



Jefferies Health Care Conference

June 9th, 2016

Jon Stonehouse, *President & Chief Executive Officer*

Bill Sheridan, *Chief Medical Officer*

Tom Staab, *Chief Financial Officer*

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

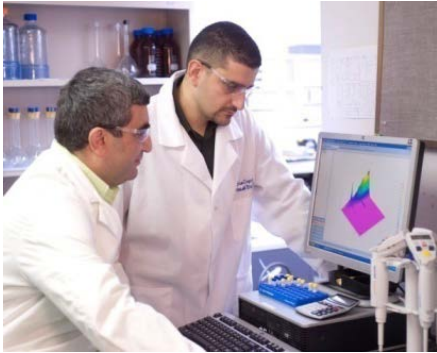


Forward-looking statement

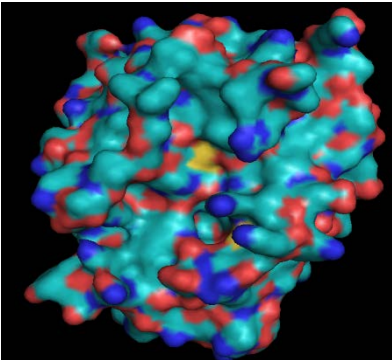
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BioCryst Strategy: Building a company focused on oral drugs for rare diseases

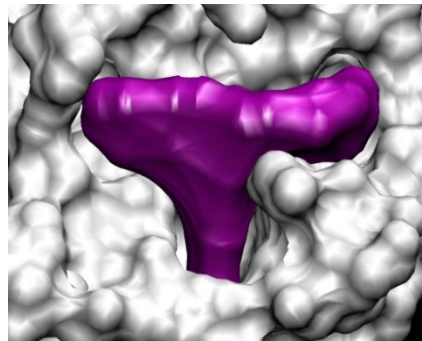
Experienced scientists



Structure-guided design



Validated targets



Value created by...

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

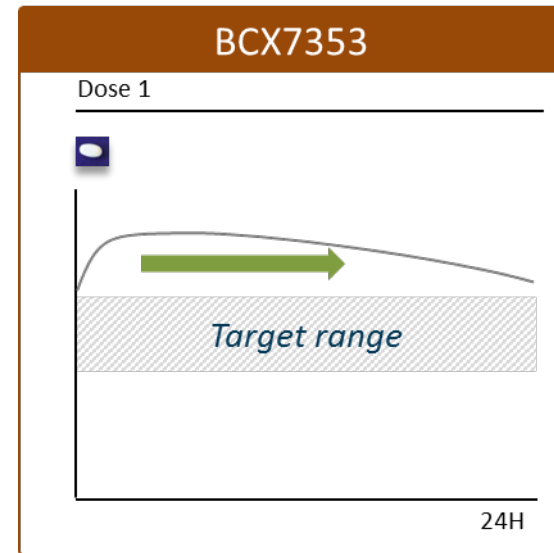
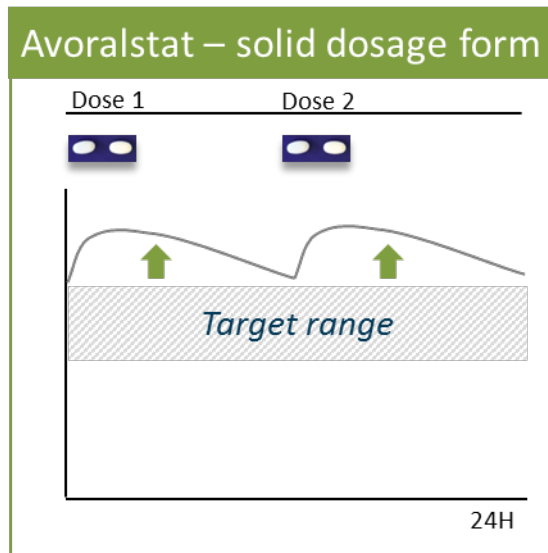
A maturing & growing pipeline

	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)							

