



Barclays Global Healthcare Conference

March 17, 2016

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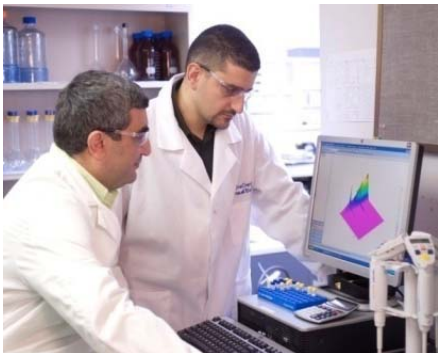


Forward-looking statement

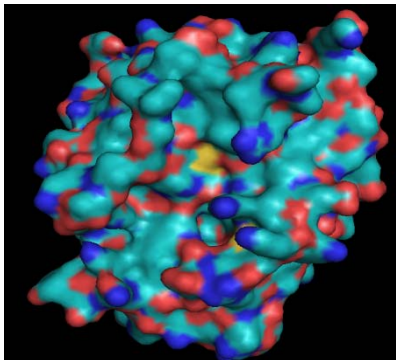
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BioCryst Strategy: Building a company focused on oral drugs for rare diseases

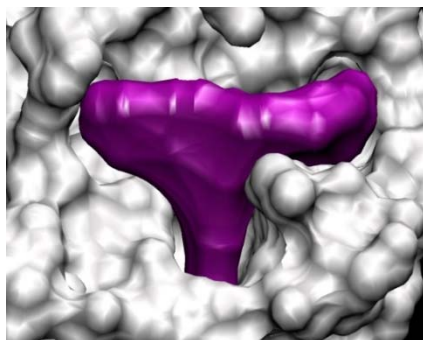
Experienced scientists



Structure-guided design



Validated targets



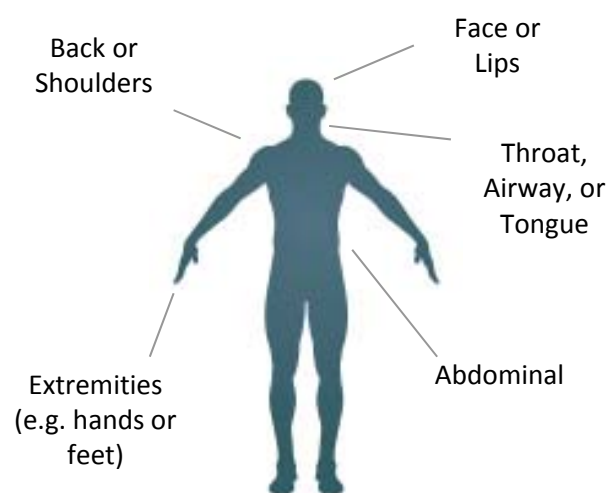
Value created by...

- ✓ Changing patients' lives
- ✓ Retaining full commercial rights
- ✓ Building sustainability

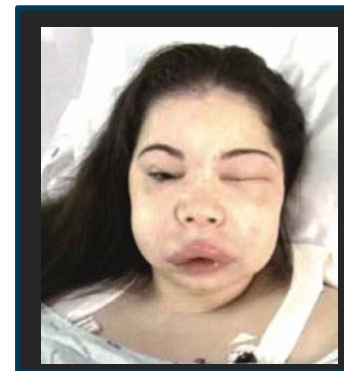
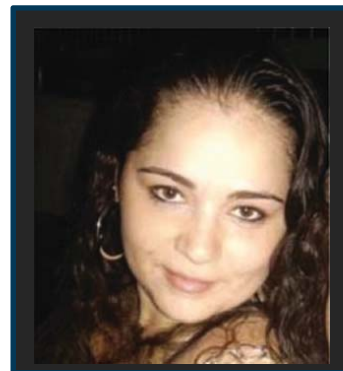
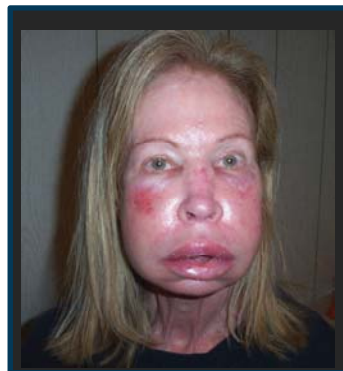
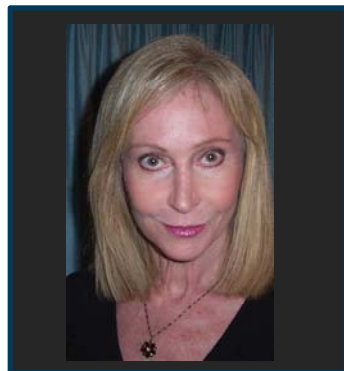
A maturing & growing pipeline

