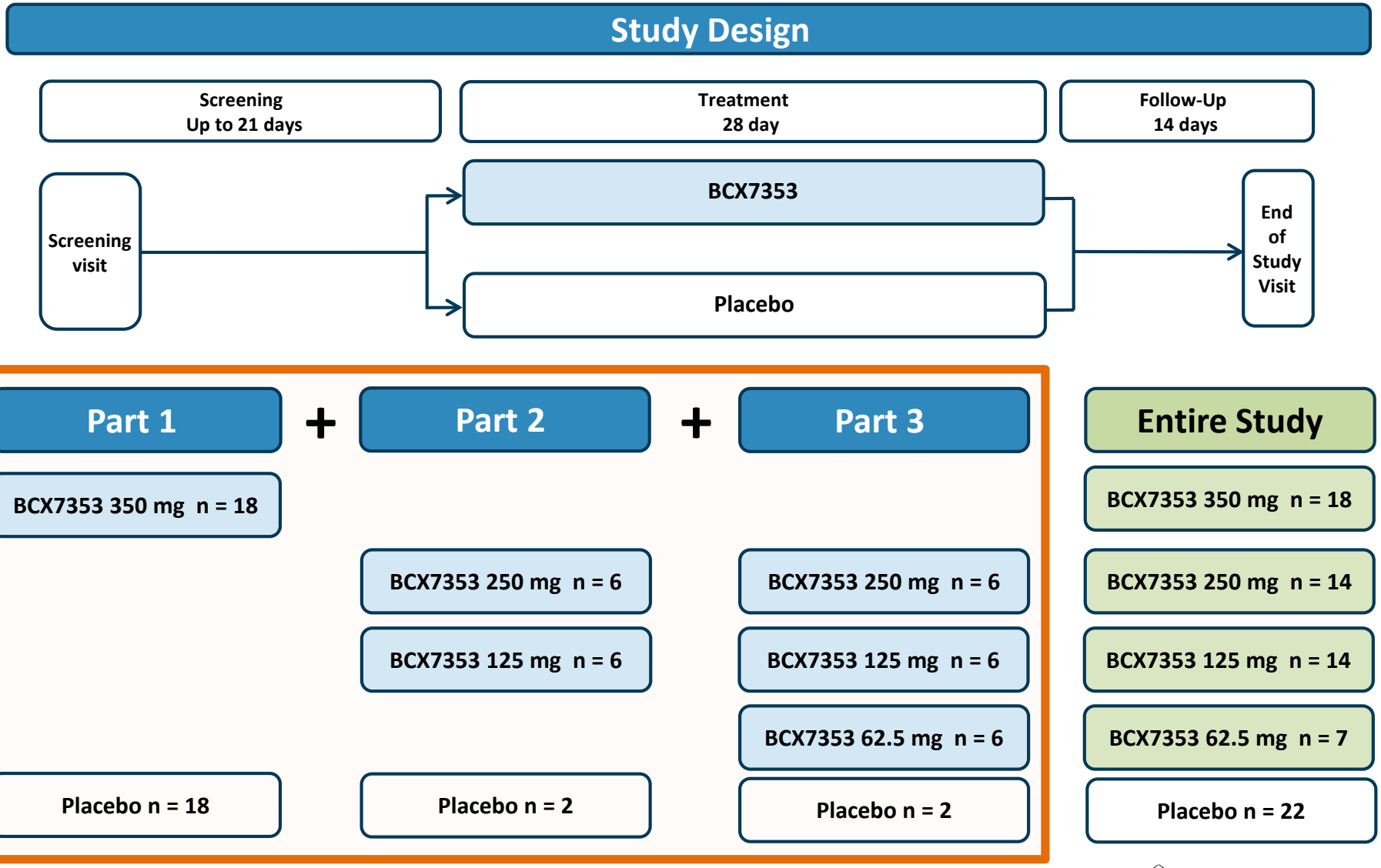
Highlights – APeX-1 Final Analysis

- 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

APeX-1 - Trial design and final enrollment



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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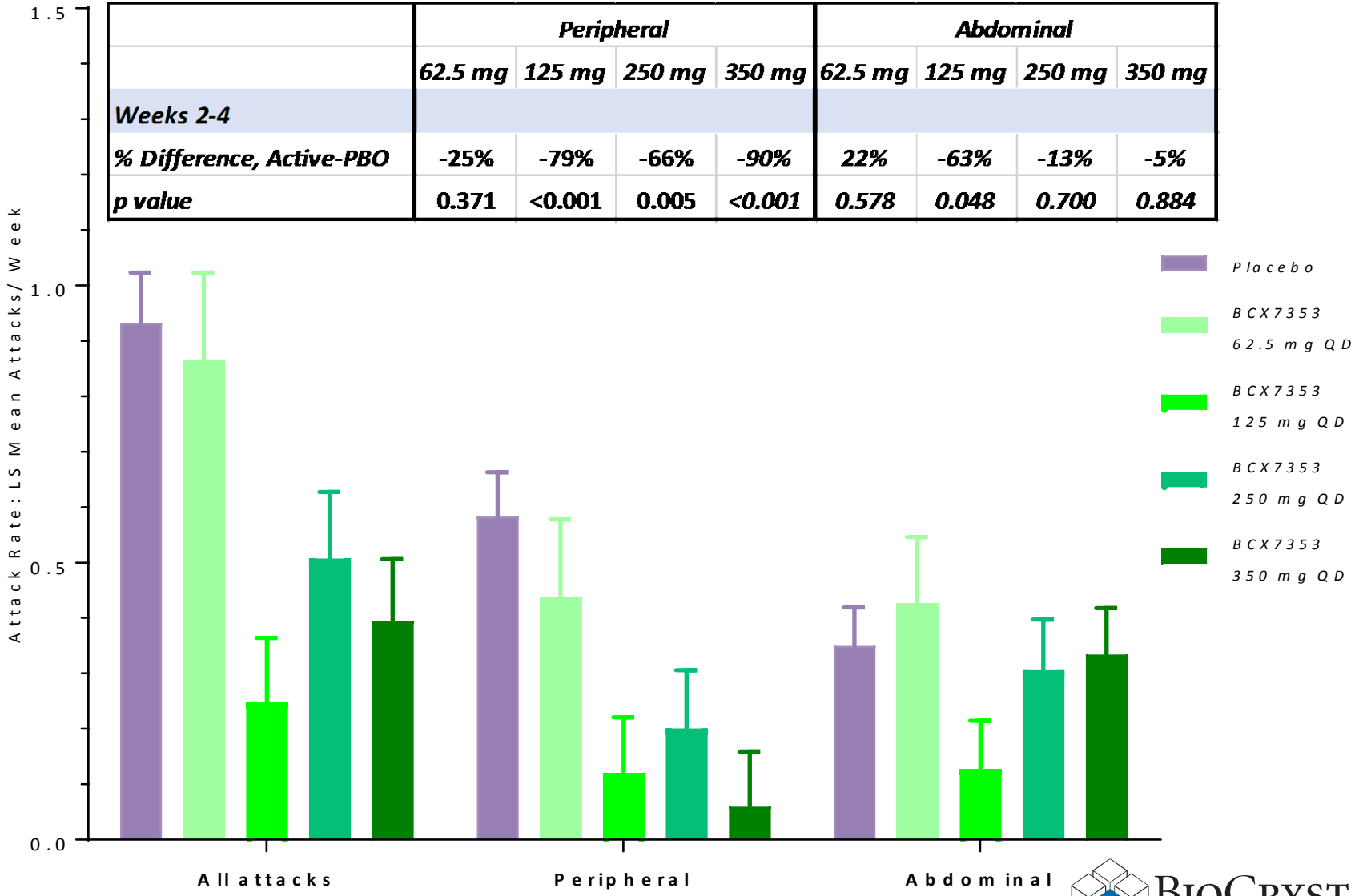
APeX-1 - Angioedema attack rates by prespecified anatomical location, PP

	Peripheral				Abdominal			
	62.5 mg	125 mg	250 mg	350 mg	62.5 mg	125 mg	250 mg	350 mg
Weeks 2-4								
% Difference, Active-PBO	-25%	-79%	-66%	-90%	22%	-63%	-13%	-5%
p value	0.371	<0.001	0.005	<0.001	0.578	0.048	0.700	0.884



