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# Bank of America Merrill Lynch Health Care Conference

May 11<sup>th</sup>, 2016

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*Jon Stonehouse, President & Chief Executive Officer*



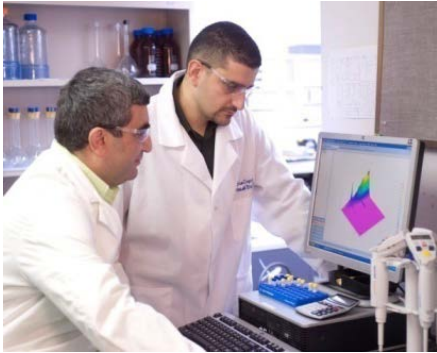
# Forward-looking statement

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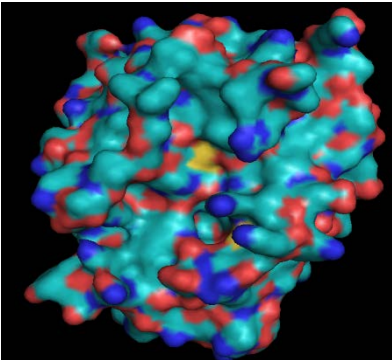
BioCryst's presentation may contain forward-looking statements, including statements regarding future results, unaudited and forward-looking financial information and company performance or achievements. These statements are subject to known and unknown risks and uncertainties which may cause our actual results, performance or achievements to be materially different from any future results or performances expressed or implied in this presentation. You should not place undue reliance on the forward-looking statements. For additional information, including important risk factors, please refer to BioCryst's documents filed with the SEC and located at <http://investor.shareholder.com/biocryst/sec.cfm>

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

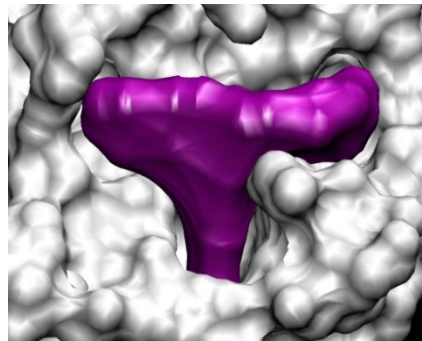
Experienced scientists



Structure-guided design



Validated targets



Value created by...

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

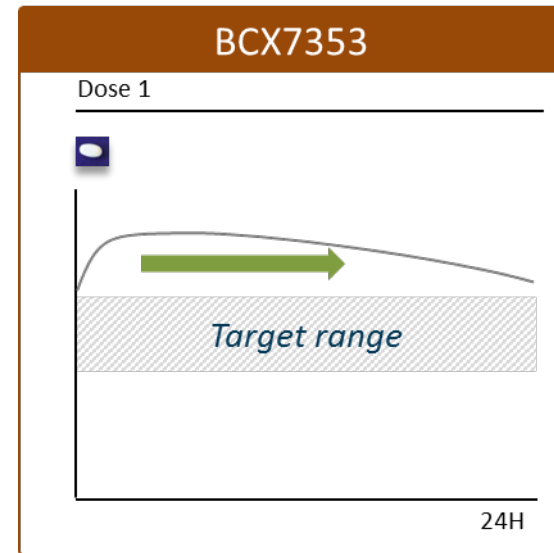
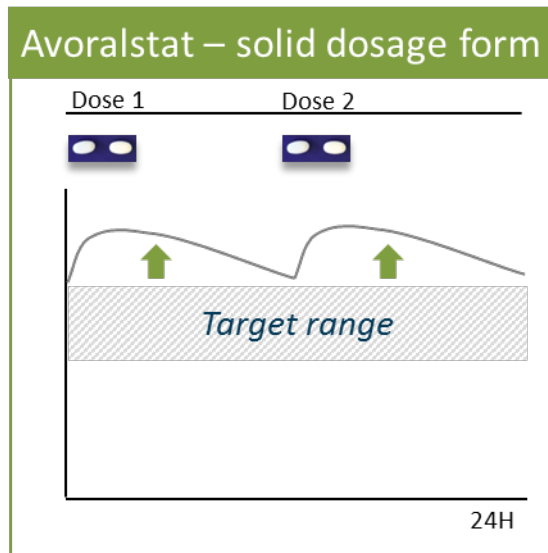
# A maturing & growing pipeline