	Lead optimization	Pre-clinical	Ph 1	Ph 2	Ph 3	Filed	Approved
CORE STRATEGY							
Avoralstat liquid gel caps					Discontinued		
Avoralstat solid dosage (HAE)							
BCX7353 (HAE)							
Next generation kallikrein inhibitors							
Rare disease 1							
Rare disease 2							
NON DILUTIVE ASSETS							
RAPIVAB® (peramivir inj.)							
BCX4430 (broad spectrum antiviral)							

Unpredictability of HAE attack onset and severity drives need

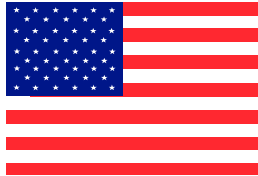


- Most patients have experienced **years of misdiagnosis or apathy** about their condition
- **Attacks are unpredictable**, regardless of underlying frequency
- **Any attack can cascade into a painful or dangerous event**, regardless of where it starts
- Nearly all patients have a **history of emergency treatment and/or hospitalization** for attacks
- Even **non-threatening attacks significantly disrupt** daily life



Images obtained from www.haeimages.com

HAE market is growing quickly with substantial upside



- **\$1.2B HAE Market**
- 30% annual growth



- **~\$100M market in 2015**
- Significant upside through better prophylactic options

