34rd Annual J.P. Morgan Healthcare Conference

January 13, 2016

Jon Stonehouse, *President & Chief Executive Officer*

BioCryst Pharmaceuticals, Inc.

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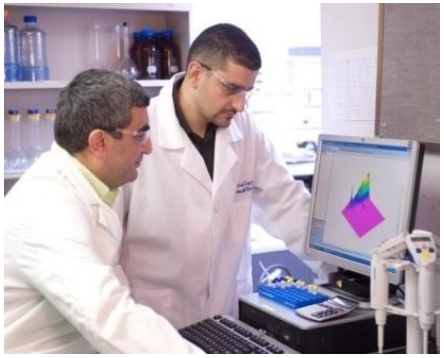
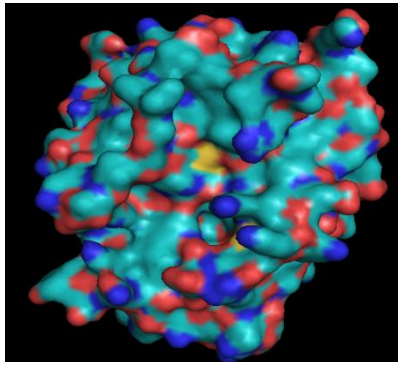
BioCryst PHARMACEUTICALS, INC.

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

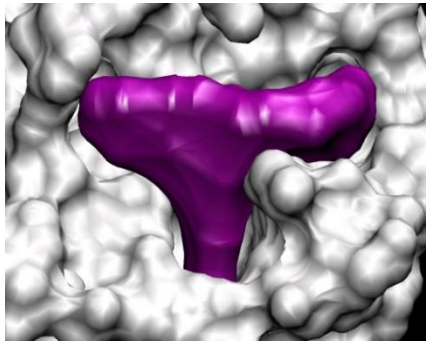
Experienced scientists



Structure-guided design



Validated targets



Value created by…

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

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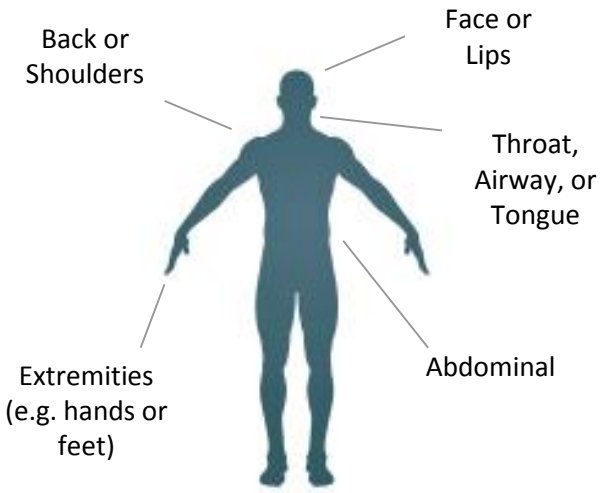
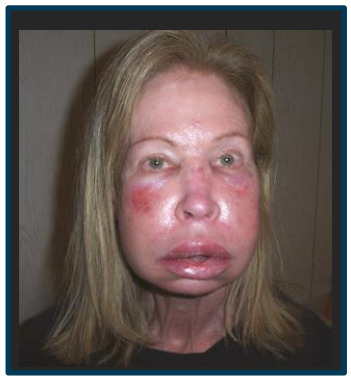
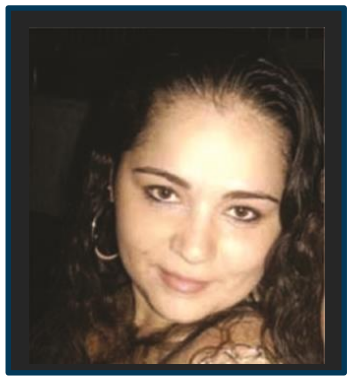
Our strategy is paying off: a maturing & growing pipeline

	Pre-clinical	Ph 1	Ph 2	Ph 3	Filed	Approved
CORE STRATEGY						
Avoralstat (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 <u>significantly</u> disrupt** daily life


Images obtained from www.haeimages.com

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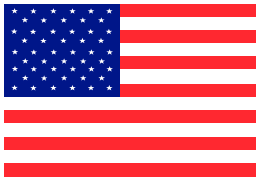
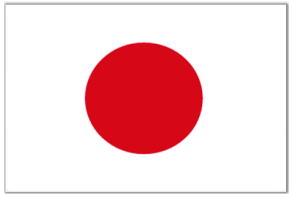
HAE market is growing quickly with substantial upside