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

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

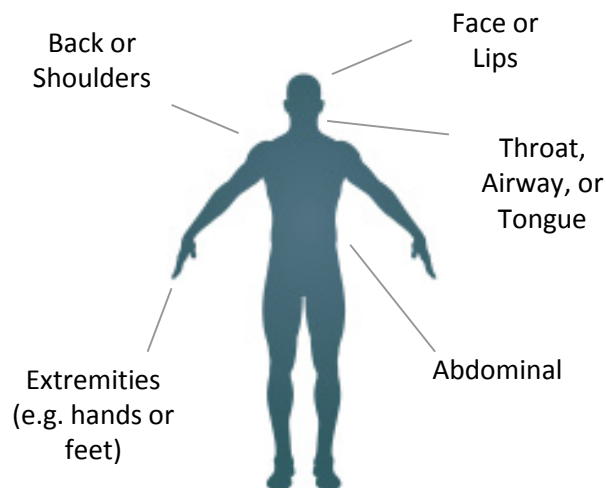
Avoralstat solid dose form

BCX7353

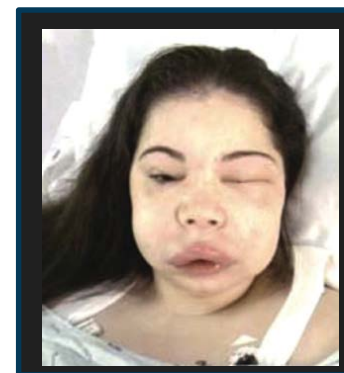
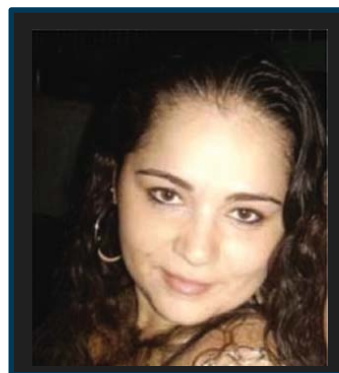
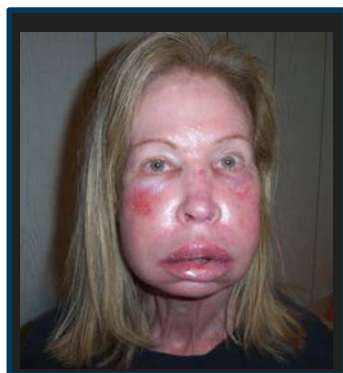
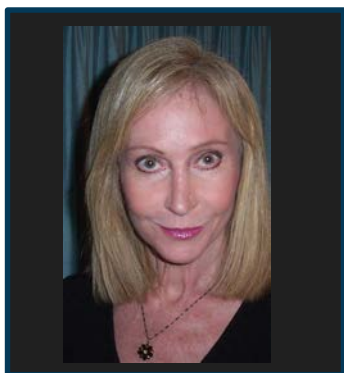


We have two shots at achieving our goal

# Unpredictability of HAE attack onset and severity drives need

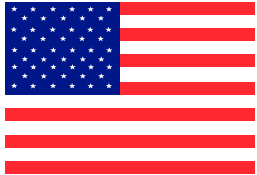


- Most patients have experienced **years of misdiagnosis or apathy** about their condition
- **Attacks are unpredictable**, regardless of underlying frequency
- **Any attack can cascade into a painful or dangerous event**, regardless of where it starts
- Nearly all patients have a **history of emergency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



Images obtained from [www.haeimages.com](http://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

