Baird 2016 Global Health Care Conference

September 8th, 2016

Jon Stonehouse, *President & Chief Executive Officer*

Bill Sheridan, *Chief Medical Officer*

Rob Bennett, *V.P. Investor Relations & Operations*

BioCryst Pharmaceuticals, Inc.

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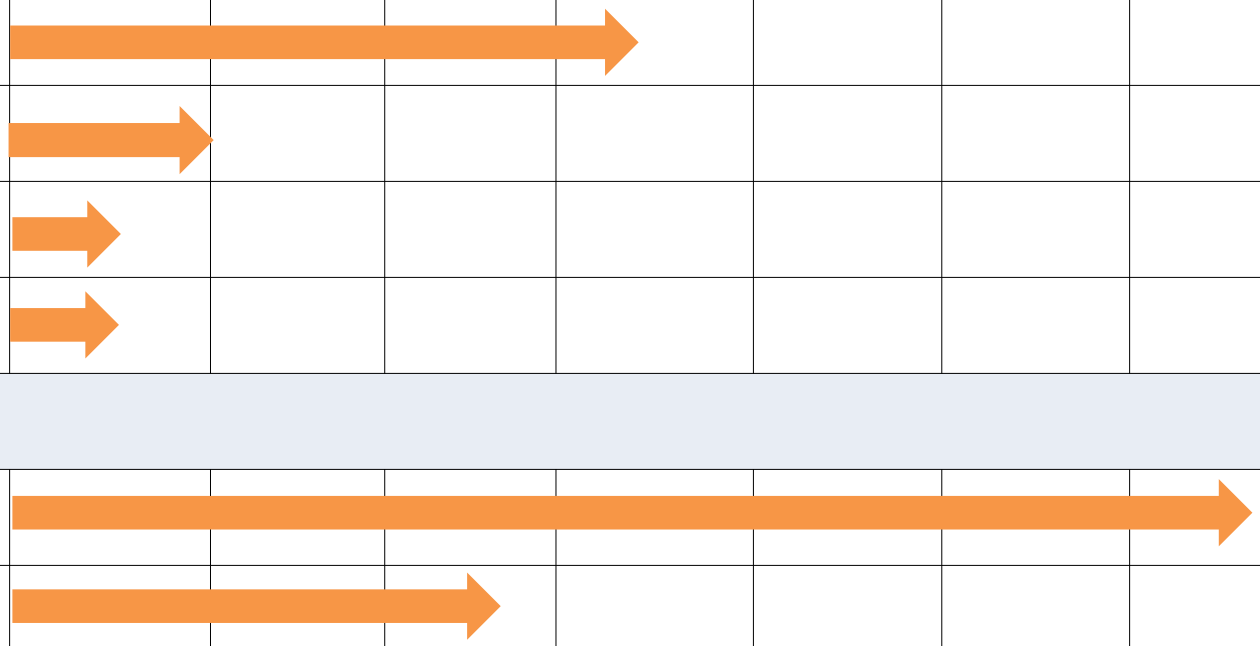
A maturing & focused pipeline