- **$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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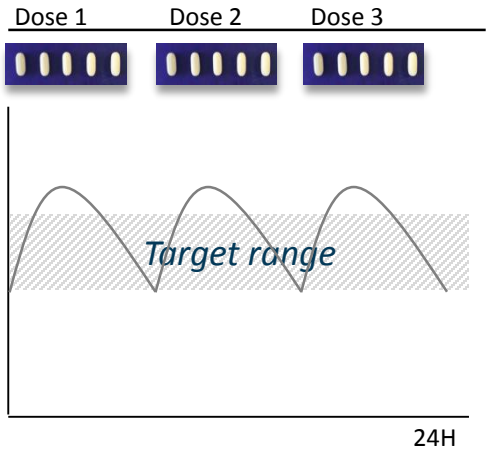
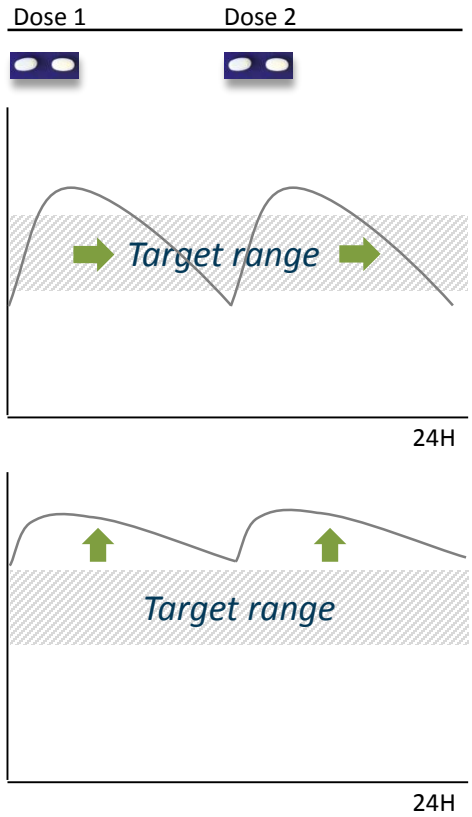
BioCryst has multiple ways to bring a highly attractive asset to market

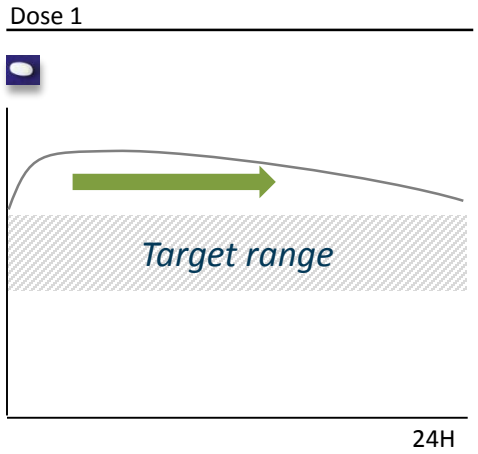
Avoralstat – soft gel caps



Avoralstat – solid dosage form



BCX7353



Goal: Conveniently dosed, highly effective, oral drug

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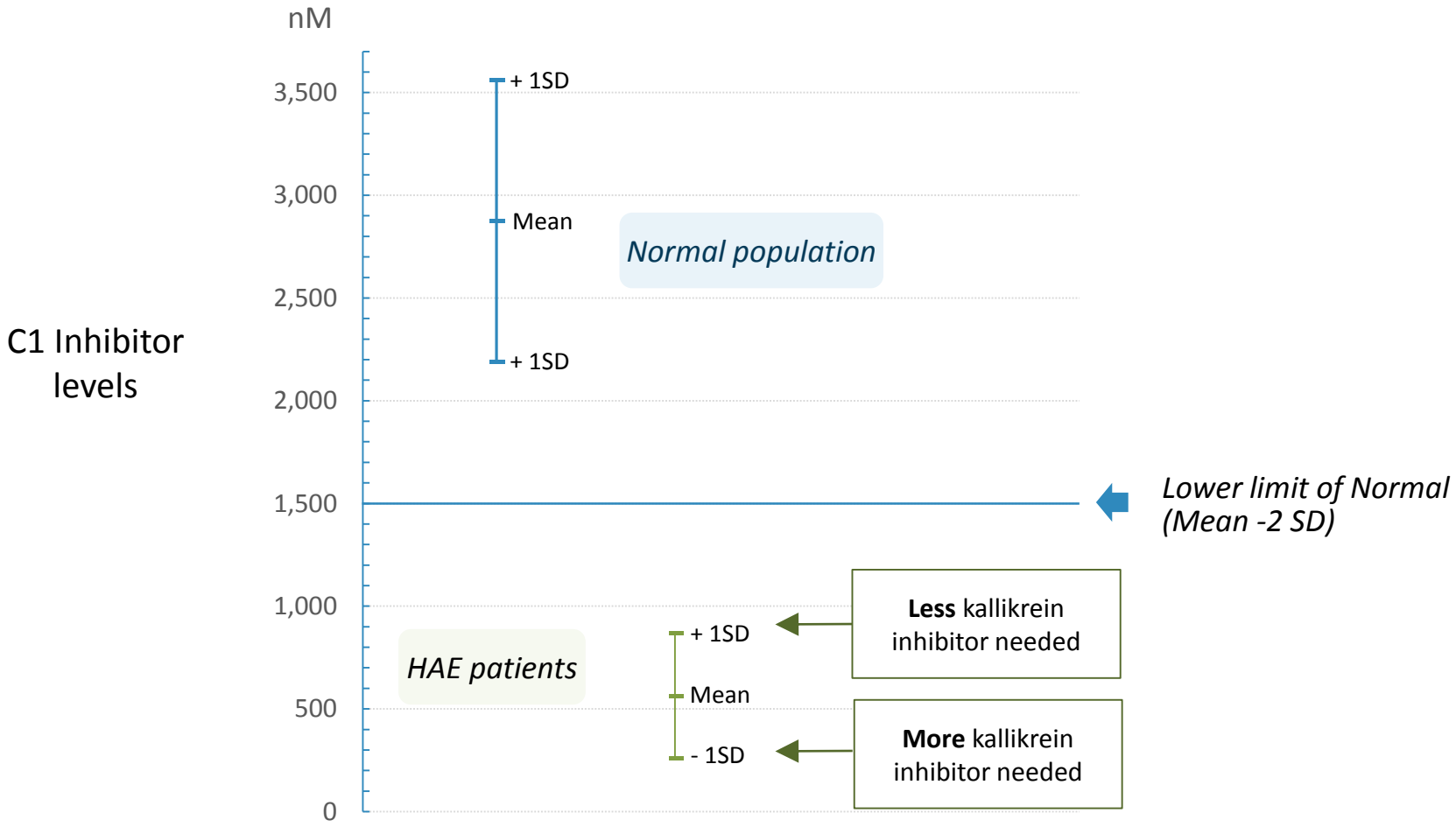
C1 inhibitor levels in healthy people determine target levels of kallikrein inhibition to restore normal phenotype for patients with HAE