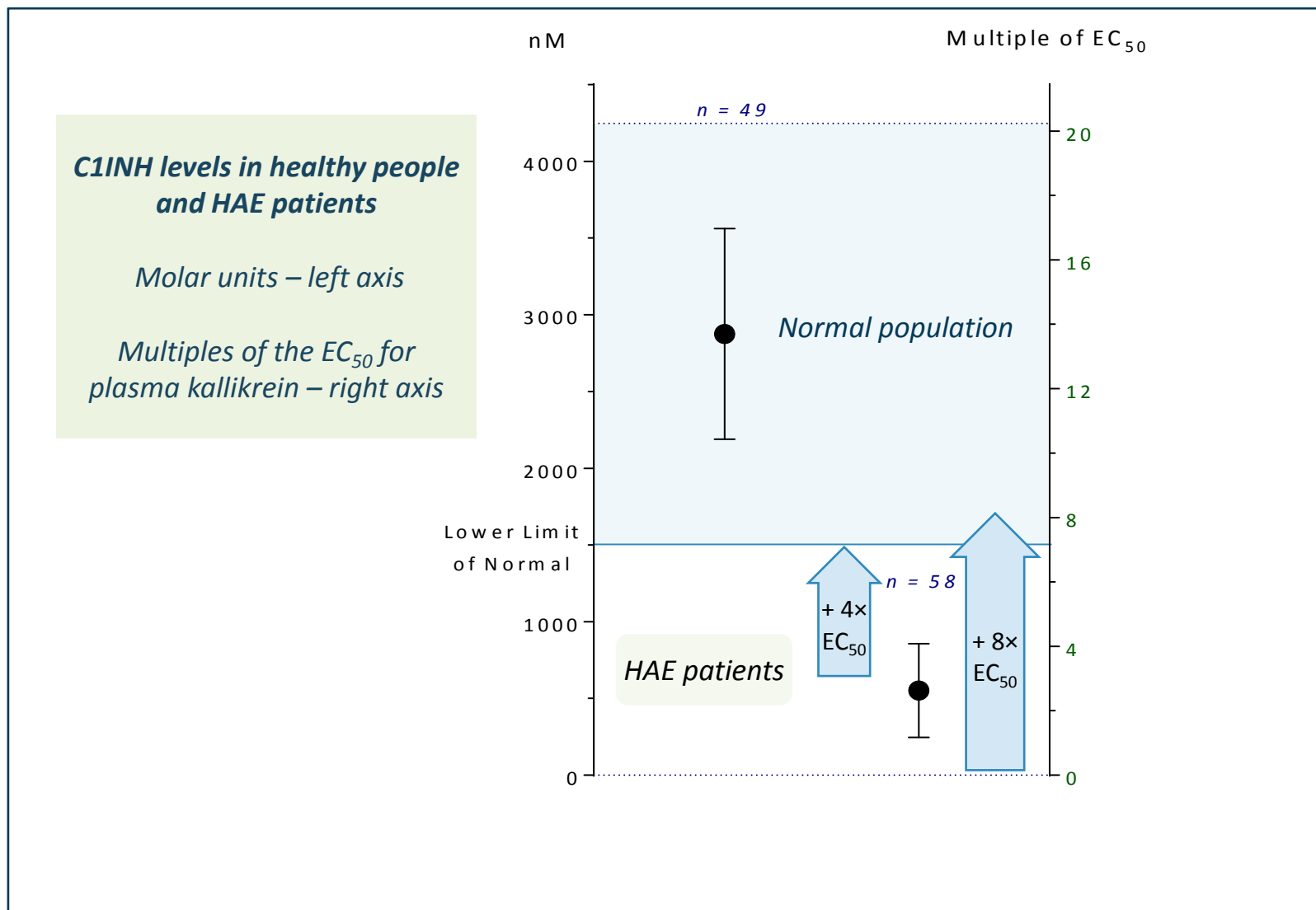


- **HAE significantly under-diagnosed** (~ 500 known patients out of estimated 3,000 prevalence)
- Opportunity for market expansion

