15th Annual Needham Healthcare Conference

April 12, 2016

Jon Stonehouse, *President & Chief Executive Officer*

Dr. Bill Sheridan, *Chief Medical Officer*

Robert Bennett, *Vice President, Investor Relations & Operations*

BioCryst Pharmaceuticals, Inc.

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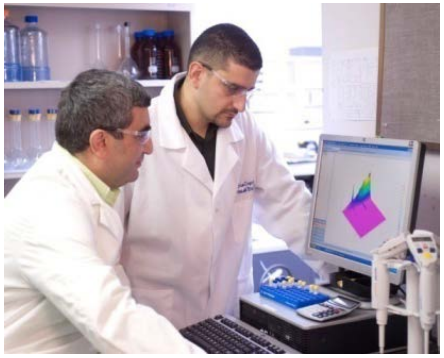
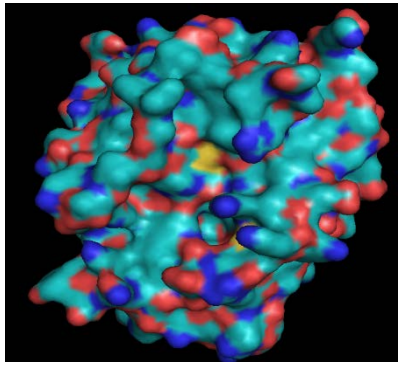
BIOCRYST PHARMACEUTICALS, INC.

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

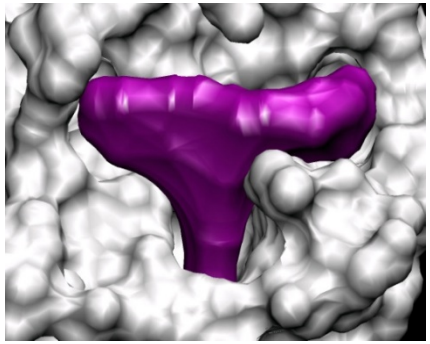
Experienced scientists



Structure-guided design



Validated targets



Value created by…

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

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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)							

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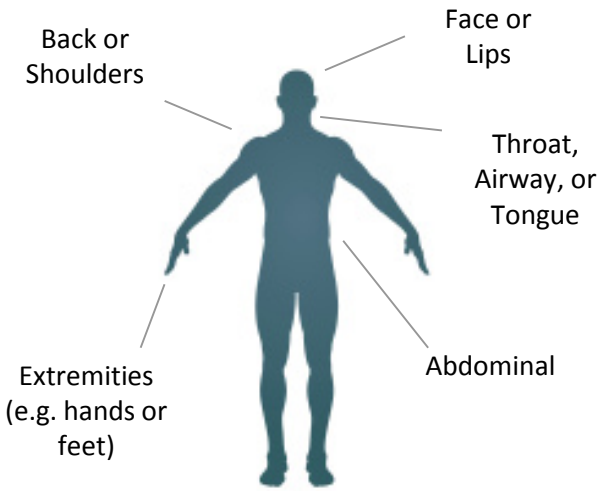
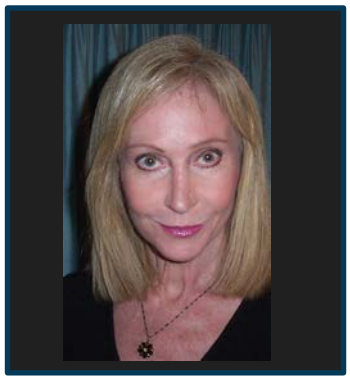
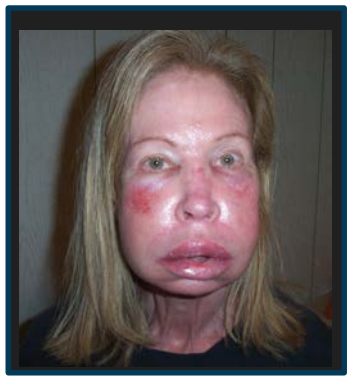
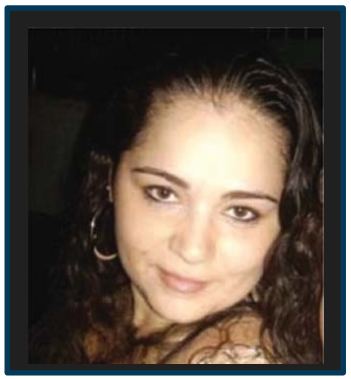
Unpredictability of HAE attack onset and severity drives need