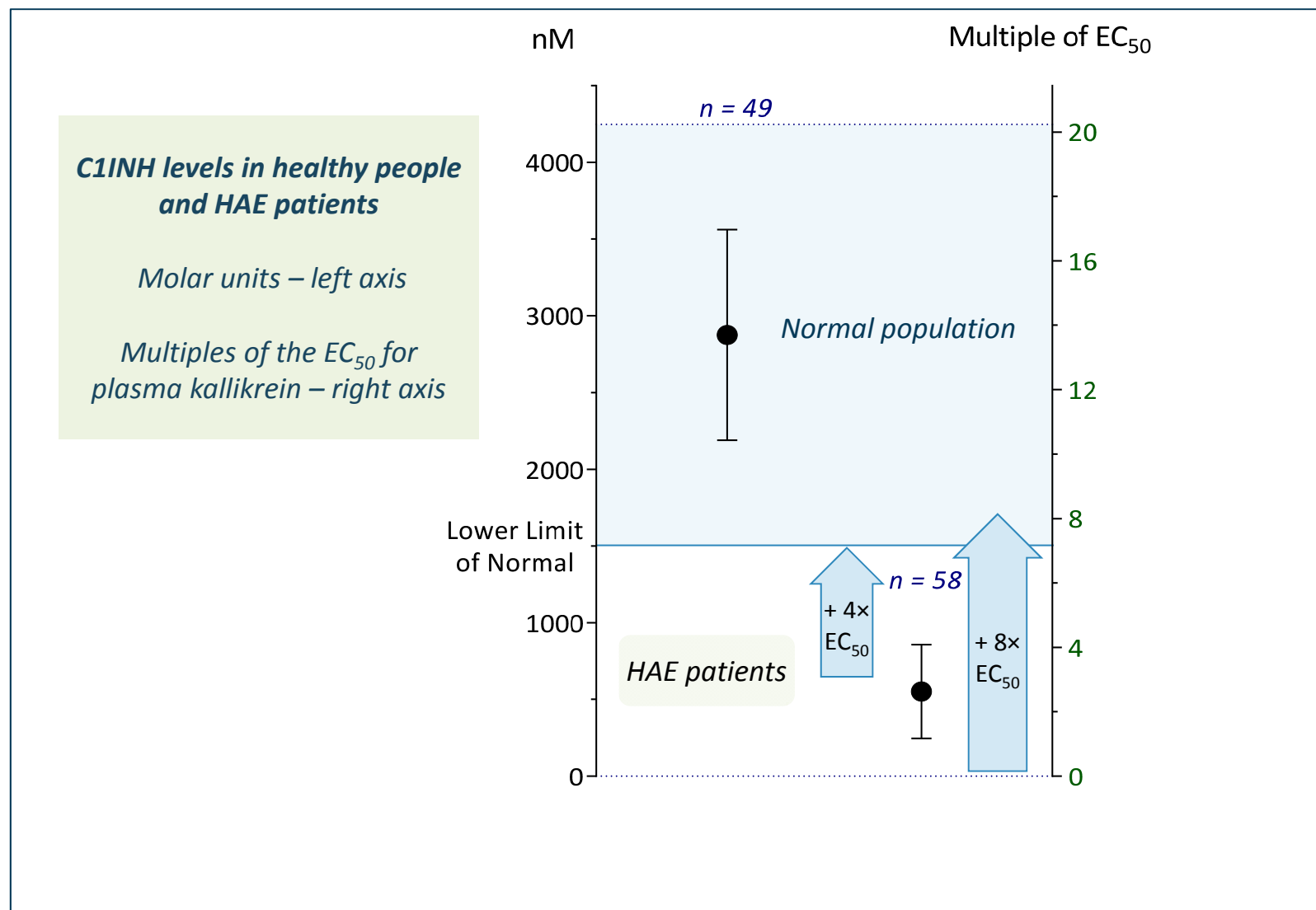


- **HAE significantly under-diagnosed** (~ 500 known patients out of estimated 3,000 prevalence)
- Opportunity for market expansion

HAE market in US alone will exceed \$2.0B by 2020 – Europe, Japan, and many other global markets provide long-term upside for oral prophylactic therapy

Source: Internal estimates based on analyst reports, earnings reports, and market data

Adding ~4 to ~8 times the EC_{50} of a kallikrein inhibitor should restore normal function in many ($4 \times EC_{50}$) to all ($8 \times EC_{50}$) patients with HAE



Tarzi, MD, et al. Clin Exp Immunol 2007; 149(3): 513-6
 Literature report of means and SD of C1INH in normal and patients with HAE
 (Lower Limit of Normal shown [LLN: mean – 2*SD])

Phase 1 first-in-human randomized double-blind evaluation of oral BCX7353 in Western and Japanese healthy volunteers

Single dose cohorts

Cohort	Dose, mg	N BCX7353	N placebo
1	10	6	2
2	30	4	2
3	100	6	2
4	250	6	2
	250 (fed)		
5	500	6	2
6	1000	6	2
7 (Japanese)	100	6	2
8 (Japanese)	500	6	2