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

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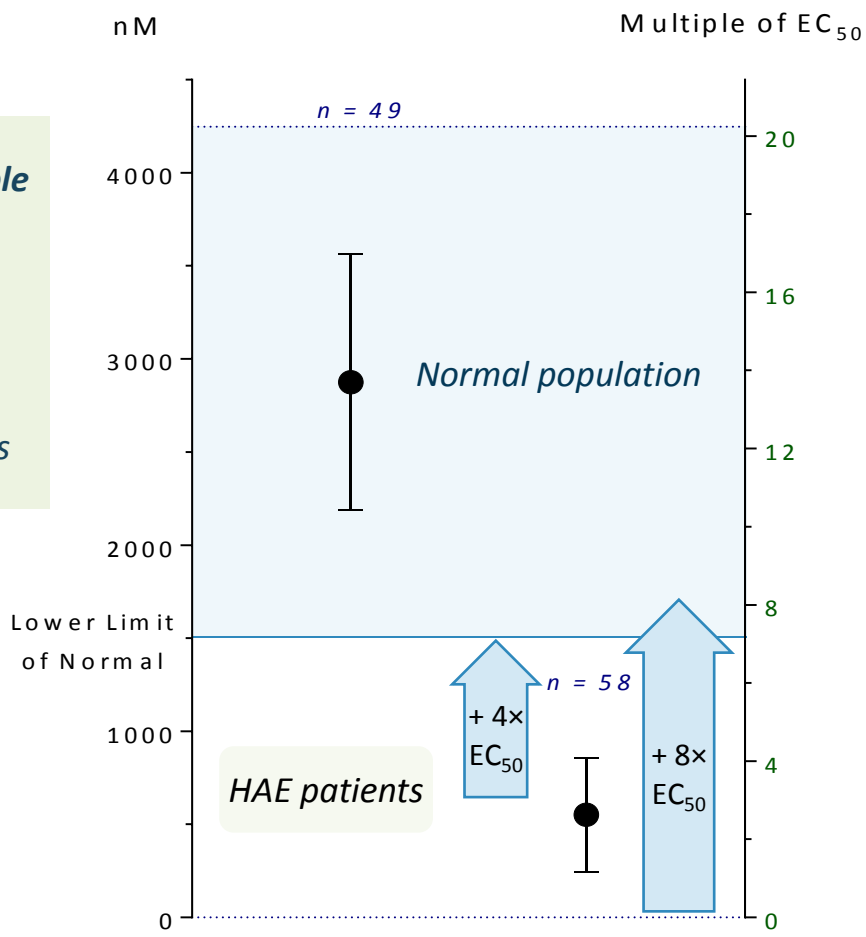
Adding ~4 to ~8 times the EC_{50} of a kallikrein inhibitor should restore normal function in many ($4\times EC_{50}$) to all ($8\times EC_{50}$) patients with HAE

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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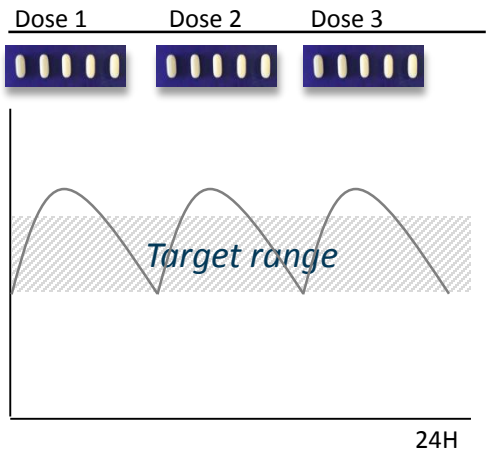
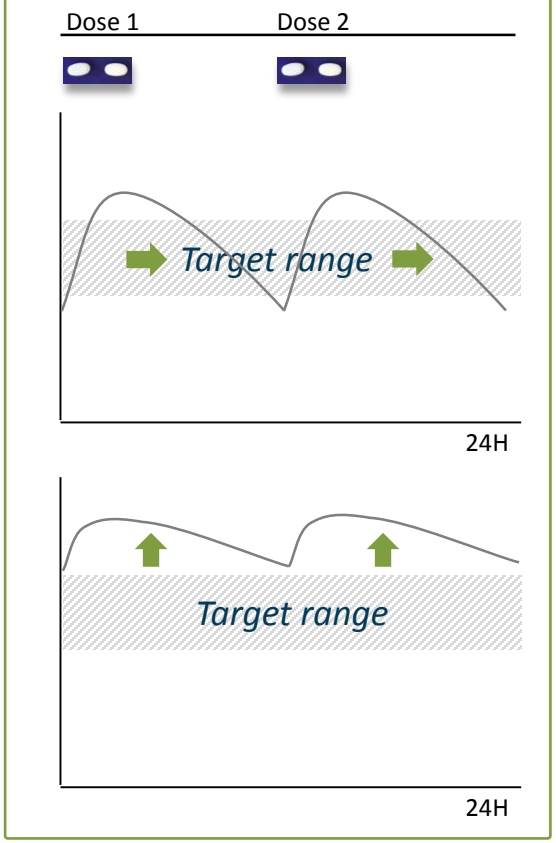
Avoralstat