- 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 <u>significantly</u> disrupt** daily life


Images obtained from www.haeimages.com

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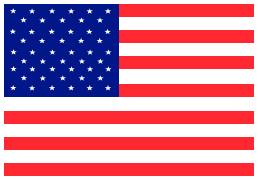
HAE market is growing quickly with substantial upside



- **$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

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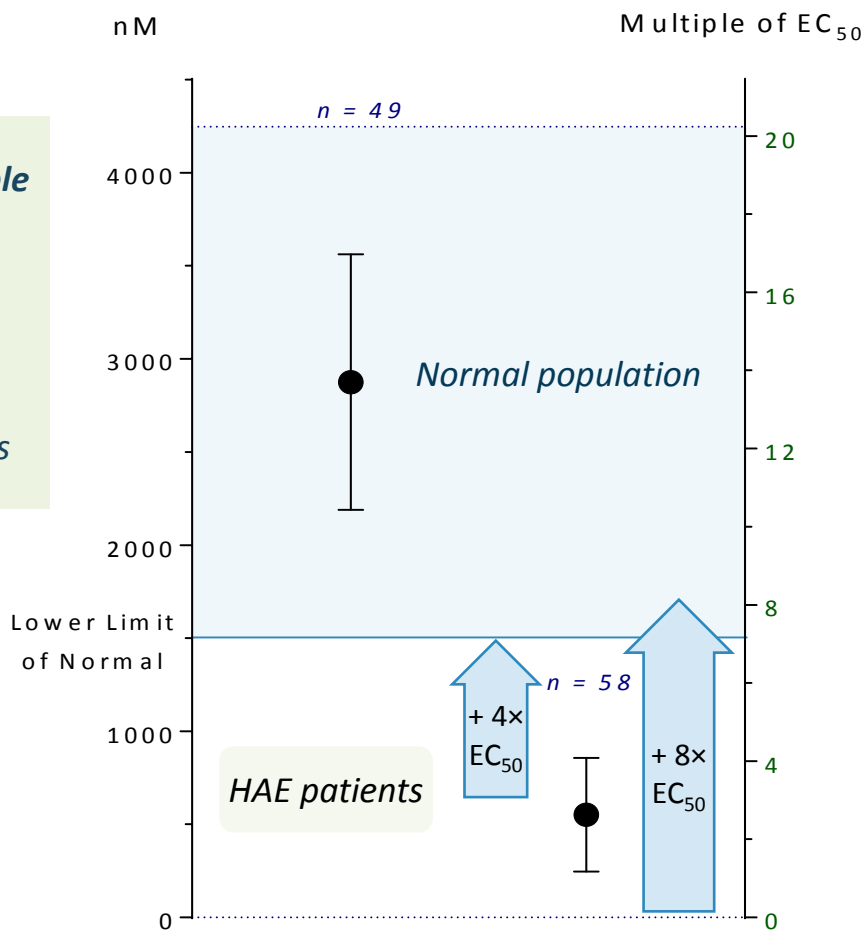
Adding ~4 to ~8 times the EC_{50} of a kallikrein inhibitor should restore normal function in many ($4 \times EC_{50}$) to all ($8 \times EC_{50}$) patients with HAE

C1INH levels in healthy people and HAE patients

Molar units – left axis

Multiples of the EC_{50} for plasma kallikrein – right axis



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])

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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