	Lead optimization	Pre-clinical	Ph 1	Ph 2	Ph 3	Filed	Approved
CORE STRATEGY							
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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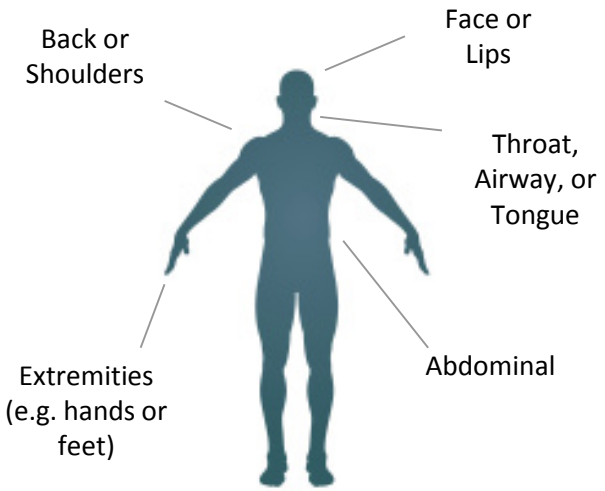
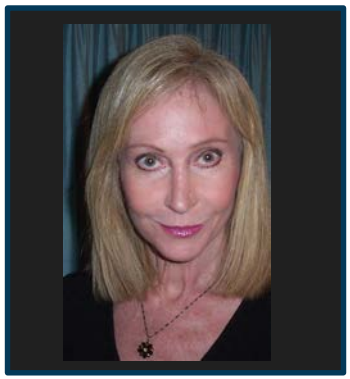
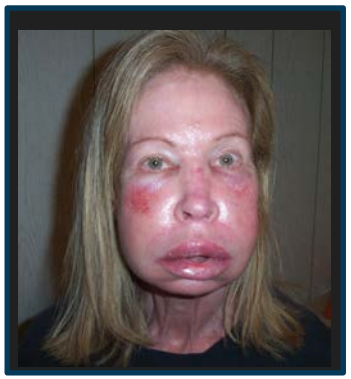
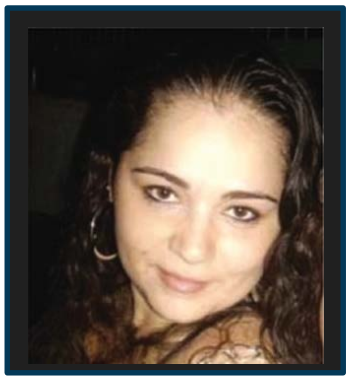
Unpredictability of HAE attack onset and severity drives need



- 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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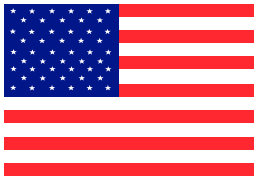
HAE market is growing quickly with substantial upside



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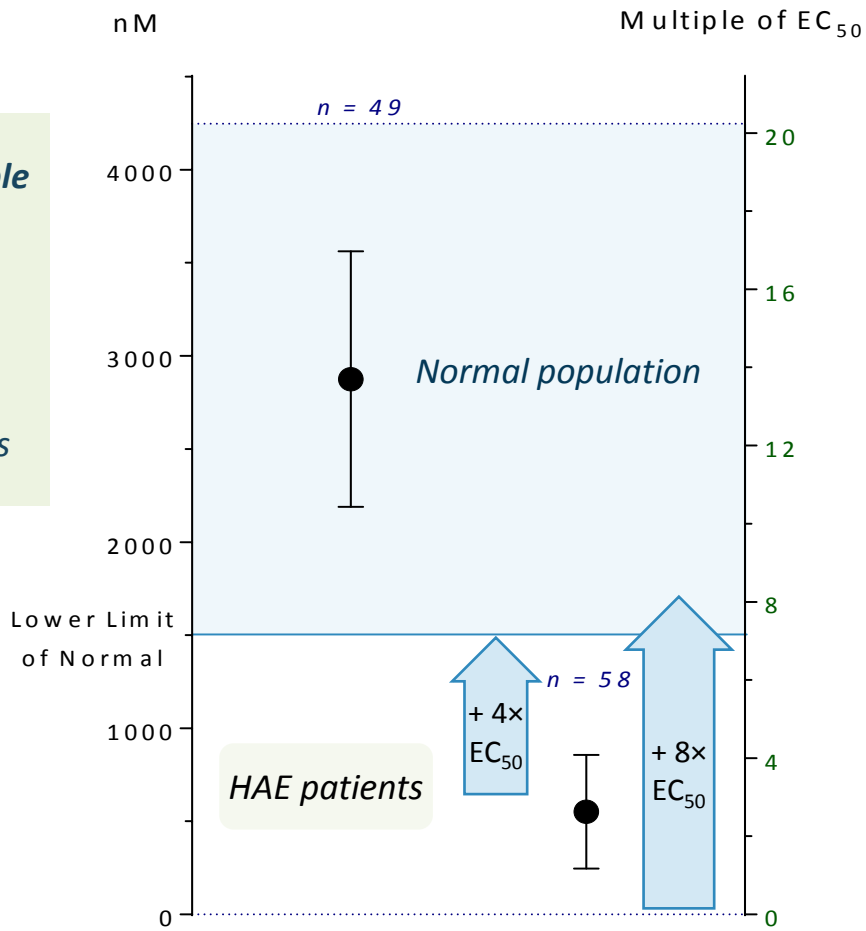
Adding ~4 to ~8 times the EC_{50} of a kallikrein inhibitor should restore normal function in many ($4\times EC_{50}$) to all ($8\times EC_{50}$) patients with HAE