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

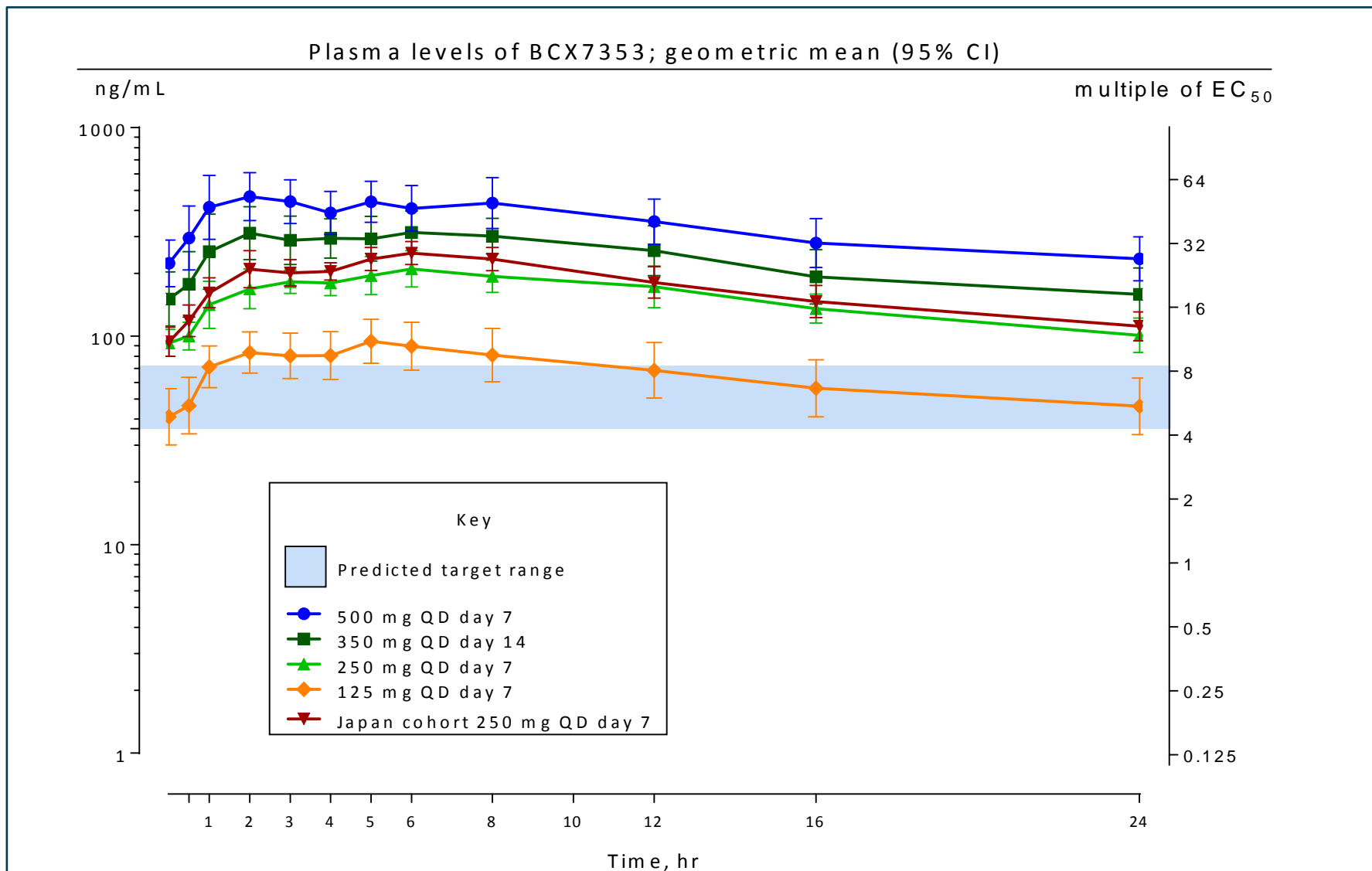
# Adding ~4 to ~8 times the $EC_{50}$ of a kallikrein inhibitor should restore normal function in many ( $4 \times EC_{50}$ ) to all ( $8 \times EC_{50}$ ) patients with HAE