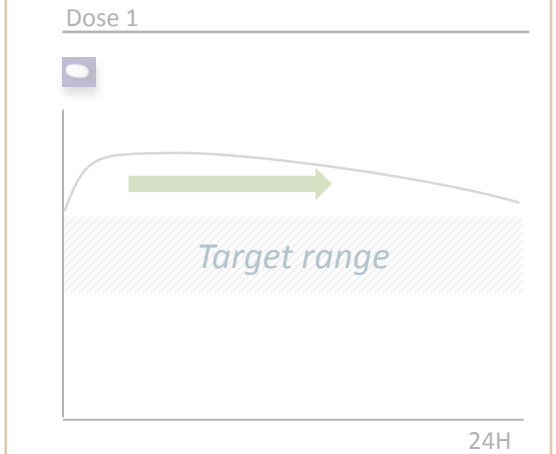
Avoralstat – soft gel caps



Avoralstat – solid dosage form



BCX7353



Goal: Conveniently dosed, highly effective, oral drug

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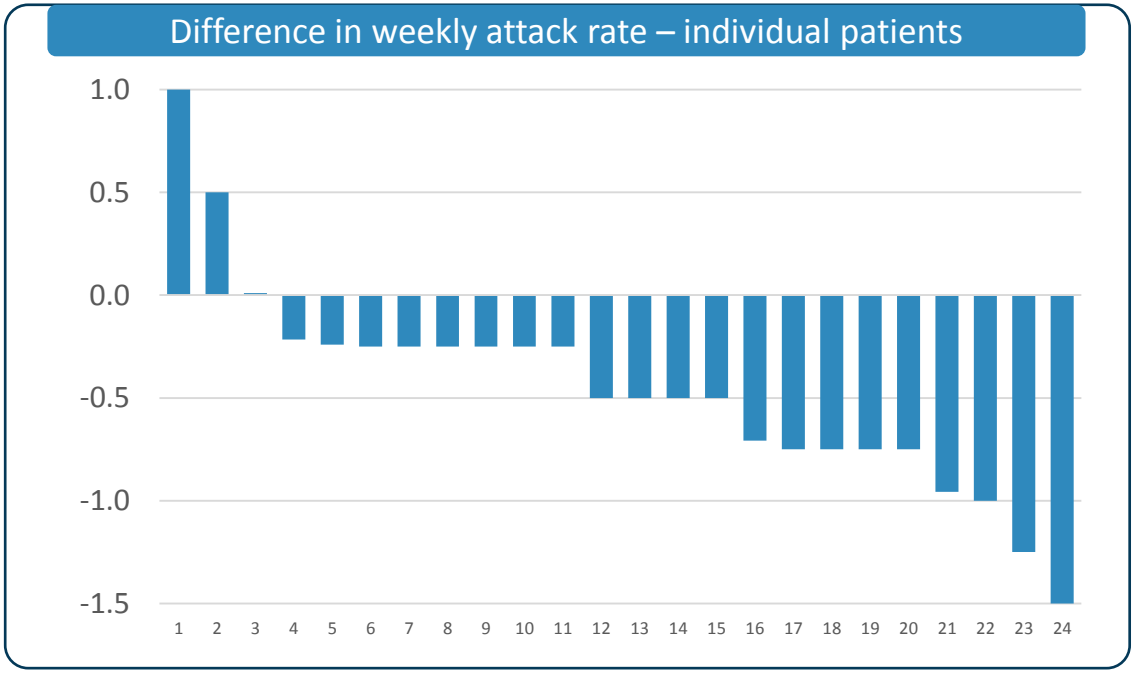
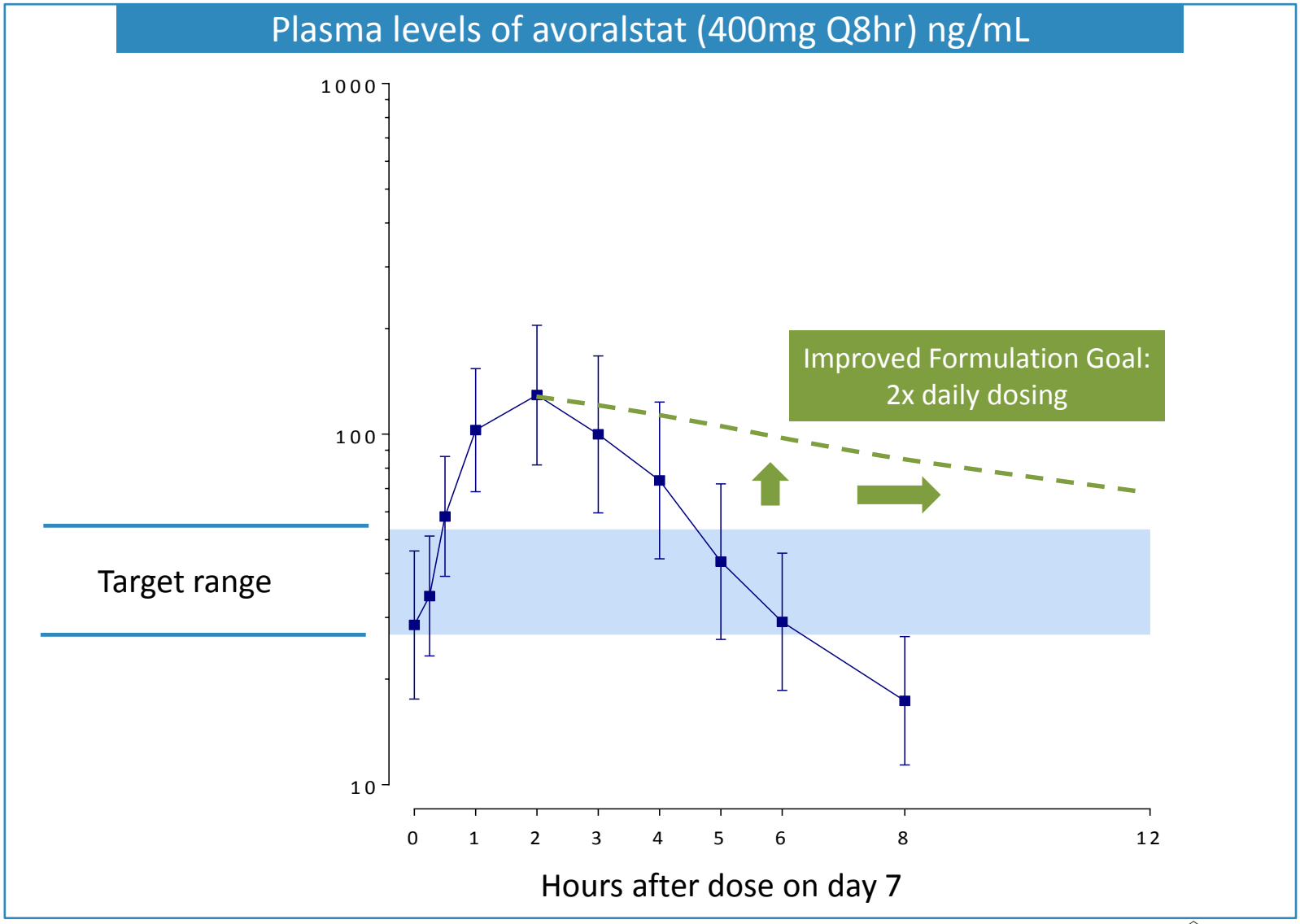
OPuS-1: Avoralstat proof of concept in high attack frequency patients