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

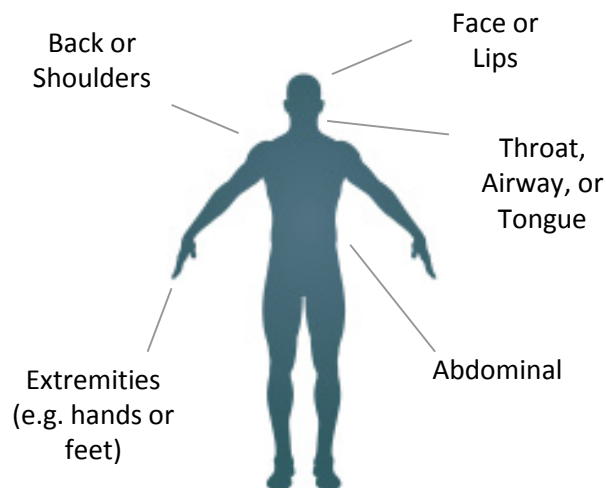
Avoralstat solid dose form

BCX7353

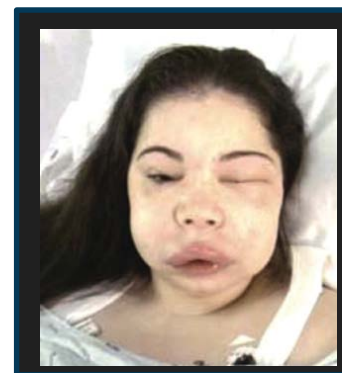
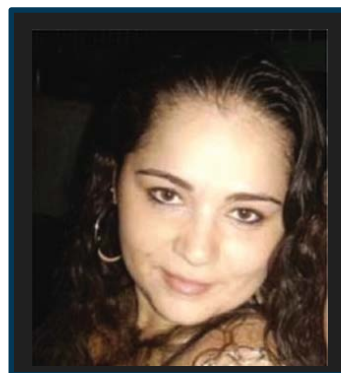
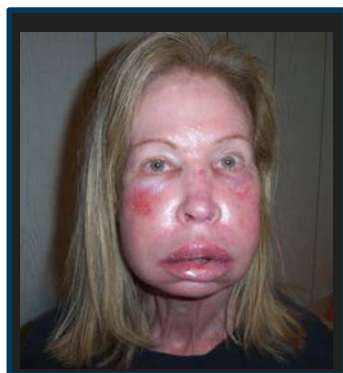
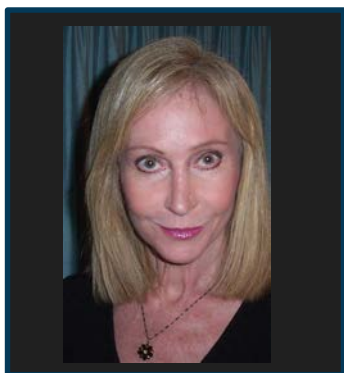


We have two shots at achieving our goal

Unpredictability of HAE attack onset and severity drives need

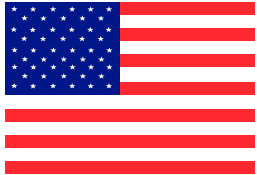


- Most patients have experienced **years of misdiagnosis or apathy** about their condition
- **Attacks are unpredictable**, regardless of underlying frequency
- **Any attack can cascade into a painful or dangerous event**, regardless of where it starts
- Nearly all patients have a **history of emergency 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