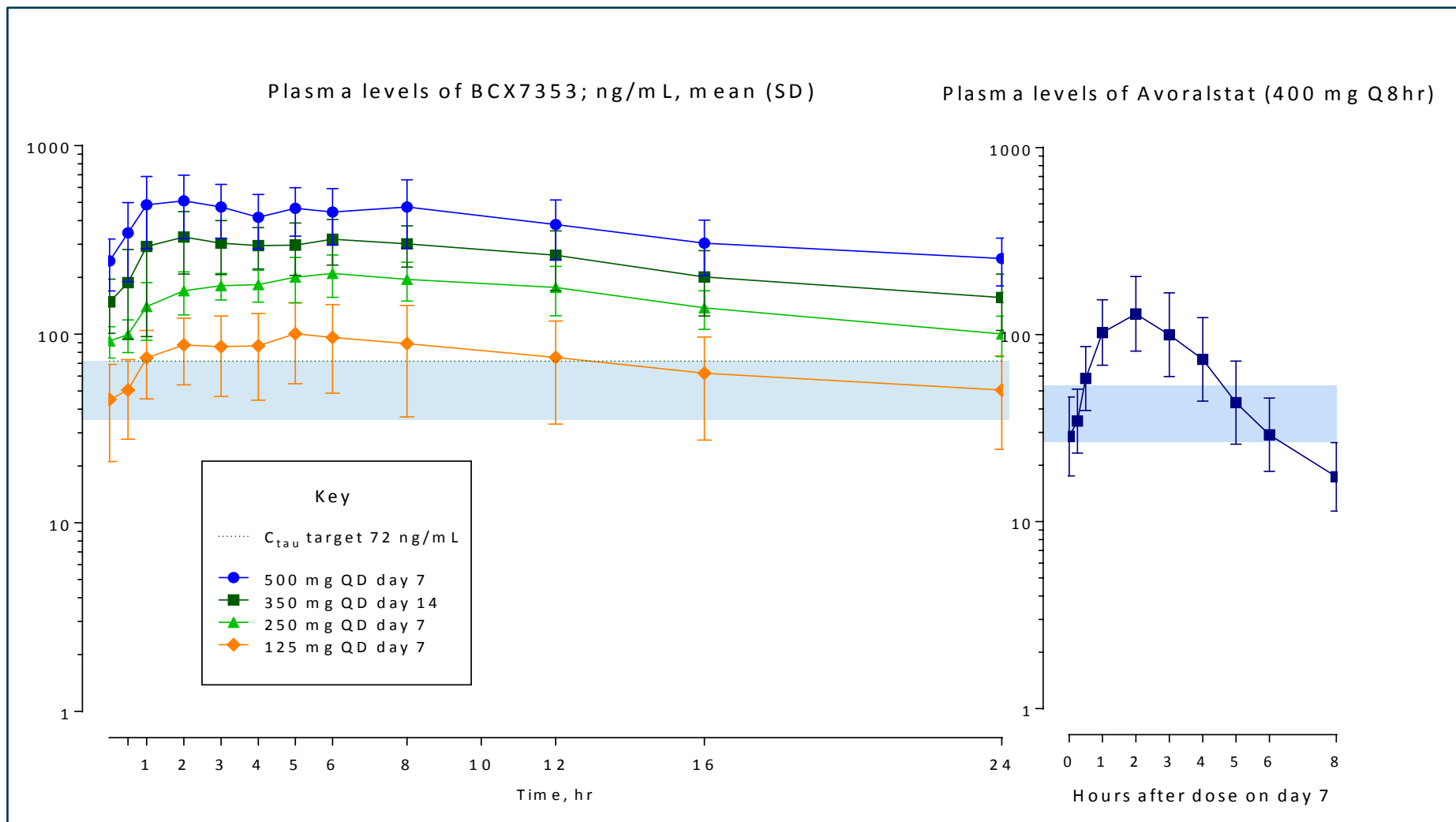
Tarzi, MD, et al. Clin Exp Immunol 2007; 149(3): 513-6  
Literature report of means and SD of C1INH in normal and patients with HAE  
(Lower Limit of Normal shown [LLN: mean – 2\*SD])