- High attack frequency: ~1.5/ week
- Significant reduction in attack rate p<0.001
- Significant improvement in patient-reported outcomes

Adjudicated attacks	Avoralstat period n=24	Placebo period n=24	Difference (95% CI)	P value
LS Mean Attack rate per week	0.82	1.27	-0.45 (-0.67 -0.23)	<0.001



BioCryst has the opportunity to improve avoralstat through solid dosage formulation



Avoralstat clinical development path forward

OPuS-2

- Trial in HAE
  - 300 mg TID
  - 500 mg TID
  - placebo
- Fully enrolled

Results early 1Q16

Solid-dose PK

- Healthy subjects
- Goals
  - BID dosing
  - Higher drug levels

Results mid-2016

OPuS-3

- Trial in HAE
- Based on:
  - OPuS-2
  - PK of solid dose

Start end of 2016

OPuS-4

- Long-term safety
- Goals:
  - 6 to 12 month dosing with avoralstat
  - Satisfy filing requirements

Results 2017

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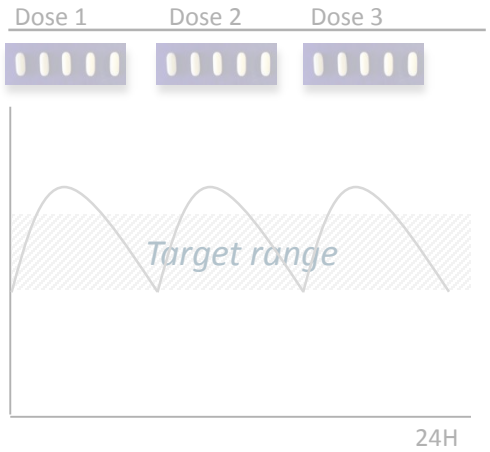
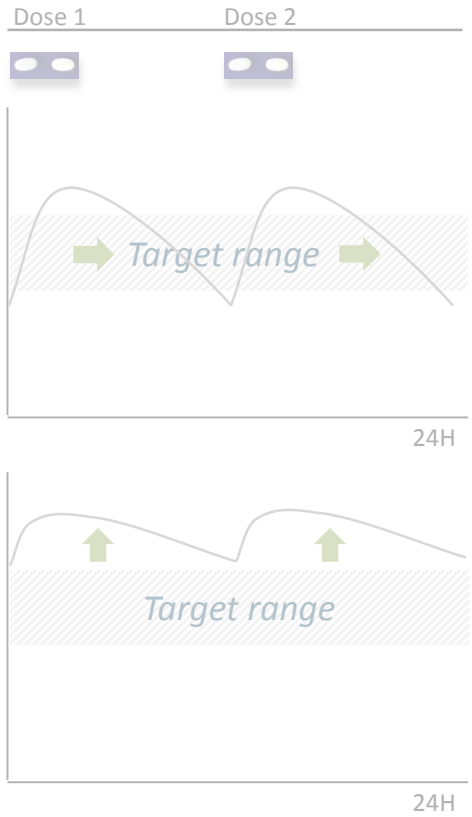
BCX7353

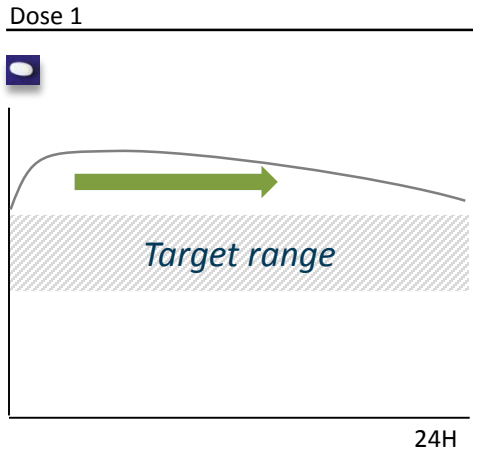
Avoralstat – soft gel caps



Avoralstat – solid dosage form



BCX7353



Goal: Conveniently dosed, highly effective, oral drug

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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