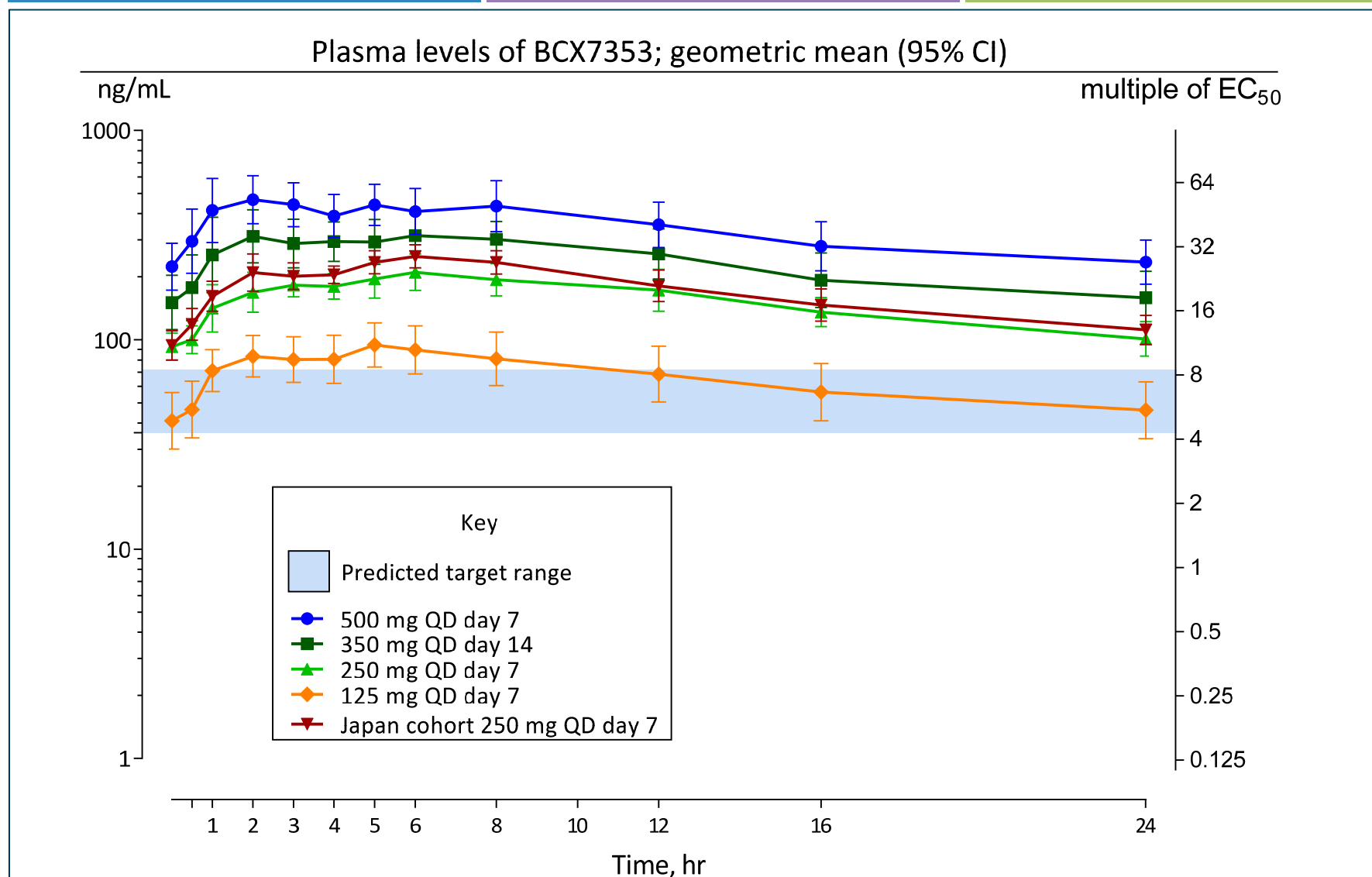
Multiple dose cohorts

Cohort	Dose, mg QD	Duration, days	N BCX7353	N placebo
1	125	7	10	2
2	250	7	10	2
3	500	7	10	2
4	350	14	10	2
5 (Japanese)	250	7	10	2