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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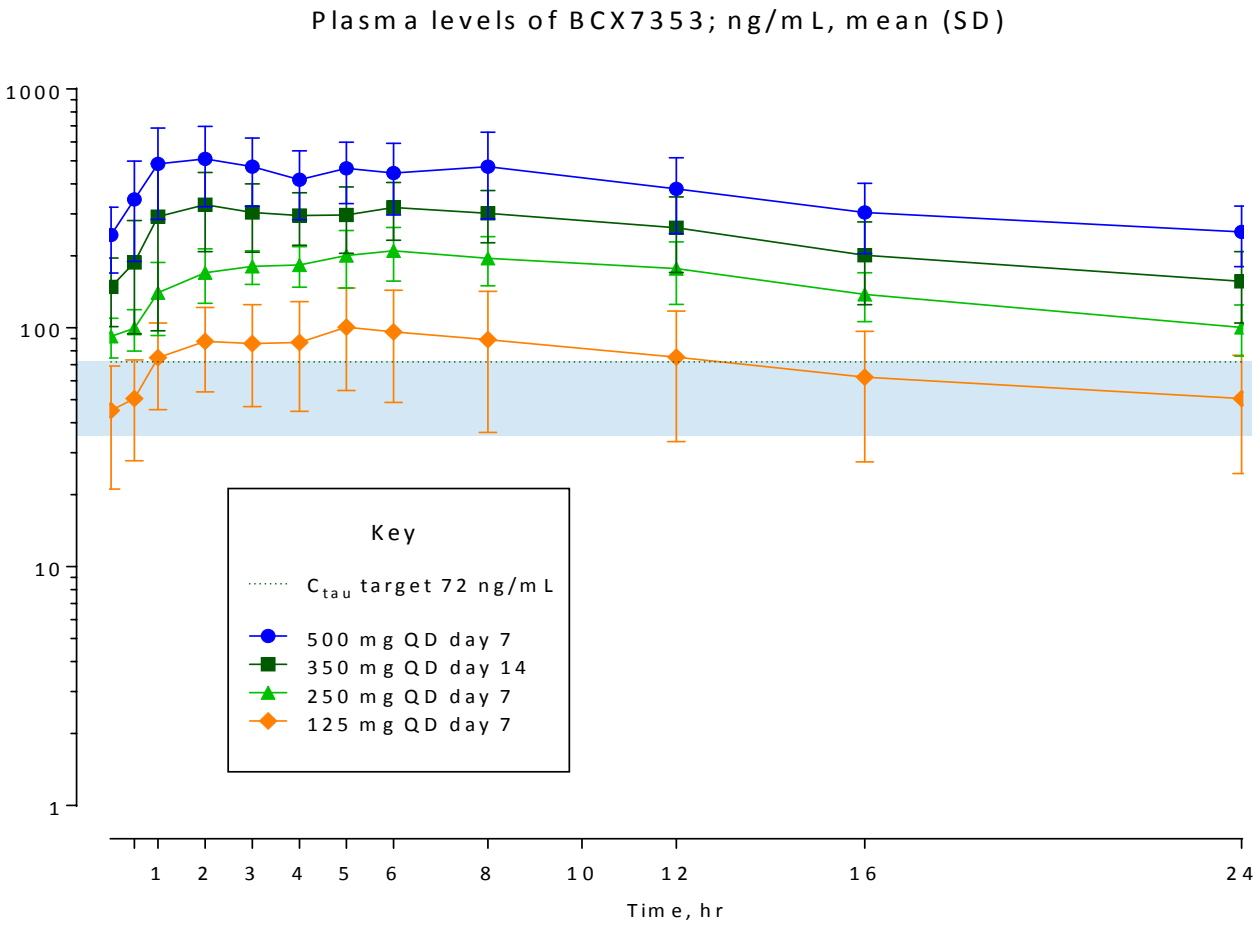
PK profile of BCX7353 dosed once daily in healthy subjects



