35th Annual J.P. Morgan Healthcare Conference

January 9-11th, 2017

Jon Stonehouse, *President & Chief Executive Officer*

BioCryst Pharmaceuticals, Inc.

Forward-looking statement

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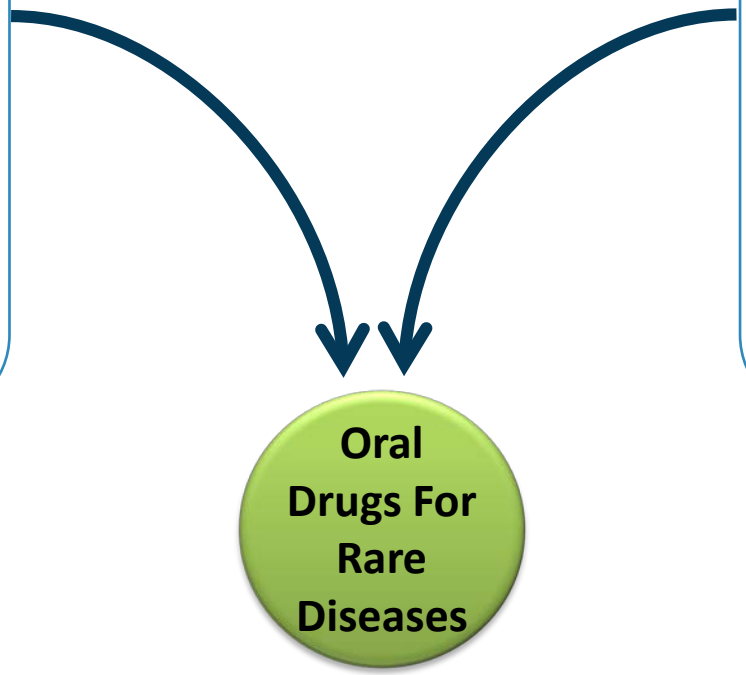
BioCryst's strategy is to develop oral drugs for rare diseases

Drug discovery through structure-based design

- Lead optimization underway for two additional rare disease targets

Significant supporting capital from antiviral programs

- RAPIVAB® (peramivir injection) and Galidesivir
- Externally funded
- Stockpiling and voucher potential



Help patients lead normal lives

BCX7353 on track to report results for hereditary angioedema (HAE) in 1Q 2017

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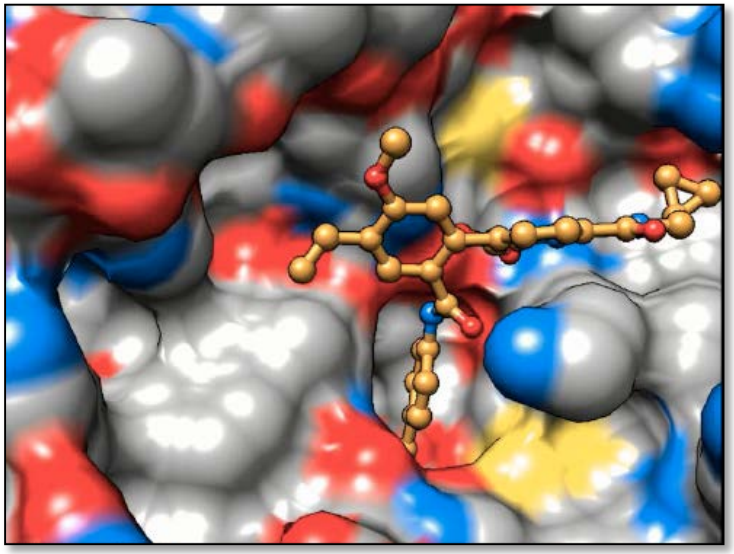
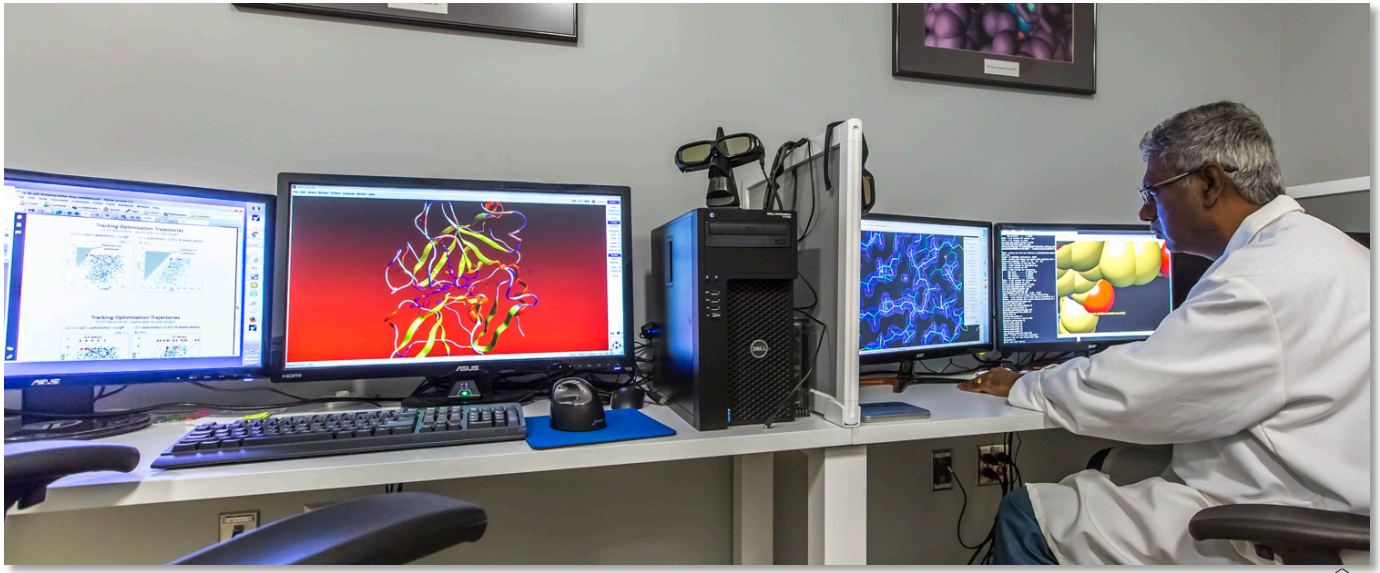
What makes BioCryst different?