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 - Most frequent treatment-emergent adverse events, other than gastrointestinal events

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
Treatment-Emergent Adverse Events occurring in ≥2 subjects overall, subject incidence n (%) in descending order System Organ Class (SOC) *Preferred Term*					
Infections and Infestations					
Nasopharyngitis	2 (29%)	0	1 (7%)	5 (28%)	6 (27%)
Upper Respiratory Tract Infection	0	0	1 (7%)	0	1 (5%)
Pharyngitis	0	0	1 (7%)	1 (6%)	0
Gastrointestinal infection	0	0	1 (7%)	1 (6%)	0
Nervous system disorders					
Headache	2 (29%)	2 (14%)	1 (7%)	1 (6%)	4 (18%)
Migraine	0	1 (7%)	0	1 (6%)	0
Musculoskeletal and connective tissue disorders					
Arthralgia	0	0	0	1 (6%)	1 (5%)
General disorders					
Fatigue	1 (14%)	0	0	2 (11%)	1 (5%)
Injury, poisoning and procedural complications					
Contusion	0	0	1 (7%)	0	1 (5%)
Investigations*					
Liver function tests	0	0	1 (7%)[1]	2 (11%)[2,3]	0

* Clinically significant changes and/or reported by investigator. Event in 250 mg group not reported as AE by investigator.

[1] Event previously reported: ALT elevation to >3X ULN (ALT 4.1 x ULN, AST 1.9 x ULN). Baseline increase in LFTs.*20 years androgen use*

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

[3] Investigator reported Grade 1 ALT elevation. *Prior androgen use.*

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APeX-1 - All gastrointestinal treatment-emergent adverse events

Category	BCX7353				Placebo
	62.5 mg	125 mg	250 mg	350 mg	
	N=7	N=14	N=14	N=18	N=22
Treatment-Emergent Adverse Events, subject incidence n (%), [number of events] in descending order SOC *Preferred Term*					
Gastrointestinal disorders					
Diarrhea	0	0	2 (14.3) [3]	4 (22.2) [6]	2 (9.1) [3]
Nausea	0	0	3 (21.4) [3]	3 (16.7) [5]	0
Abdominal pain	0	1 (7.1) [1]	1 (7.1) [1]	3 (16.7) [5]	0
Abdominal pain upper	1 (14.3) [1]	1 (7.1) [1]	0	1 (5.6) [1]	0
Gastroesophageal reflux disease	0	1 (7.1) [2]	0	0	1 (4.5) [1]
Flatulence	0	0	0	2 (11.1) [2]	0
Vomiting	0	0	0	2 (11.1) [5]	0
Constipation	0	0	0	1 (5.6) [1]	1 (4.5) [1]
Abdominal pain lower	0	0	0	1 (5.6) [2]	0
Abdominal discomfort	0	0	0	1 (5.6) [1]	0
Abdominal distension	0	0	0	1 (5.6) [1]	0
Dyspepsia	0	0	1 (7.1) [1]	0	0
Gingival erosion	0	0	0	1 (5.6) [1]	0
Toothache	0	0	1 (7.1) [1]	0	0
Breath odor	0	0	0	0	1 (4.5) [1]
Dental caries	0	0	0	0	1 (4.5) [2]