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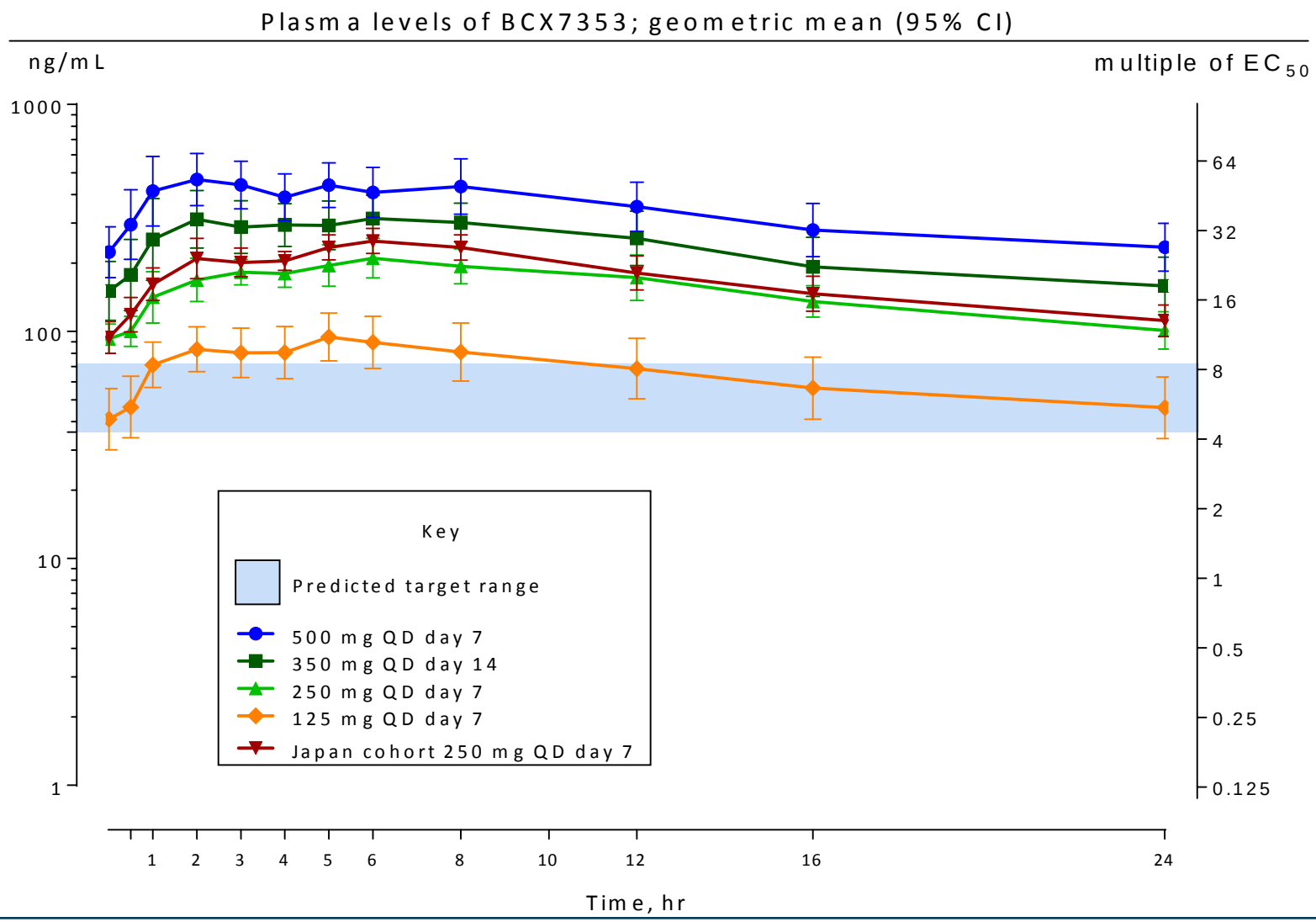
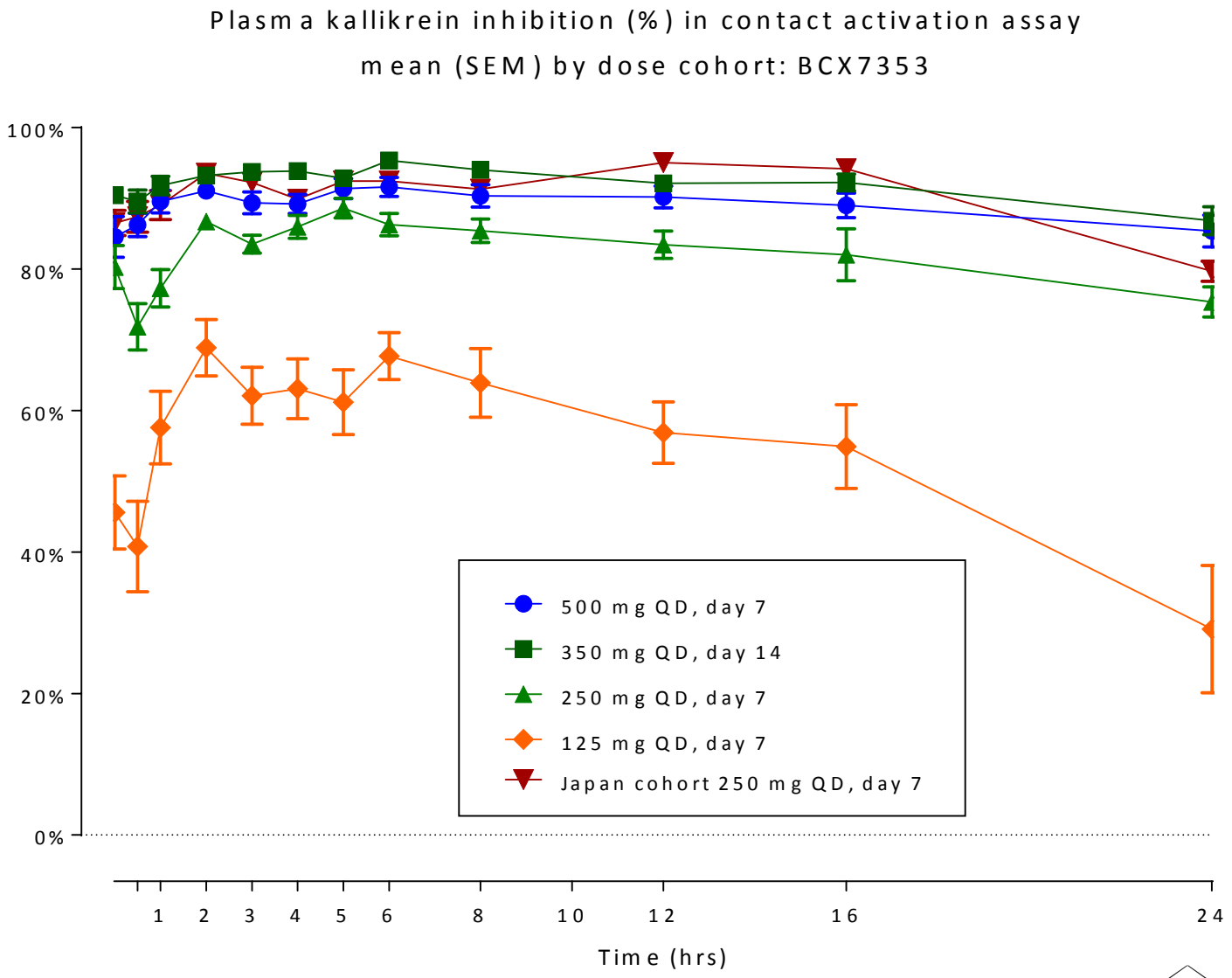
BCX7353 PK profile supports once daily dosing and meets or exceeds therapeutic target levels