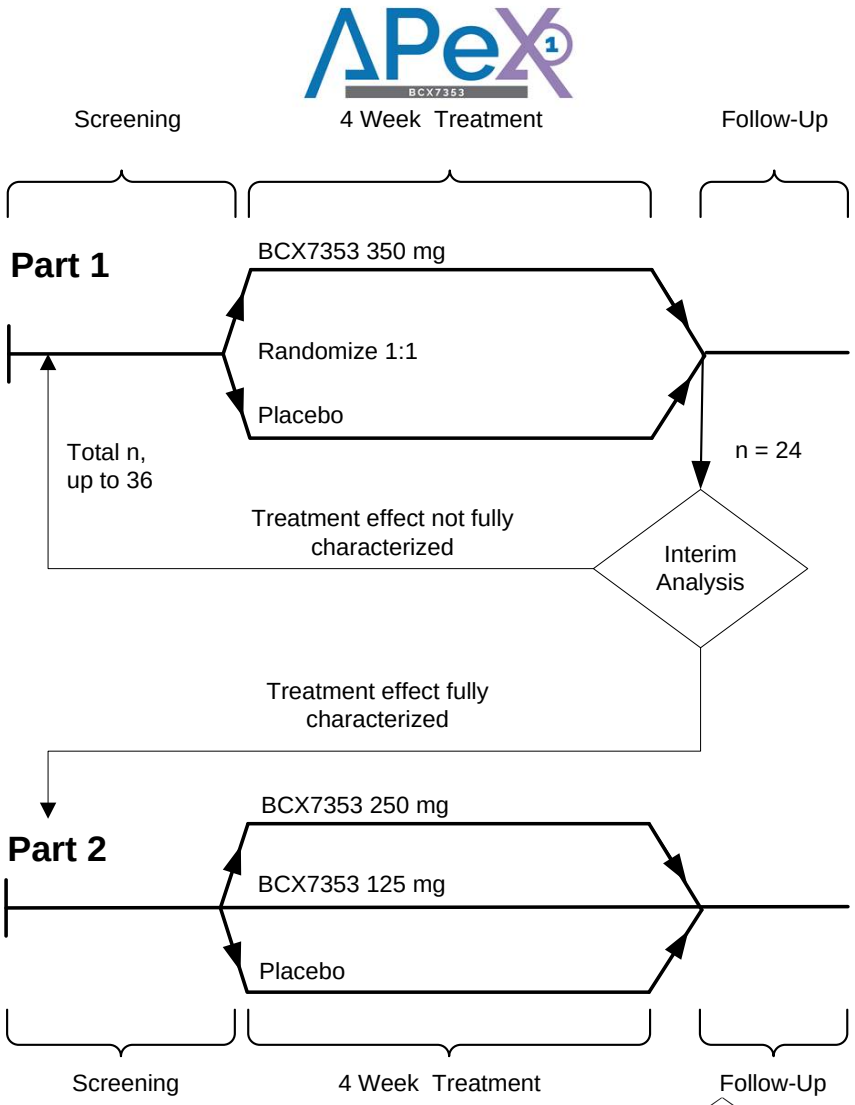
APeX-1 underway: Phase 2 placebo-controlled trial of BCX7353 in HAE patients