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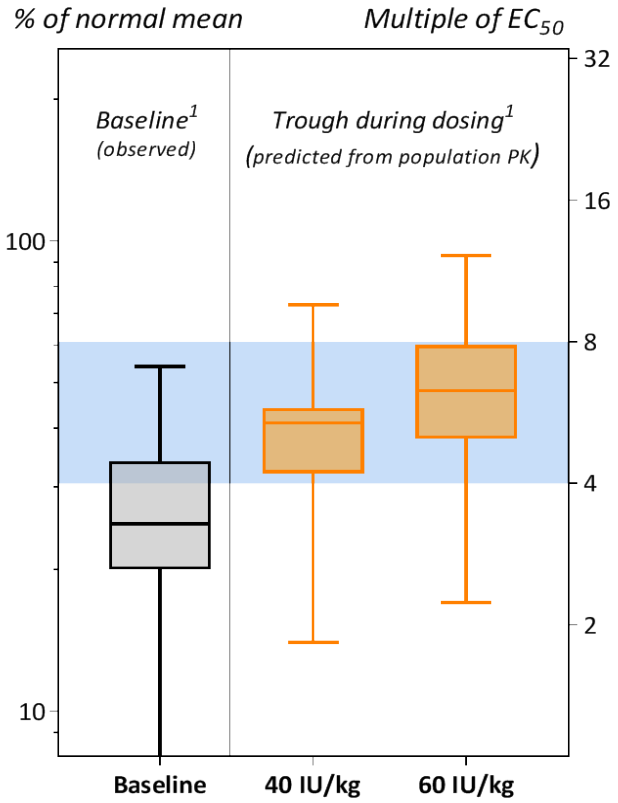
APeX-1 - Exposure comparisons of BCX7353 and SC C1INH