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



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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

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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

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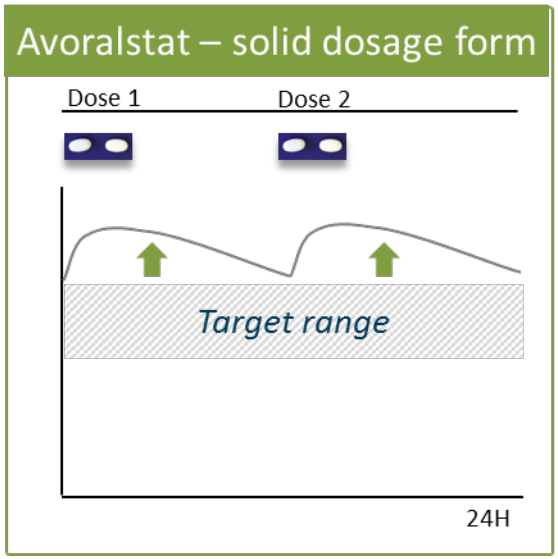
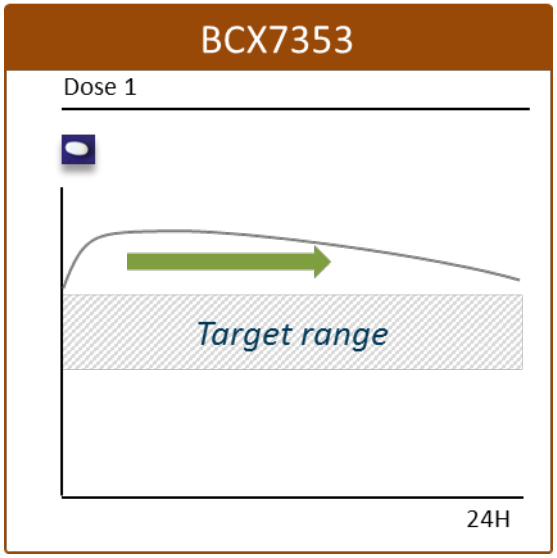
Our goal: Bring to market a conveniently dosed, highly effective oral treatment to prevent HAE attacks