Experienced drug discovery group (average tenure >15 years) focused on structure-based drug design

- Molecules built based on shape and charge of active site using iterative process
- Potent and specific enzyme blockers of challenging targets (e.g., Serine proteases, kinases, etc.)
- Ability to generate different classes of candidates quickly and efficiently
- Emphasize validated scientific targets to decrease risk





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Evidence of *in vitro* potency and specificity

RAPIVAB® (peramivir injection)		Galidesivir		BCX7353	
Neuraminidase enzyme assay	IC_{50}	Viral replication or polymerase enzyme assay	EC_{50} or IC_{50}	Serine protease enzyme assay	IC_{50} or EC_{50} (nM) or fold difference
Influenza A/H1N1 strains NA[2]	0.01-1.8 nM	Ebola virus replication [1]	11.8 µM	Plasma kallikrein K_i	< 1 nM
Influenza B strains NA[2]	0.04 – 54 nM	Marburg viruses replication [1]	4.4-6.7 µM	EC_{50} in normal plasma	< 10 nM
Mammalian NA[3]	> 300 µM	Human DNA polymerases	> 100 µM	Trypsin	> 10,000 fold
Bacterial NA[3]	> 300 µM	Human RNA polymerase II	> 100 µM	Tissue kallikrein	> 2,000 fold
Parainfluenza viral NA[3]	> 300 µM	Human mtRNA polymerase	> 100 µM	Doubling of prothrombin time	> 9,000 fold

1. Warren, T. K. *et al Nature* ***508, 402-405, doi:10.1038/nature13027 (2014).***
2. RAPIVAB Package Insert
3. Bantia, S. *et al Antimicrobial Agents and Chemotherapy* ***45, 1162-1167, doi:10.1128/AAC.45.4.1162-1167.2001 (2001)***

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BioCryst's pipeline

	Lead optimization	Pre-clinical	Ph 1	Ph 2	Ph 3	Filed	Approved
STRATEGY: Develop oral therapies for life-threatening, rare diseases							
BCX7353 (HAE)	APeX-1, part 1 results expected 1Q17						
Next generation kallikrein inhibitors							
Rare disease 1							
Rare disease 2							
SUPPORTING ASSETS: Externally funded, potential for significant capital infusions							
RAPIVAB® (peramivir injection)*							
Galidesivir (broad spectrum antiviral) I.M.							
Galidesivir (broad spectrum antiviral) I.V.							