CSL-830 Phase 3 study

C1INH levels in COMPACT study

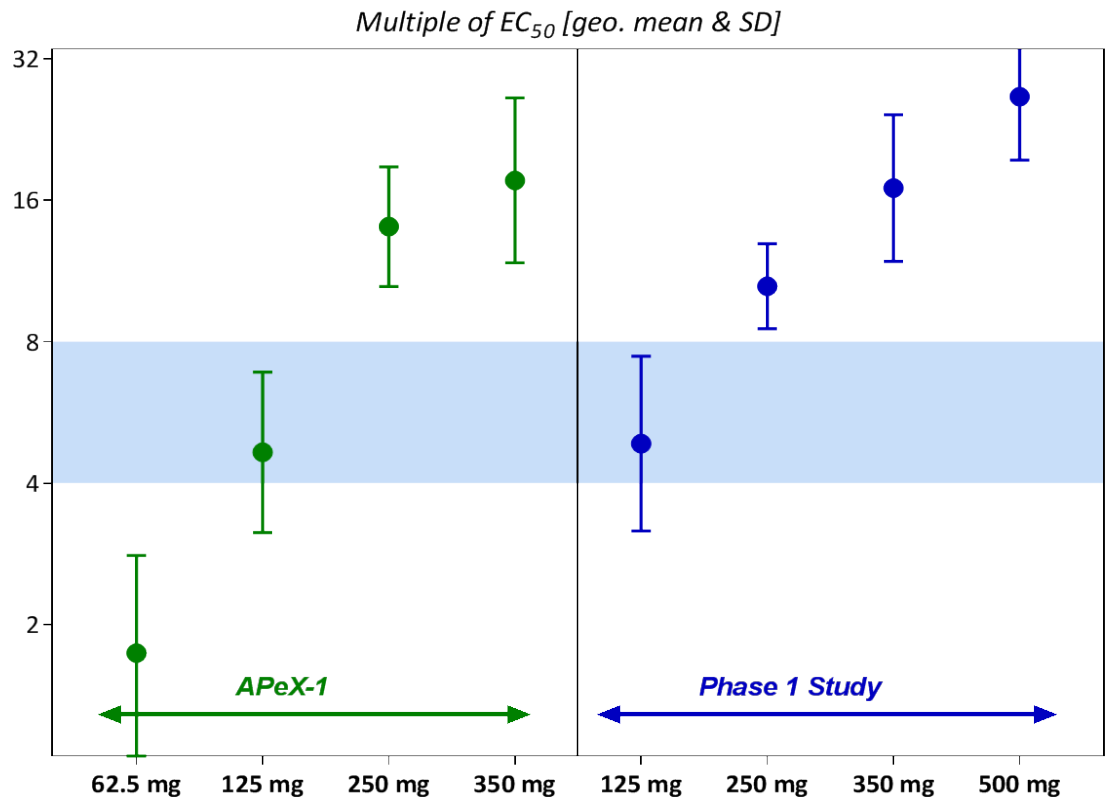


CSL Phase 3 COMPACT study

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

BCX7353 APeX-1 & Phase 1

BCX7353 Trough Concentrations



Oral daily dose of BCX7353

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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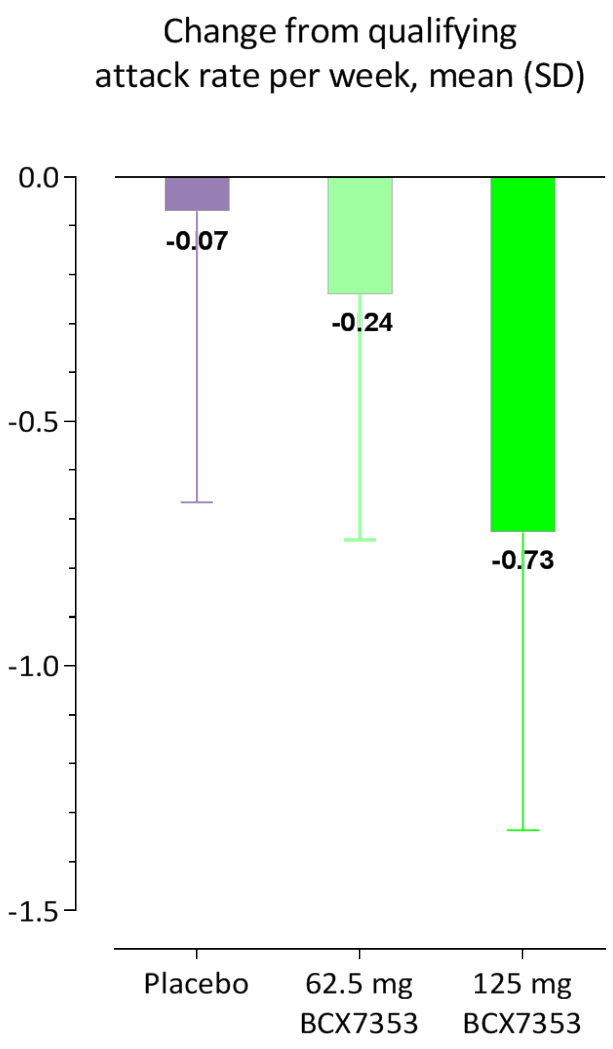
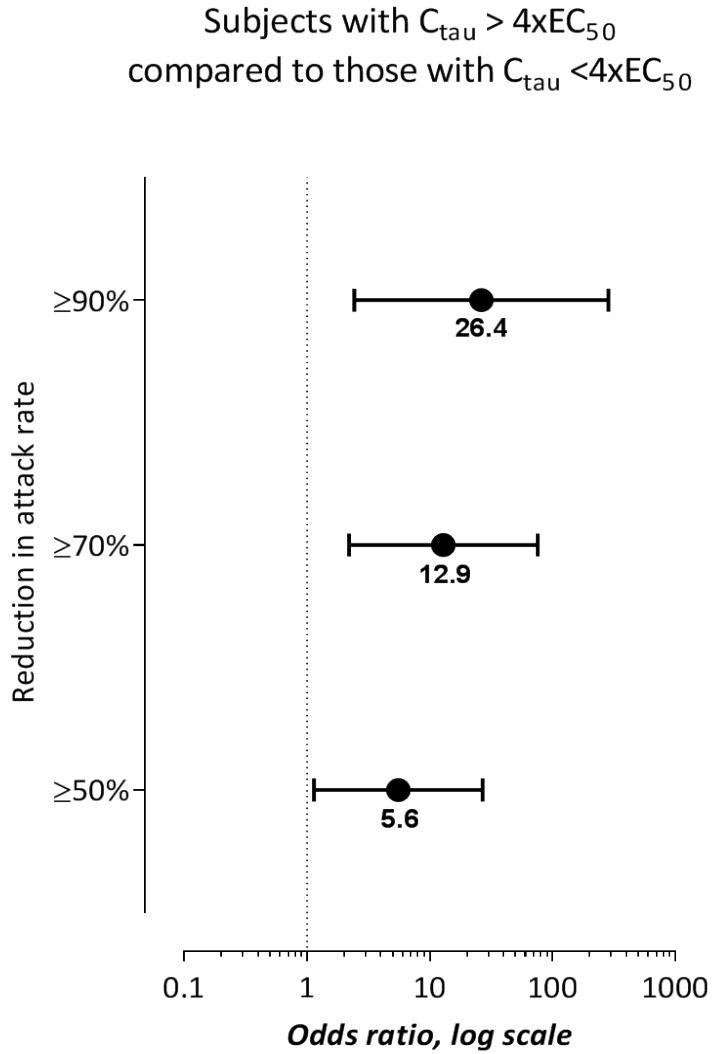
Exploratory dose-response and target-level response analyses for placebo, 62.5mg and 125mg dose levels