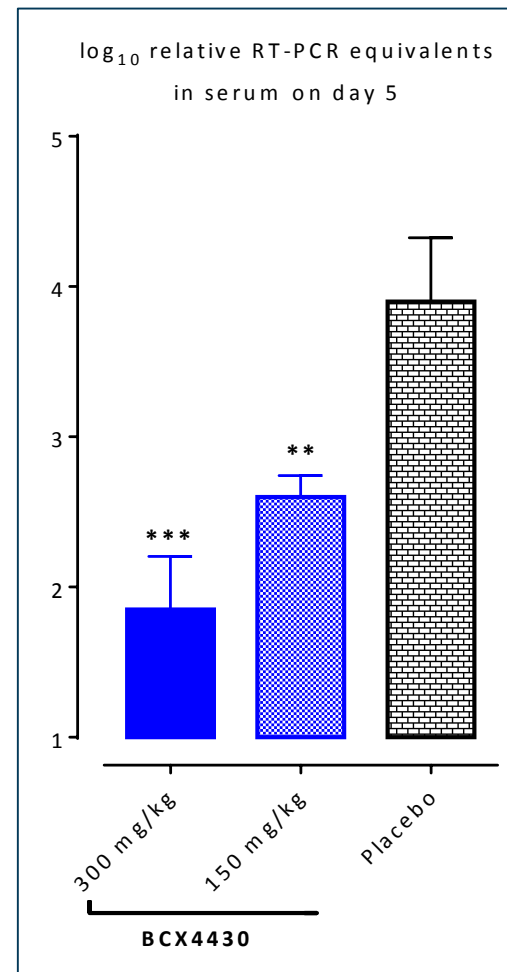
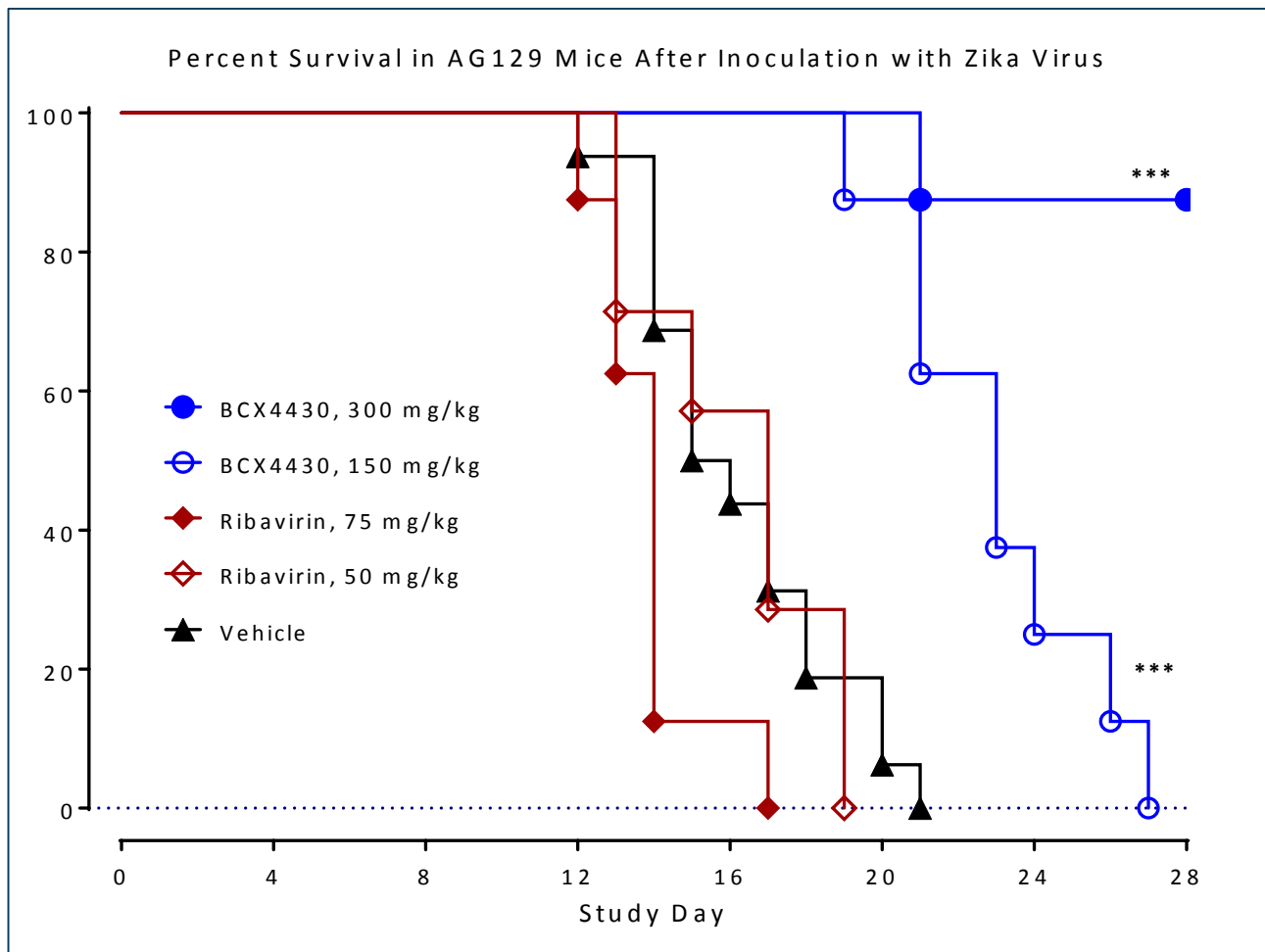
# BCX7353 PK profile supports once daily dosing and meets or exceeds therapeutic target levels