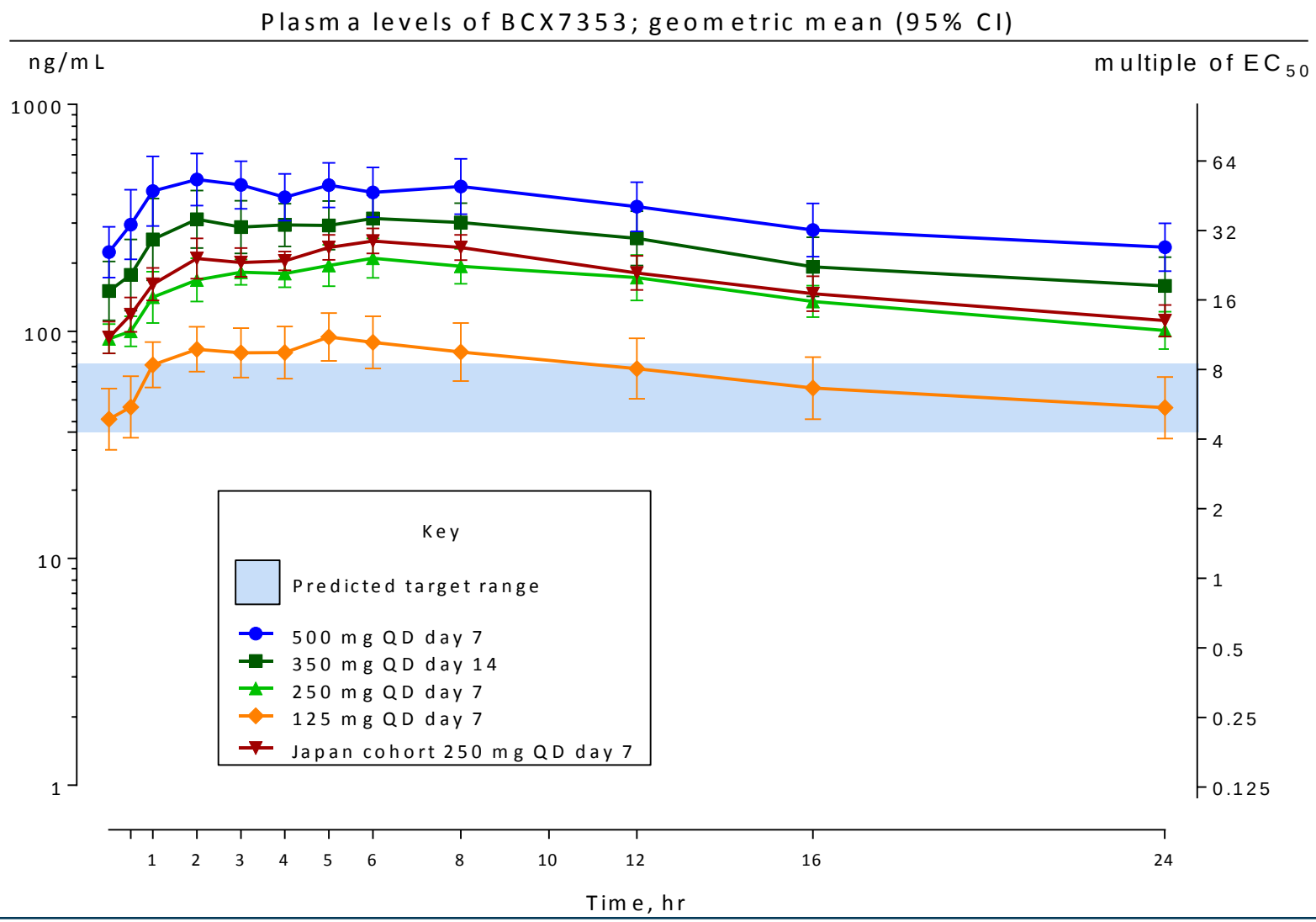
Cohort	Dose, mg QD	Duration, days	N BCX7353	N placebo
1	125	7	10	2
2	250	7	10	2
3	500	7	10	2
4	350	14	10	2
5 (Japanese)	250	7	10	2