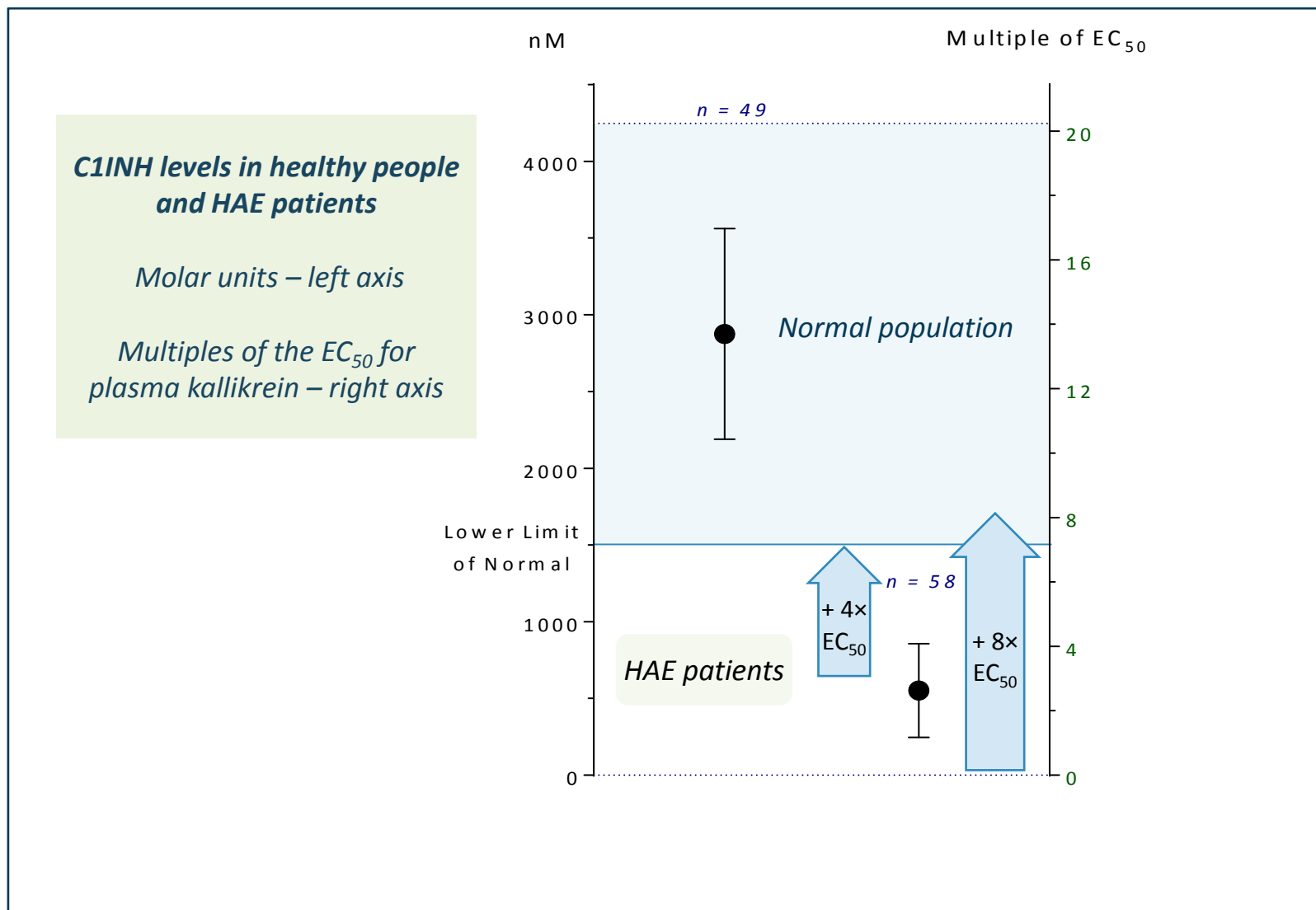


- **HAE significantly under-diagnosed** (~ 500 known patients out of estimated 3,000 prevalence)
- Opportunity for market expansion