*licensed to Seqirus, Shionogi, & Green Cross in various geographies—additional filings anticipated

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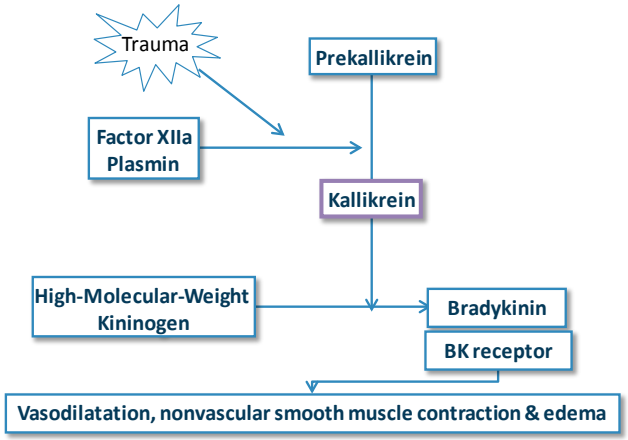
First target in strategy: Hereditary angioedema (HAE) is a high-need, high-value disease

Unpredictable, debilitating, potentially life-threatening swelling attacks



Contact activation pathway is unbalanced based on genetic deficiency of C1 inhibitor (C1-INH)

- Rare (estimated global prevalence of 1:50K)
- Growing US market ($1.2B, 30% growth in 2015)
- Significant global upside as paradigm shifts to attack prevention

High-value, growing market on track to exceed $2.0B globally

- Plasma-derived C1-INH (chronic and acute, infusion)
- Androgen steroids (chronic, oral)
- Bradykinin inhibitor (acute, injection)
- Kallikrein inhibitor (acute, injection)

Current standard of care therapies are injected/infused

Images obtained from www.haeimages.com
Market estimates based on analyst reports, earnings reports, and market data

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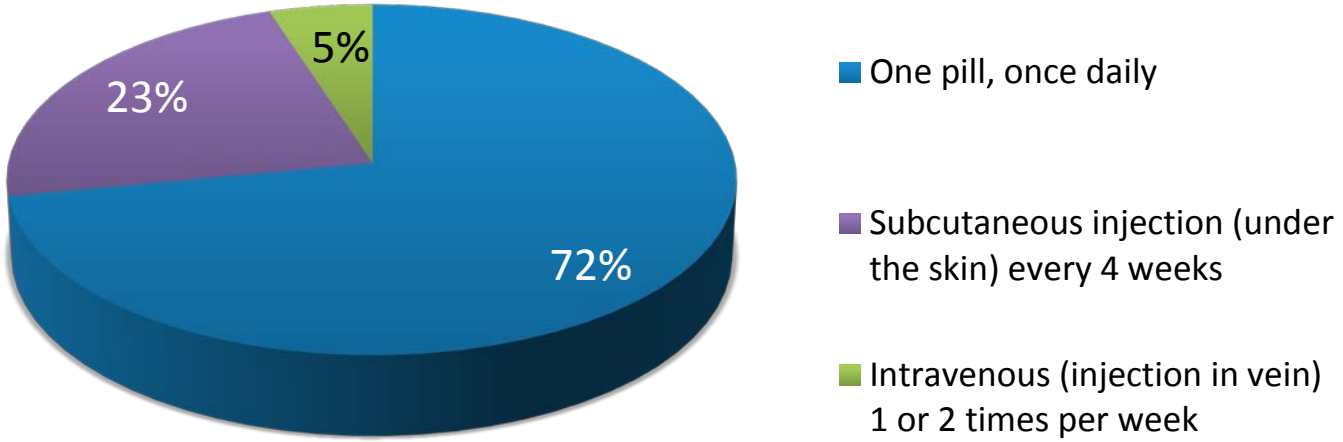
Patients with HAE overwhelmingly prefer convenient oral therapy

Preferred route of administration among US HAE patients currently taking prophylactic therapy (N=83)



Question: Which medication would you prefer assuming each is equally safe and effective and costs the same amount per year?

Source: BioCryst Proprietary Quantitative Market Research Study with Patients, 2016.