Left panel: Mean (SD) change in attack rate: on-study rate (ITT weeks 2-4) minus qualifying rate
Right panel: Odds ratio (95% CI) comparing proportion of subjects with indicated % response classified into those with trough drug level > 4xEC_{50} and < 4xEC_{50}. Placebo subjects included in < 4xEC_{50} group.
Ratio > 1 favors >4xEC_{50} group.

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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-1 - Conclusions and next steps

- Conclusion: APeX-1 results strongly support Phase 3 development
  - 125 mg dose level combines highly attractive attack frequency reductions of 73% ($p<0.001$) with a generally safe and well tolerated profile
  - PK, PD and lack of clinical benefit at 62.5 mg dose rounded out dose response
  - Exposures at 250 mg and 350 mg were not necessary to achieve efficacy and were associated with increased AE rates
  - 175 mg dose may get more patients above the target threshold
- Next Steps
  - Finalize the design of the Phase 3 and Long Term Safety trials after End of Phase 2 meeting with FDA and Scientific Advice procedure with EMA in Q4'17
  - Initiate Phase 3 and long term safety trial in Q1'18
  - Complete all other supporting activities for NDA and MAA filing (CMC, preclinical, clinical pharmacology, etc.)
  - Expand launch preparation activities over course of next year

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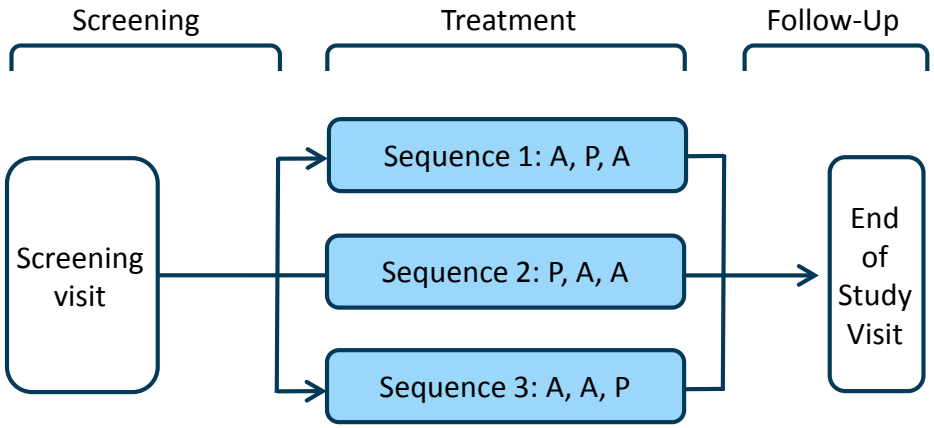
ZENITH-1 trial design (BCX7353 – Acute therapy)



Part 1: BCX7353 750 mg single doses n = 12 - 36	+	Part 2: BCX7353 500 mg single doses n = 12	+	Part 3: BCX7353 250 mg single doses 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)
- Subjects must have at least one attack per month for three months to qualify for the trial
- Primary efficacy endpoint: proportion of subjects with either improved or stable composite visual analog scale (VAS) score at 4 hours post-dose

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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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Cash position and 2017 guidance (in millions)

Cash & investments at June 30, 2017	$96
Pro forma 06/30/16 cash + net raise proceeds*	$181
Senior Credit Facility	$23

Guidance for 2017:

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

Excludes equity-based compensation.
*Range is based upon estimated Net Proceeds from $92 million raise completed in September 2017 (i.e., deducting all transaction costs). No additional cash inflows assumed.
@ 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