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

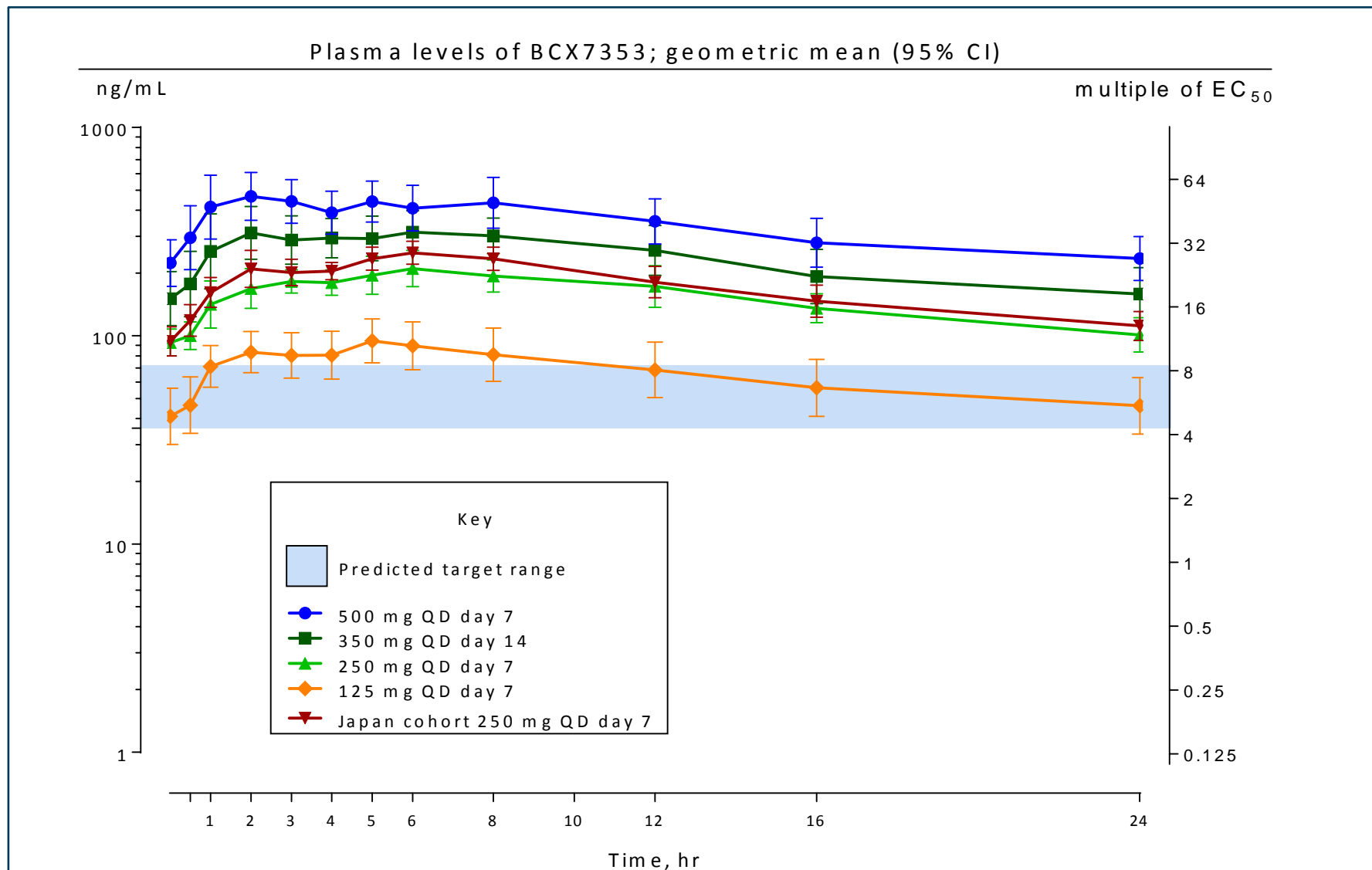
Source: Internal estimates based on analyst reports, earnings reports, and market data