Evaluations

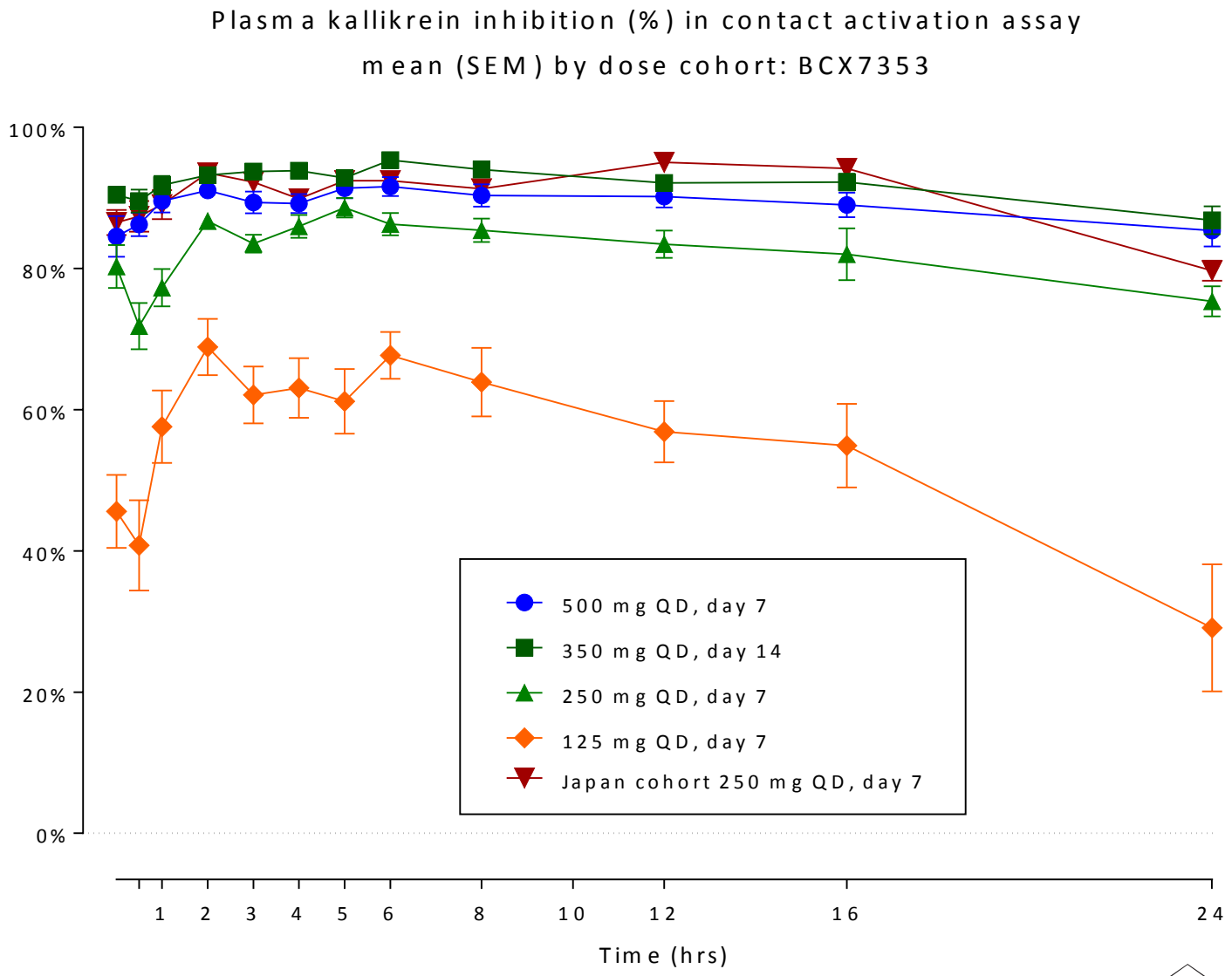
- Safety & tolerability: AEs, clinical and laboratory monitoring
- Pharmacokinetics: PK profile and PK parameters
- Pharmacodynamics: PD profile of inhibition of plasma kallikrein after oral dosing
- PK-PD correlations: E_{max} model
- Modeling of efficacious dose range for HAE studies: population simulations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

BioCryst Pharmaceuticals, Inc.

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 APeX-1 proof of concept trial preliminary design

APeX-1: Randomized placebo-controlled 4-week study of BCX7353 in HAE patients

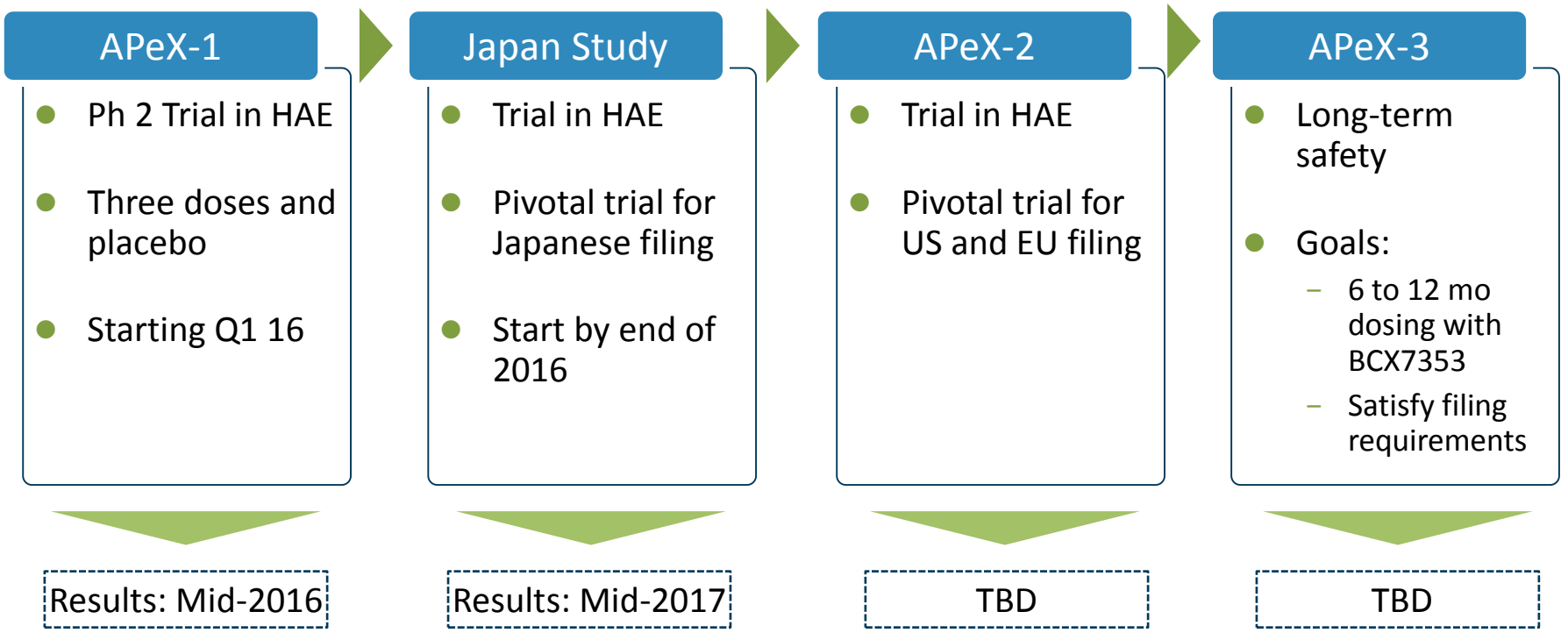
- Phase 2 dose-ranging
  - 3 doses of BCX7353
    - Lowest dose not less than 100 mg
    - Highest dose not greater than 350 mg