Design

- Part 1: proof of concept
  - 350 mg QD BCX7353 vs placebo
  - Interim analysis at n = 24
  - Option to add up to 12 subjects for total n = 36
  - Powered at 90% (α=0.05) to detect a reduction in number of HAE attacks of ≥ 70% on BCX7353
- Part 2: dose ranging
  - 250 mg QD and 125 mg QD BCX7353 and placebo
  - n = 14
  - 6:6:2 randomization

Endpoints

- Number of HAE attacks by treatment group will be analyzed as weekly attack rate, number of attacks, proportion of subjects with no attacks, number of attack-free days
- Additional endpoints include full safety assessments, QOL, PK/PD



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First-in-human phase 1 clinical study of broad-spectrum antiviral nucleoside analog BCX4430, administered by intramuscular (i.m.) injection

SAD Cohort	Dose, mg/kg	Number of Subjects
1	0.3	6 active, 2 placebo
2	0.75	6 active, 2 placebo
3	1.8	6 active, 2 placebo
4	4	6 active, 2 placebo
5	7	6 active, 2 placebo
6	10	6 active, 2 placebo
Lidocaine evaluation	4	14 active

MAD Cohort	Dose, mg/kg QD for 7 days	Number of Subjects
1	2.5	7 active, 2 placebo
2	5	8 active, 2 placebo
3	10	8 active, 2 placebo

- Study BCX4430-101 evaluated the safety, tolerability, and pharmacokinetics of 1 dose and 7 days of daily dosing by i.m. injection in 91 healthy volunteers
- All planned cohorts were completed
- Effect of adding lidocaine (local anesthetic) to i.m. injections was also evaluated

Clinical study (ClinicalTrials.gov identifier: NCT02319772) funded with Federal funds under NIH contract HHSN272201300017C

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BCX4430 administered by i.m. injection was generally safe and well tolerated over the range of doses and durations tested

Single doses of 0.3 mg/kg through 10 mg/kg

- 50 subjects received single doses of BCX4430 (12 subjects received placebo)
- No serious or severe adverse events occurred
- The most frequently reported AE across all cohorts was injection site pain: 23 subjects (46%)
- No clinically significant laboratory abnormalities occurred at any dose
- Co-administration of lidocaine with BCX4430 was found to ameliorate injection site pain, without altering the plasma PK profile of BCX4430

Once daily doses of 2.5 mg/kg through 10 mg/kg for 7 days

- 23 subjects received daily doses of BCX4430 with lidocaine (6 subjects received placebo)
- All subjects except 1 completed planned dosing through 7 days (one subject developed gastroenteritis unrelated to study drug)
- No serious or severe adverse events occurred
- The most frequently reported AE across all cohorts was injection site pain: 5 subjects (22%)
- No clinically significant laboratory abnormalities occurred at any dose
- With co-administration of lidocaine, the injections were well tolerated