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

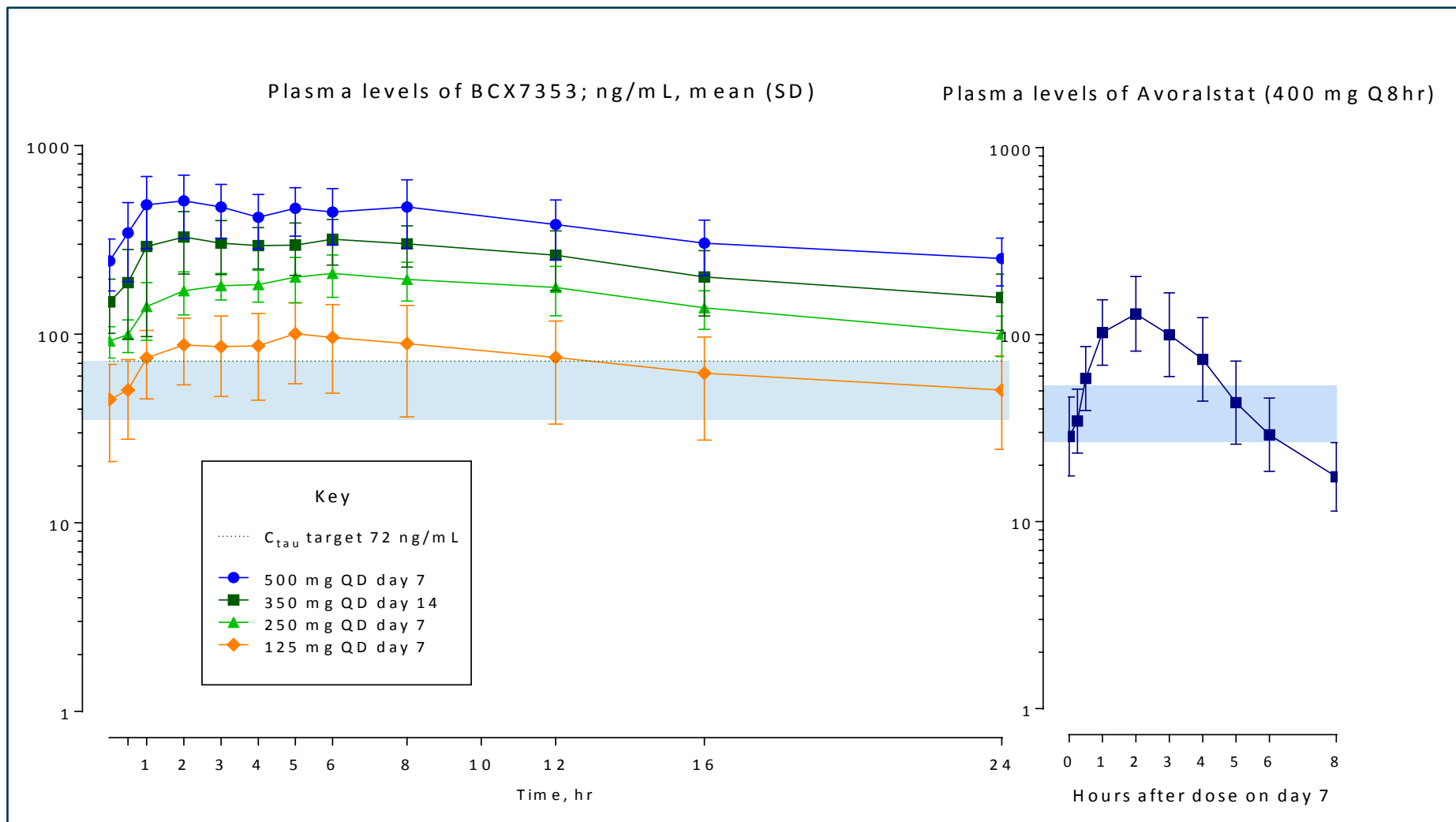


Tarzi, MD, et al. Clin Exp Immunol 2007; 149(3): 513-6
Literature report of means and SD of C1INH in normal and patients with HAE
(Lower Limit of Normal shown [LLN: mean – 2*SD])