- Randomized placebo-controlled cohort study
- 4 week duration of blinded study drug orally once daily
- Primary endpoint: mean acute angioedema attack rate (same as in OPuS-1 & OPuS-2)
- Planned start in early 2016, following regulatory review and IRB approvals

Trial completion and results expected mid-2016

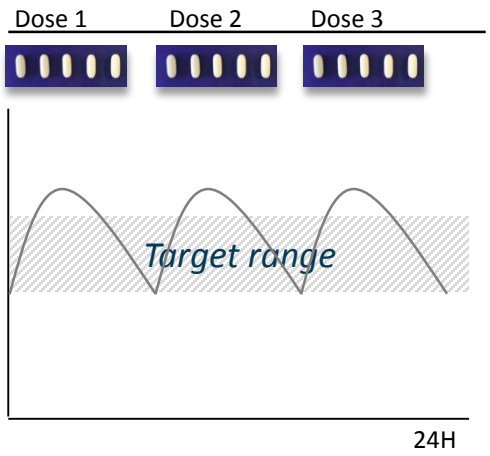
BioCryst Pharmaceuticals, Inc.

BCX7353 clinical development path forward

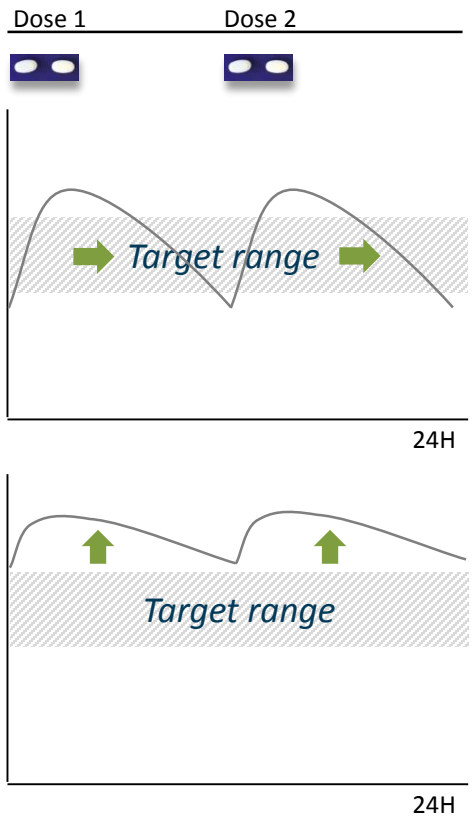


HAE strategy to achieve goal

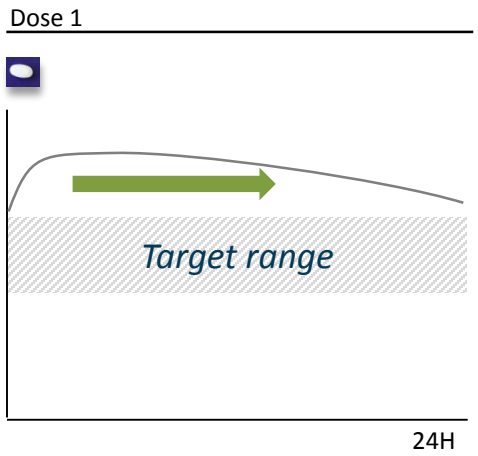
Avoralstat – soft gel caps



Avoralstat – solid dosage form



BCX7353



Goal: Conveniently dosed, highly effective, oral drug

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Cash position & 2015 guidance (in millions)

Cash & investments at September 30, 2015	$119.7
Operating cash utilization through Sept 30	$28.1
Operating cash generation through Sept 30 with Seqirus upfront	$5.7
2015 Guidance	
Operating cash utilization[+]	($8 – 18)
Operating expenses[#]	$75 – 95
Cash runway	Into 2017

+Excludes hedge adjustment and includes upfront cash payment.

Excludes equity-based compensation.

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Key 2016 HAE Program Milestones

Avoralstat

✓ Start OPuS-4 (long-term safety)	(early 2016)
• OPuS-2 results (TID efficacy at 300mg and 500mg)	(early 1Q16)
• PK study of solid dose form	(mid-2016)
• End-of-Phase 2 meeting with the FDA	(mid-2016)
• Start OPuS-3 pivotal trial	(end of 2016)

BCX7353

• Start APeX-1 proof of concept trial	(1Q 2016)
• Complete APeX-1 proof of concept trial	(mid-2016)
• Start pivotal study in Japan under *sakigake* process	(end of 2016)

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