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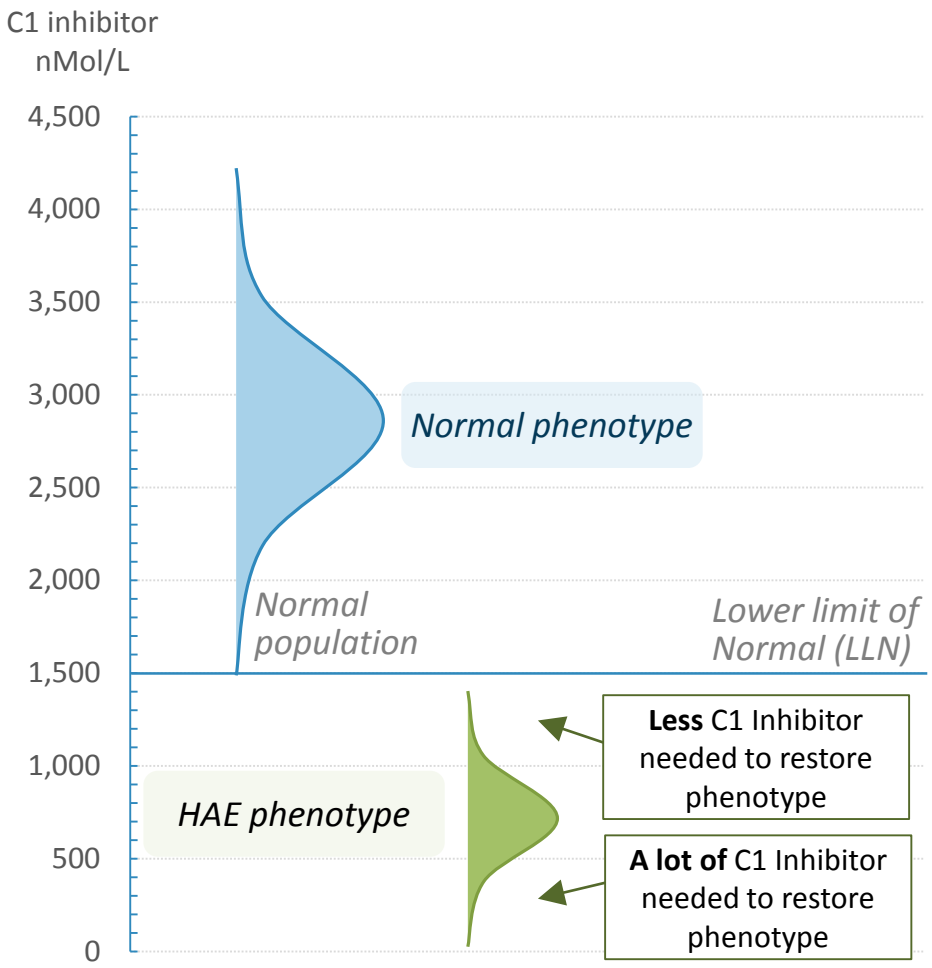
C1 inhibitor levels in healthy people confirm the target range for restoring the normal phenotype of kallikrein inhibition in patients with HAE



Hypothesis: *Increasing C1INH levels to > lower limit of normal (LLN) should eliminate angioedema attacks in HAE patients*

Questions:

1. *How much drug is needed to maintain kallikrein inhibition equivalent to > LLN for C1INH?*
2. *With higher drug doses, do we maintain higher blood levels?*
3. *Does maintaining higher blood levels give better response rates in HAE patients?*
4. *Can daily oral dosing with BCX7353 maintain the drug levels needed?*
5. *What proportion of patients could be expected to achieve these drug levels with daily oral dosing of BCX7353?*