# 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

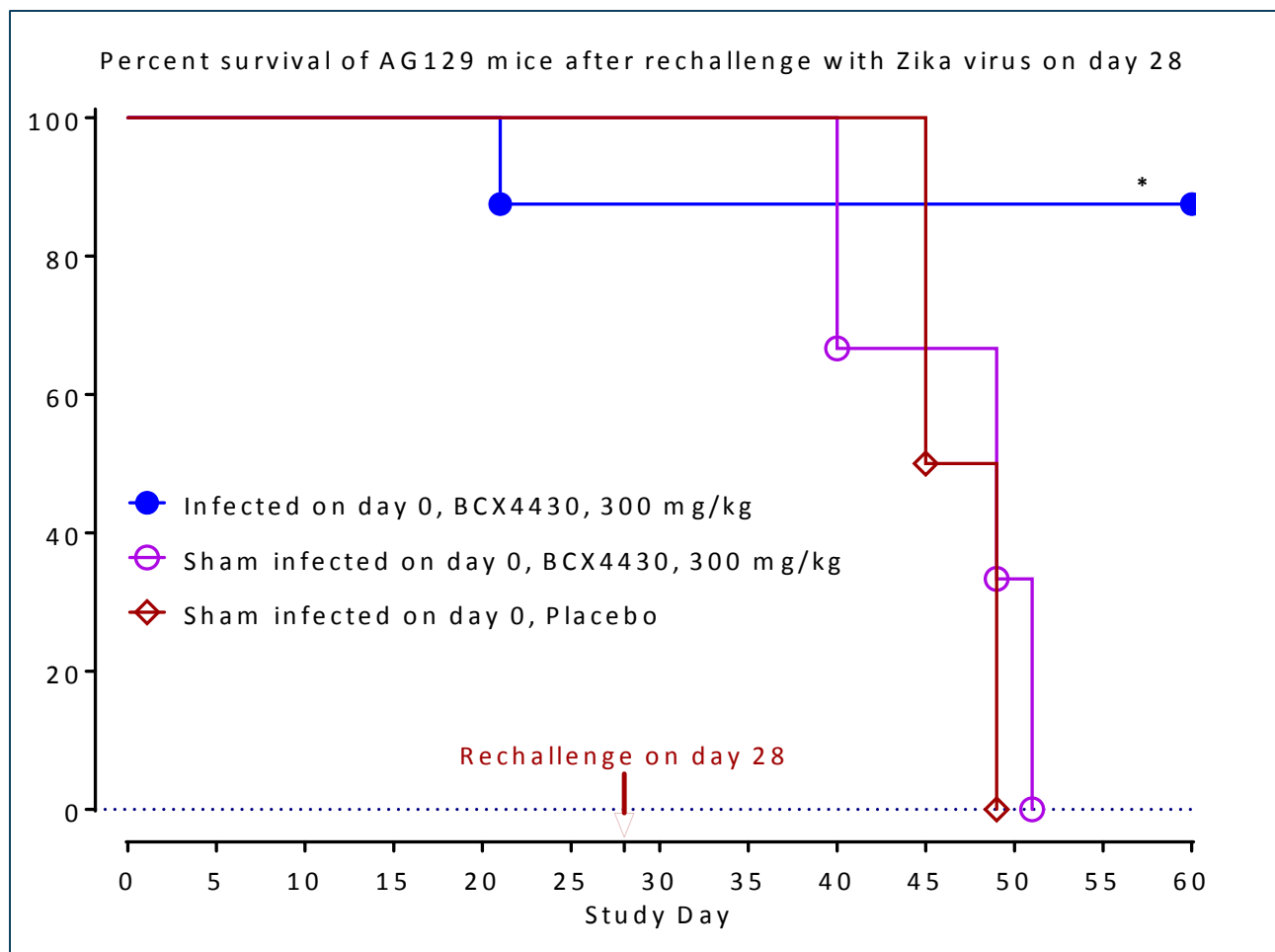


**BCX4430 has also shown a survival benefit in nonclinical studies of Ebola virus, Marburg virus & Yellow Fever virus infections**