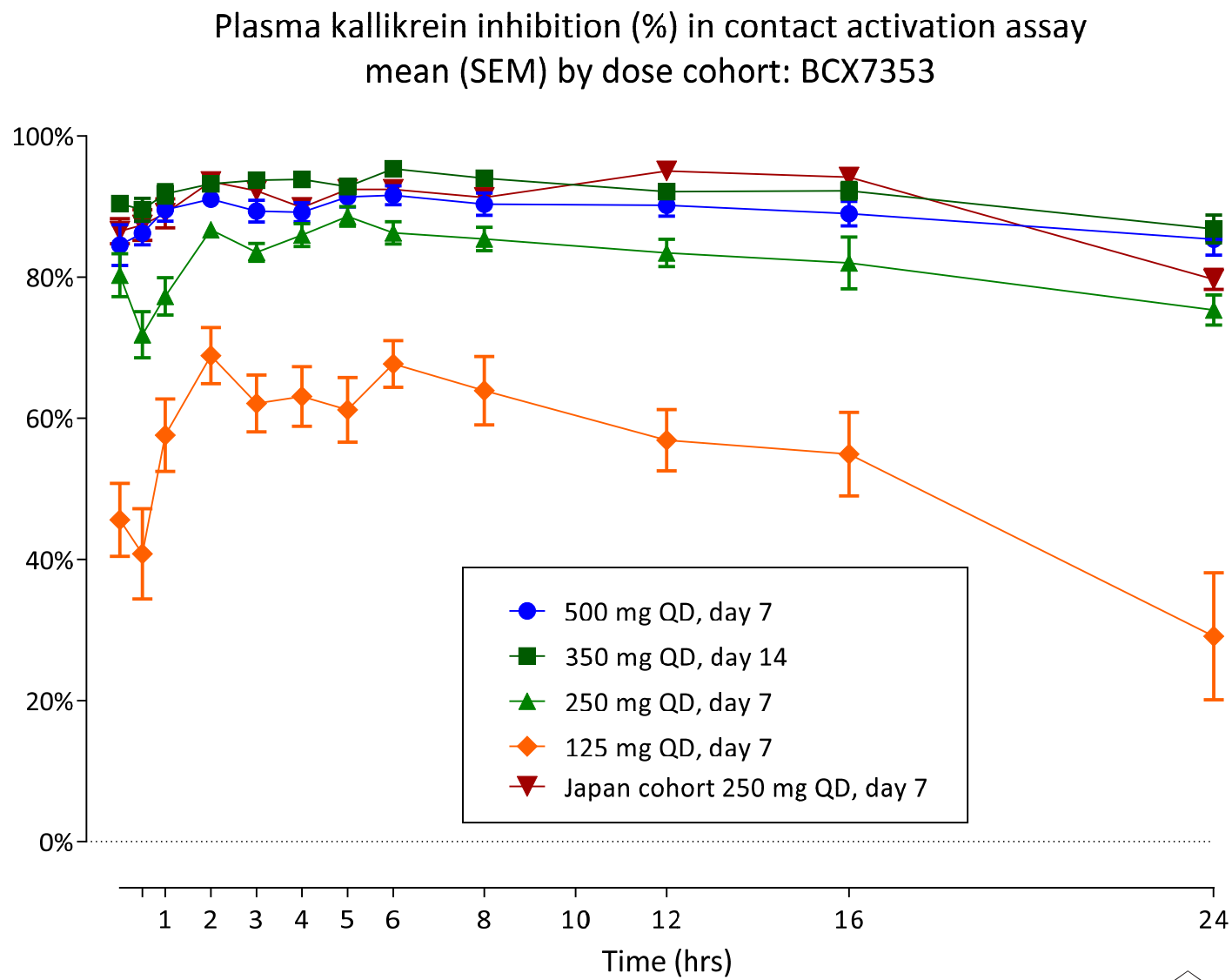
Evaluations

- Safety & tolerability: AEs, clinical and laboratory monitoring
- Pharmacokinetics: PK profile and PK parameters
- Pharmacodynamics: PD profile of inhibition of plasma kallikrein after oral dosing
- PK-PD correlations: E_{\max} model
- Modeling of efficacious dose range for HAE studies: population simulations of PK-PD dataset

BCX7353 PK profile supports once daily dosing and meets or exceeds therapeutic target levels



Daily dosing with BCX7353 achieves high levels of kallikrein inhibition in Western and Japanese healthy volunteers



BCX7353 was generally safe and well tolerated over the range of doses and durations tested in Western and Japanese subjects