Avoralstat solid dose form

BCX7353





We have two shots at achieving our goal

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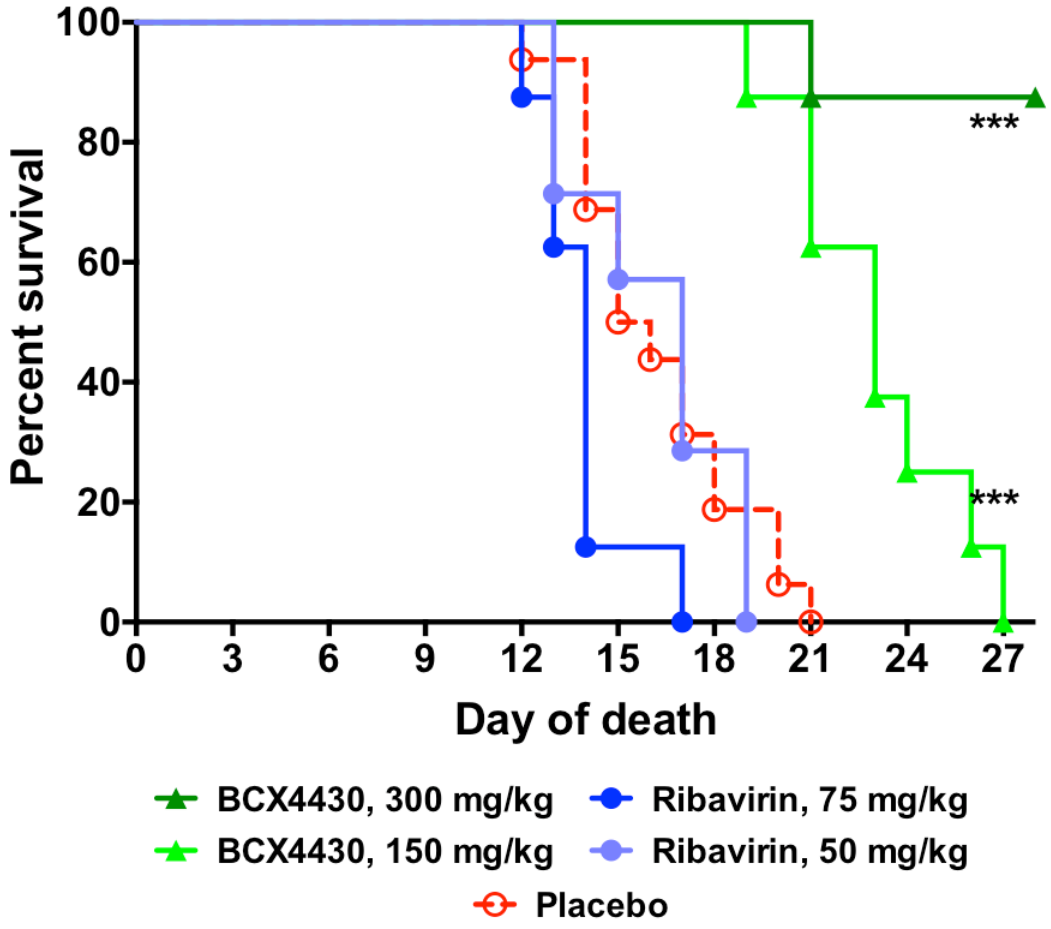
BCX4430 is active against Zika infection in immune deficient mice



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

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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.

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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)

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