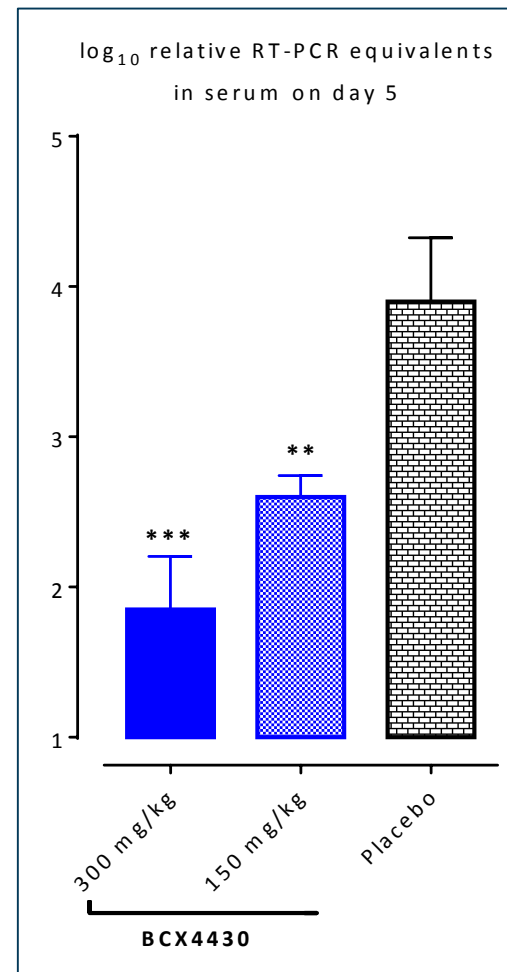
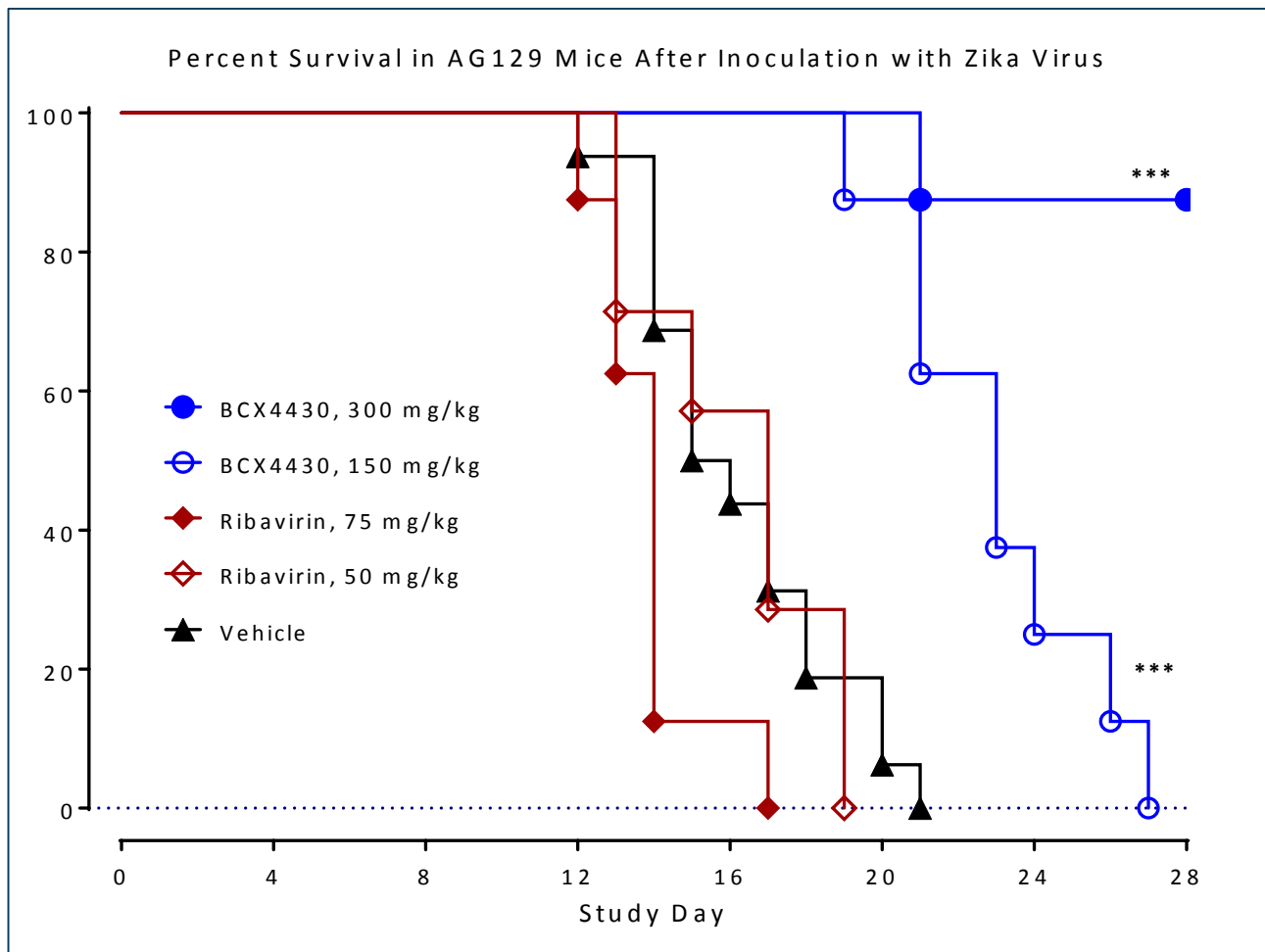
BCX7353 PK profile supports once daily dosing and meets or exceeds therapeutic target levels



PK profile: Multiple days of BCX7353 & avoralstat dosed once daily in healthy subjects – last day of dosing