Single doses of 10 mg through 1000 mg (total N = 46)

- No SAEs
- No clinically significant laboratory abnormalities
- 31 of 35 (89%) AEs were mild (grade 1)
- Four grade 2 events:
 - one Western subject in 100 mg cohort with moderate (grade 2) nausea and vomiting (2 AEs)
 - one Western subject in 100 mg cohort with moderate (grade 2) hay fever
 - one Japanese subject in 500 mg cohort with moderate (grade 2) self-limited diarrhea

Once daily doses of 125 mg, 250 mg and 500 mg for 7 days; 350 mg for 14 days (total N = 50)

- No SAEs
- No clinically significant laboratory abnormalities
- 63 of 70 (90%) AEs were mild (grade 1)
- Six grade 2 events and one grade 3 event:
 - 350 mg QD x 14d Western cohort: one subject grade 2 upper abdominal pain (discontinued from study)
 - 500 mg QD x 7d Western cohort: one subject grade 2 syncope, one subject grade 2 headache, one subject grade 2 diarrhea and upper abdominal pain (discontinued from study), one subject grade 3 skin hypersensitivity reaction
 - 250 mg QD x 7d Japanese cohort: one subject grade 2 maculopapular rash

BCX7353 clinical development path forward

APeX-1

- Ph 2 Trial in HAE
- Three doses and placebo
- Results year end 2016

Japan Study

- Trial in HAE
- Pivotal trial for Japanese filing

APeX-2

- Trial in HAE
- Pivotal trial for US and EU filing

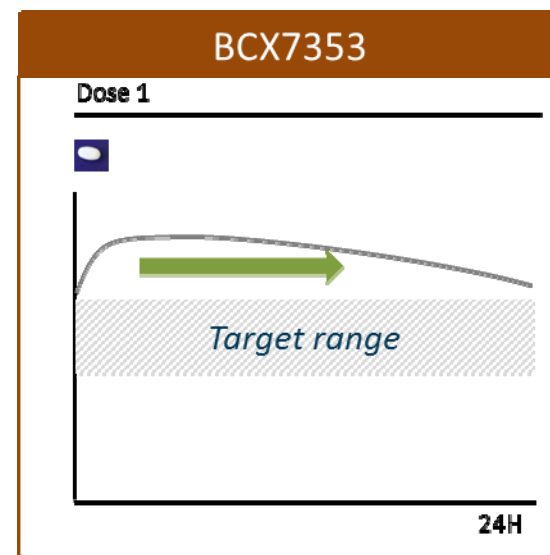
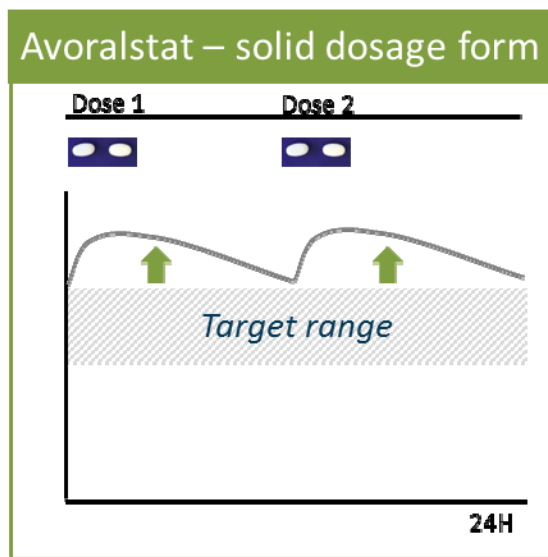
APeX-3

- Long-term safety
- Goals:
 - 6 to 12 mo dosing with BCX7353
 - Satisfy filing requirements

Our goal: Bring to market a conveniently dosed, highly effective oral treatment to prevent HAE attacks