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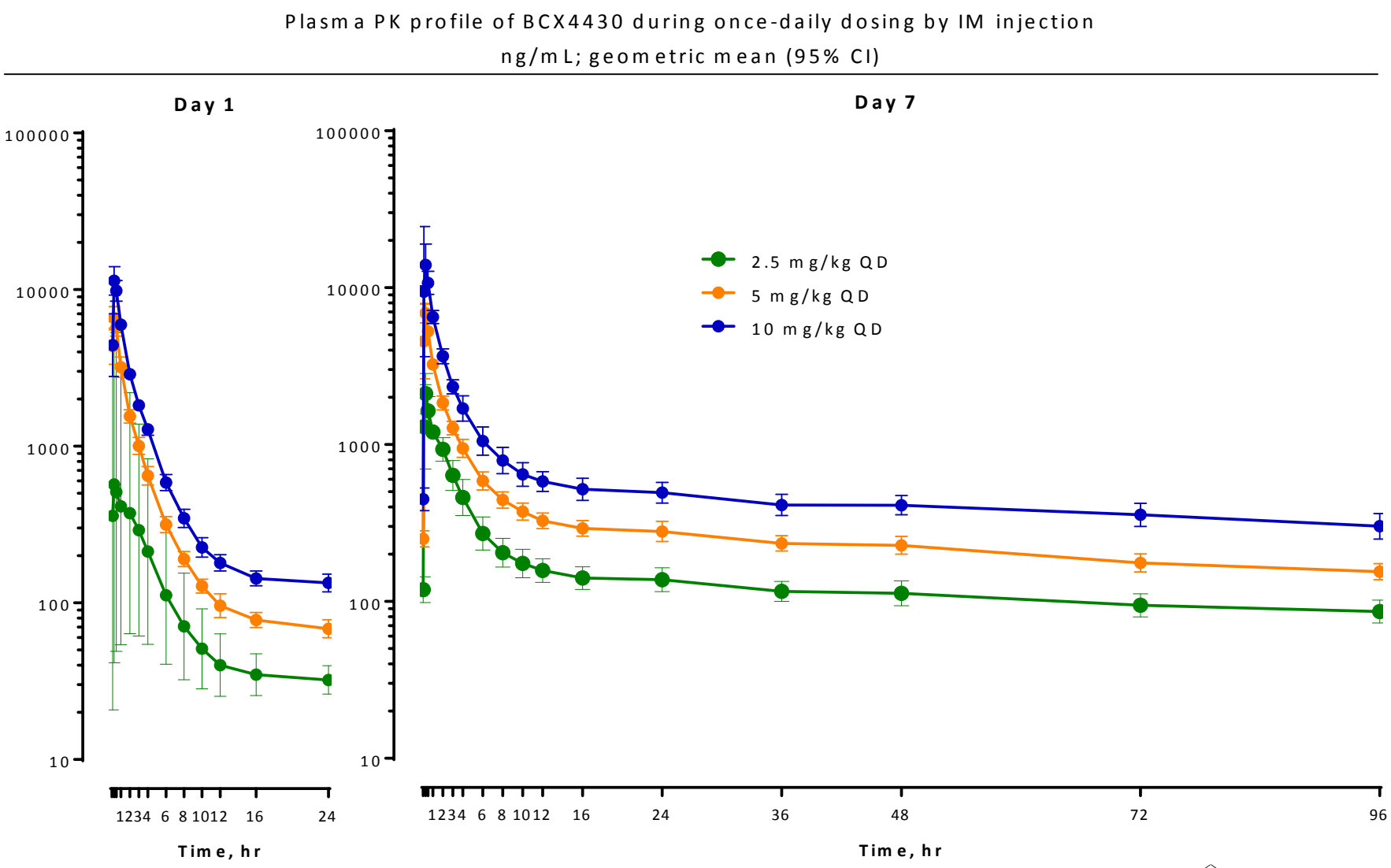
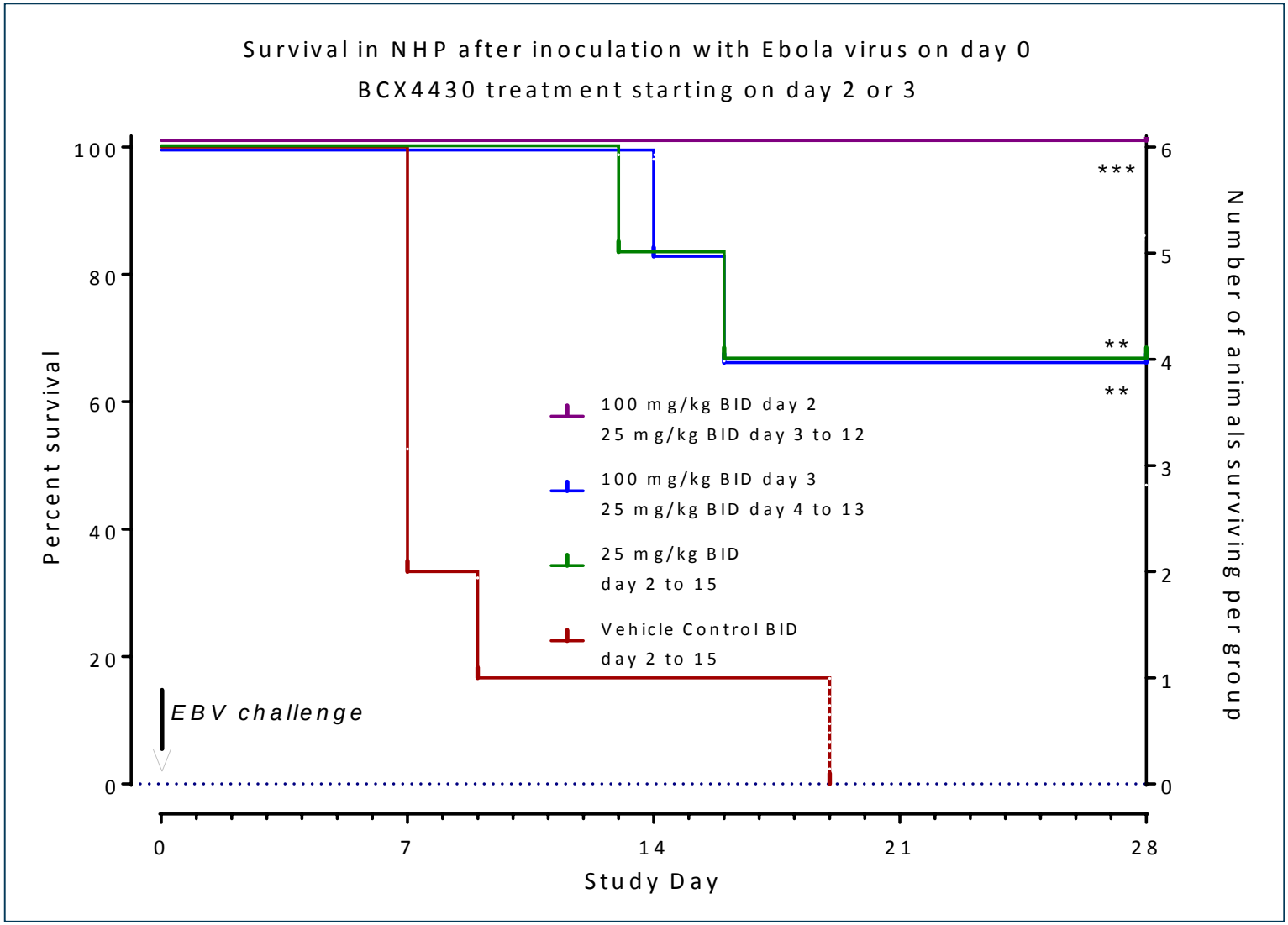
Plasma concentration-time profile of BCX4430 on the first and last day of dosing by daily intramuscular injection