BCX4430 is active against Zika virus infection in immune-deficient mice

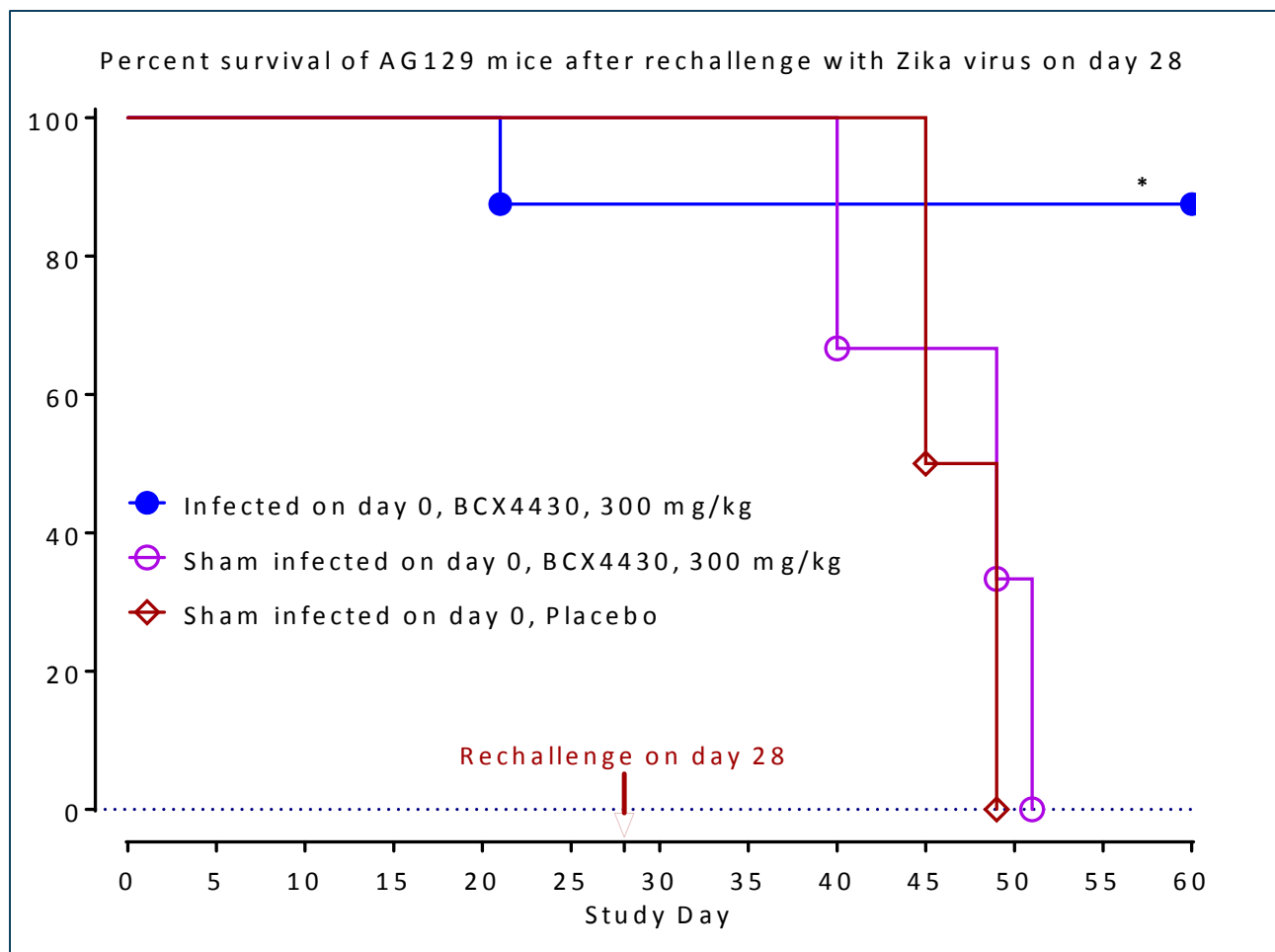


BCX4430 has also shown a survival benefit in nonclinical studies of Ebola virus, Marburg virus & Yellow Fever virus infections

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

Data courtesy of Dr. Justin Julander, Utah State University and NIAID
Presented to WHO March 2016