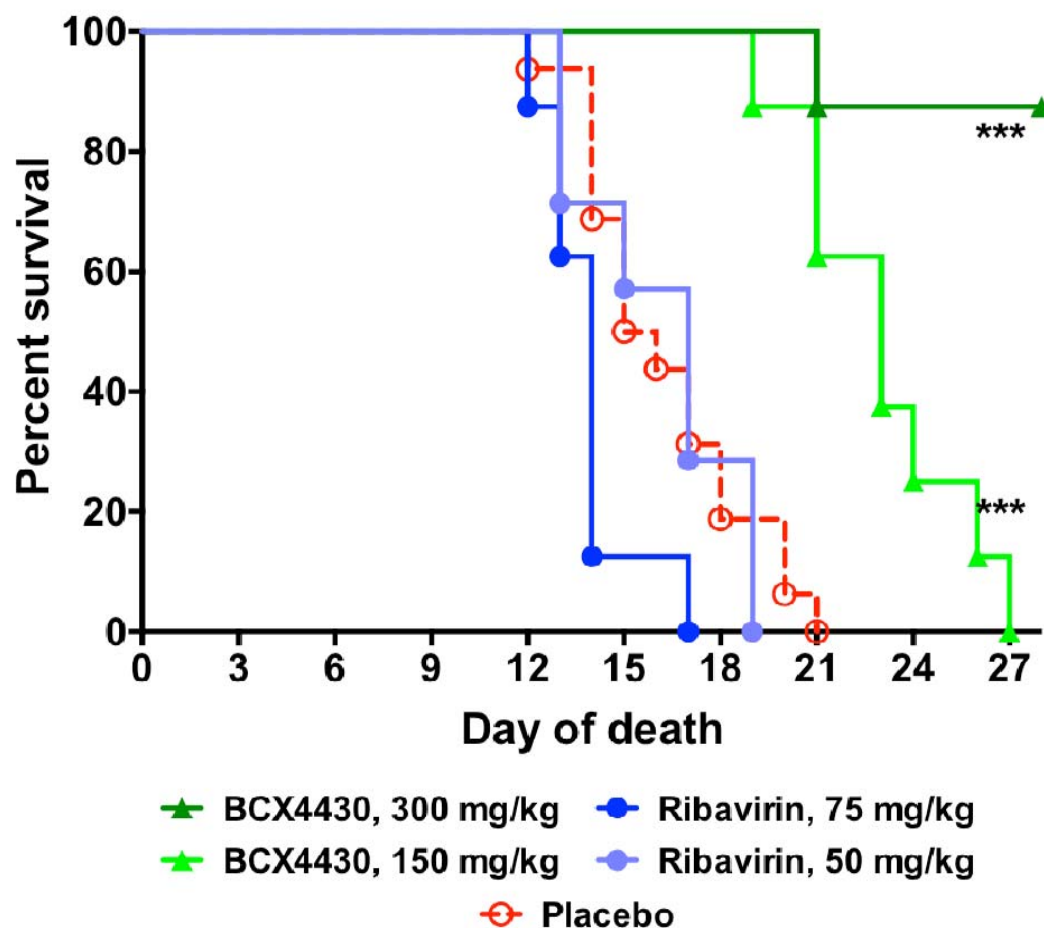
Avoralstat solid dose form

BCX7353



We have two shots at achieving our goal

BCX4430 is active against Zika infection in immune deficient mice



BCX4430 has also shown a survival benefit in animals against Ebola, Marburg & Yellow Fever infections

*** ($p < 0.001$)

Animal data courtesy of Dr. Justin Julander, Utah State University and NIAID

Cash position & 2016 guidance (in millions)

Cash & investments at December 31, 2015	\$100.9
Gross operating cash utilization 2015	\$42.2
Operating cash utilization 2015 with RAPIVAB [®]	\$13.1

2016 Guidance

Operating cash utilization	\$55 – 75
Operating expenses [#]	\$78 – 98
Cash runway	Mid-2017

[#] Excludes equity-based compensation.

Key HAE Program Milestones

Avoralstat

- Complete avoralstat solid dose form PK study for twice daily dosing and report results (mid-2016)

BCX7353

- Report BCX7353 APeX-1 clinical trial results (year end 2016)