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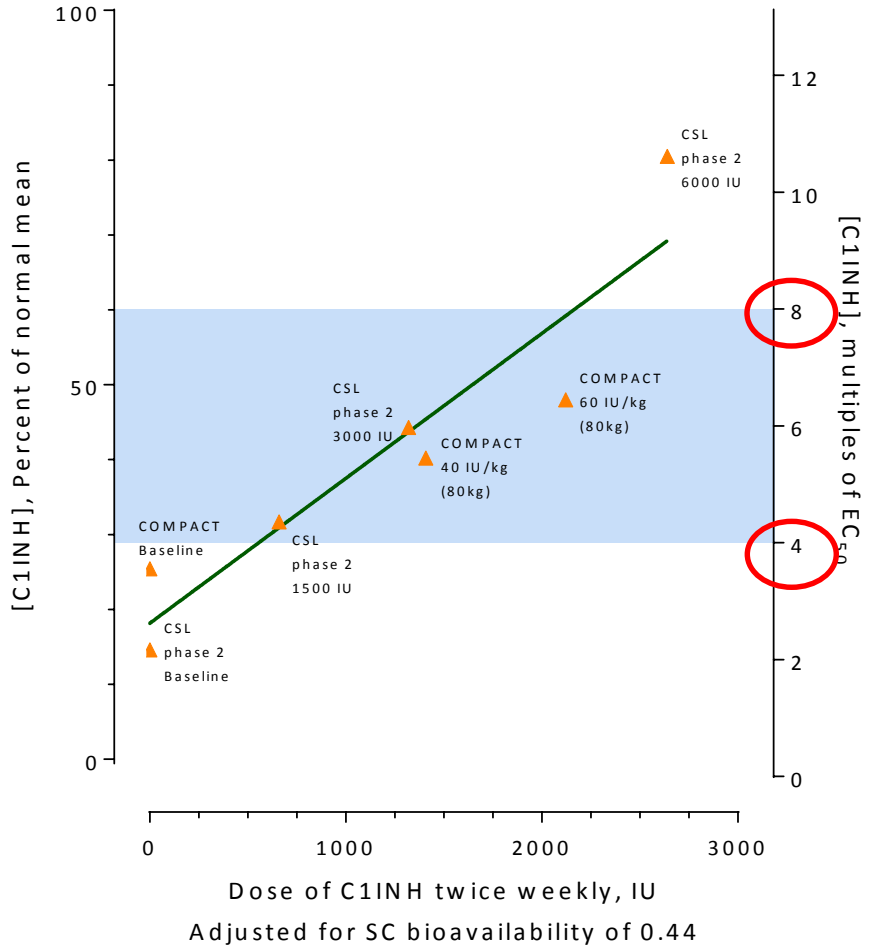
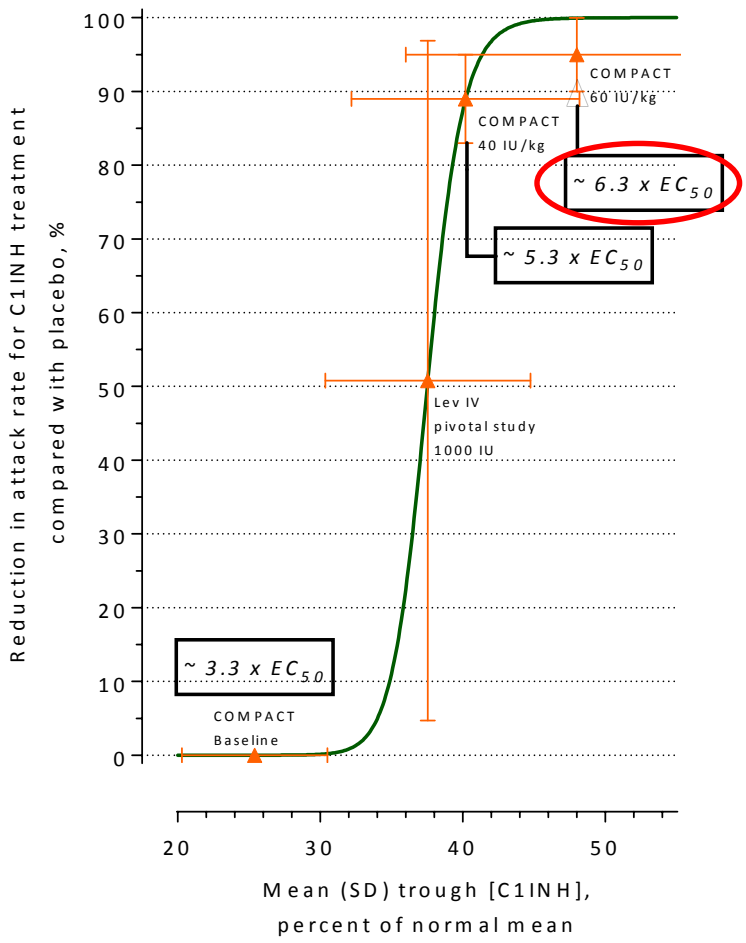
Higher trough levels of total C1INH (endogenous + dosed) during twice weekly administration for prophylaxis of HAE are associated with better efficacy

Dose-exposure analysis of SC C1INH



Estimated exposure-response for C1INH



Sources: Zuraw, B. L. *et al. Allergy* ***70, 1319-1328***, FDA Clinical Review (Cinryze), Cinryze label, CSL presentation at ACAAI 2016