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

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

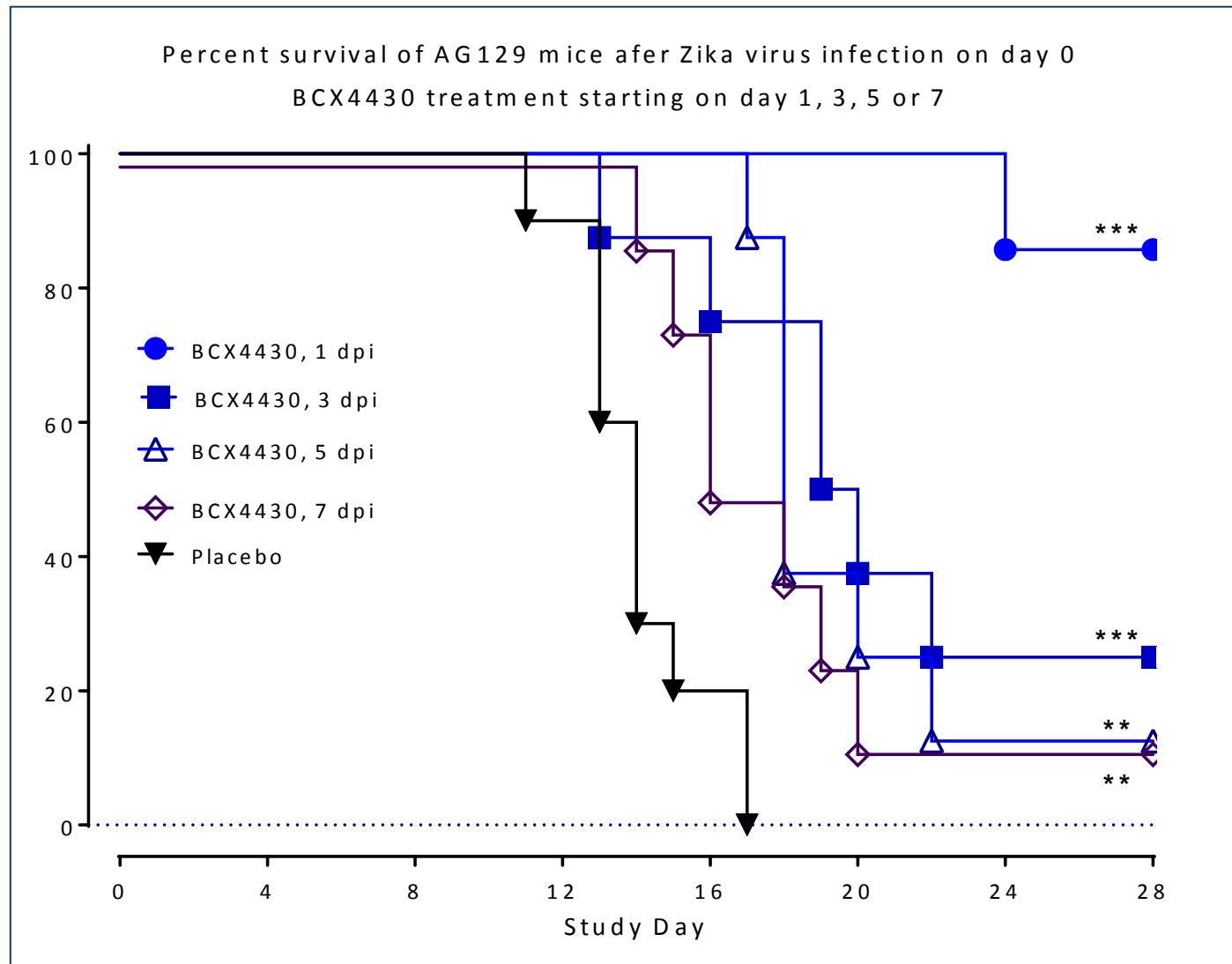
# AG129 mice treated with BCX4430 and surviving after initial infection are immune to re-challenge with Zika virus



\*  $p < 0.05$

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 <sup>#</sup>	\$78 – 98
Cash runway	Mid-2017

<sup>#</sup> Excludes equity-based compensation.

# Key HAE Program 2016 Milestones

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## 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)