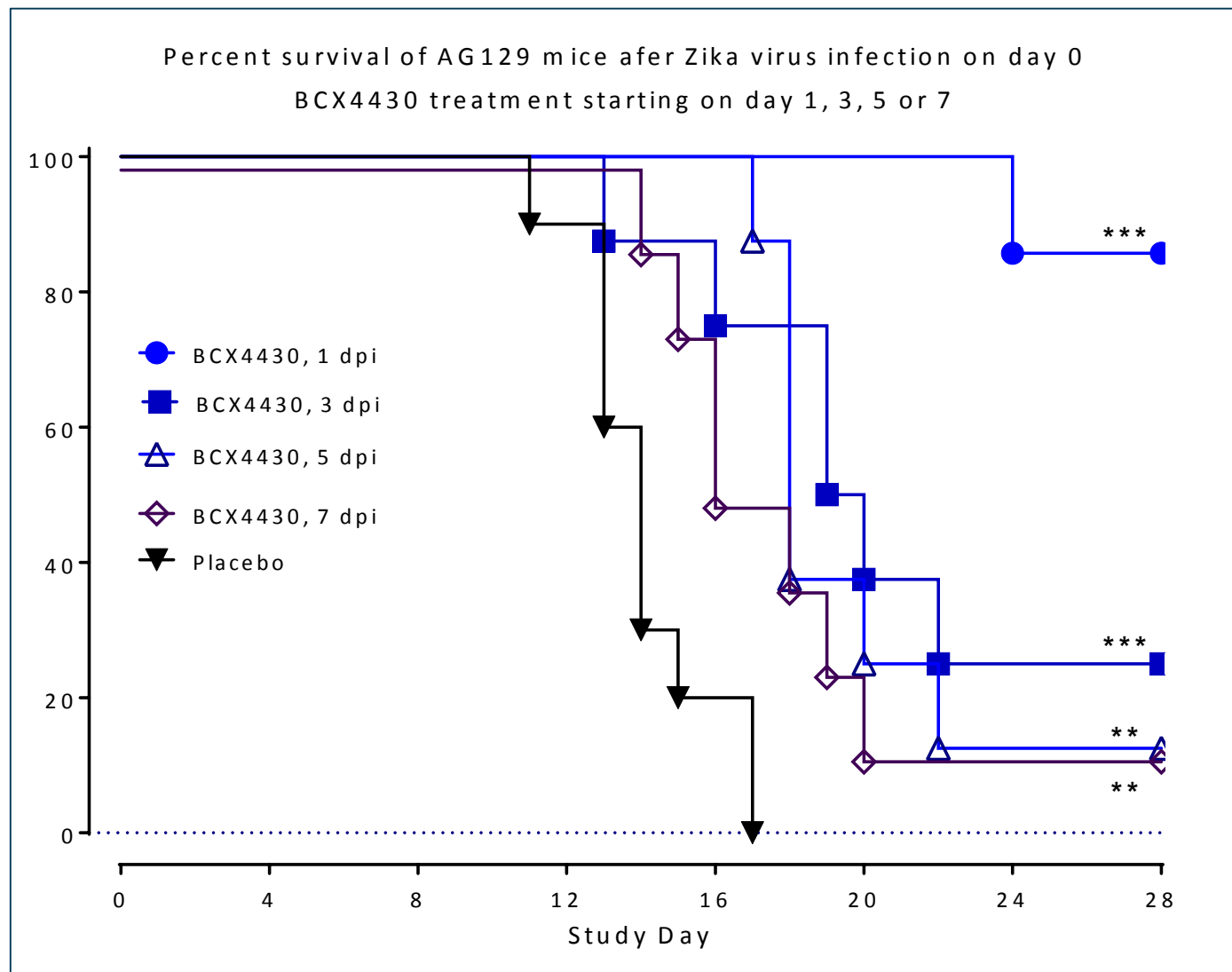
AG129 mice treated with BCX4430 and surviving after initial infection are immune to re-challenge with Zika virus



* $p < 0.05$

Data courtesy of Dr. Justin Julander, Utah State University and NIAID
Presented at ICAR conference San Diego April 2016

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

Cash position & 2016 guidance (in millions)

Cash & investments at December 31, 2015	\$100.9
Gross operating cash utilization 1Q16	\$22.4
Cash & investments at March 31, 2016	\$78.9

2016 Guidance

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

[#] Excludes equity-based compensation.

Key HAE Program 2016 Milestones

Avoralstat

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

BCX7353

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