Phase 1 study of BCX4430 administered via IM injection in healthy volunteers: conclusions

- The study achieved all of its objectives
- BCX4430 was generally safe and well tolerated at doses up to 10 mg/kg once daily for 7 days
- Exposure was dose-proportional
- These results support the continued development of BCX4430 as a parenterally administered broad-spectrum antiviral drug for the treatment of serious emerging viral infections

Nonclinical study results for BCX4430 in NHP model of Ebola virus disease



*** $p < 0.001$ ** $p = 0.005$

Study conducted at USAMRIID and funded by NIH/NIAID under contract HHSN272201300017C

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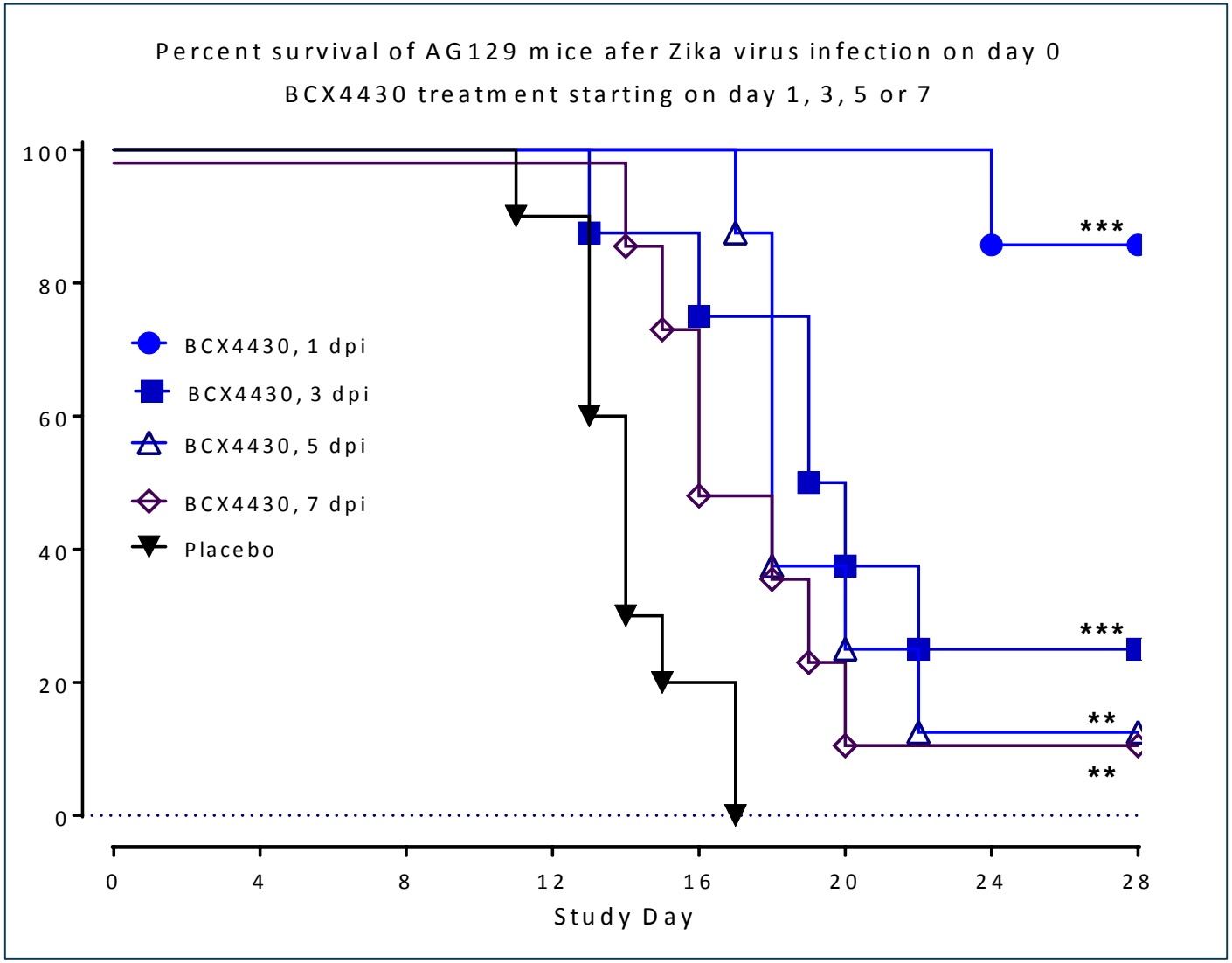
BCX4430 significantly improves survival of immune-deficient mice when treatment is delayed after Zika virus infection



*** $p < 0.001$ ** $p < 0.01$

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
Operating cash utilization through June 30, 2016	($37.9)
Cash & investments at June 30, 2016	$64.3
2016 Guidance	
Operating cash utilization	$55 – 75
Operating expenses[#]	$78 – 98
Cash runway	Mid-2017

Excludes equity-based compensation.

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