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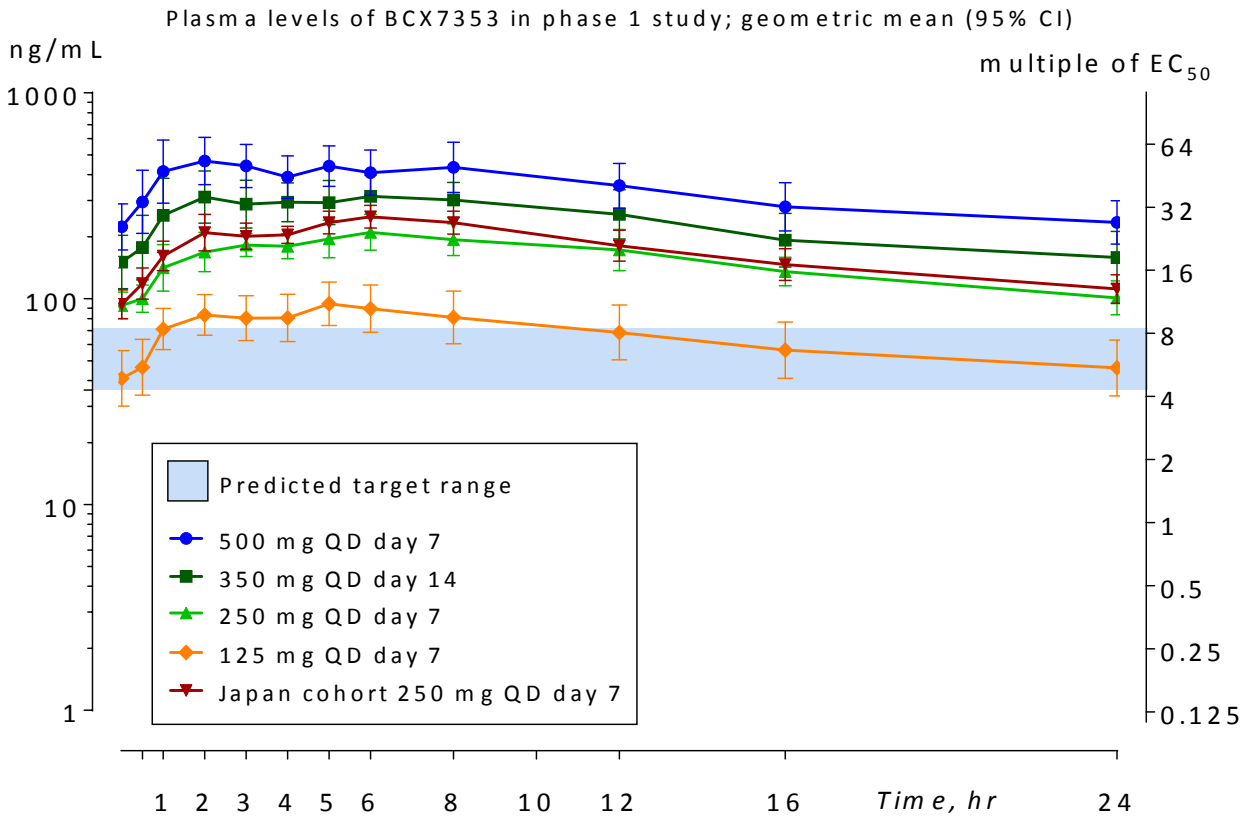
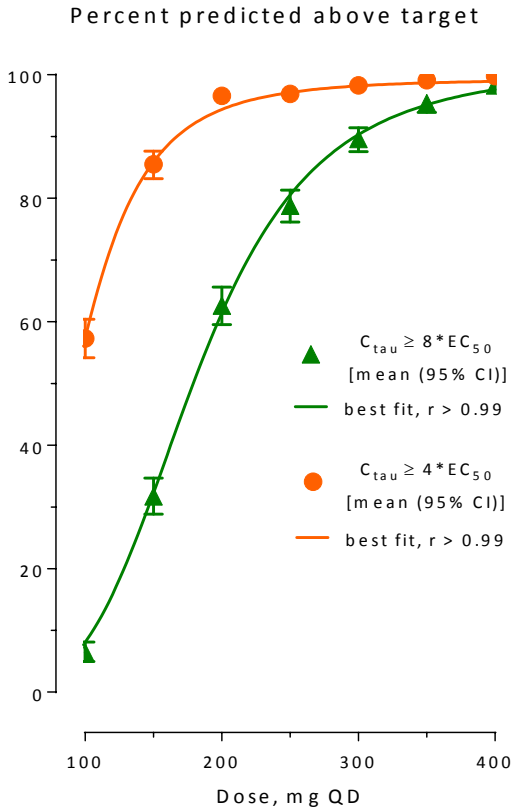
Daily oral dosing with BCX7353 in healthy subjects achieves trough levels that meet or exceed the target range for efficacy

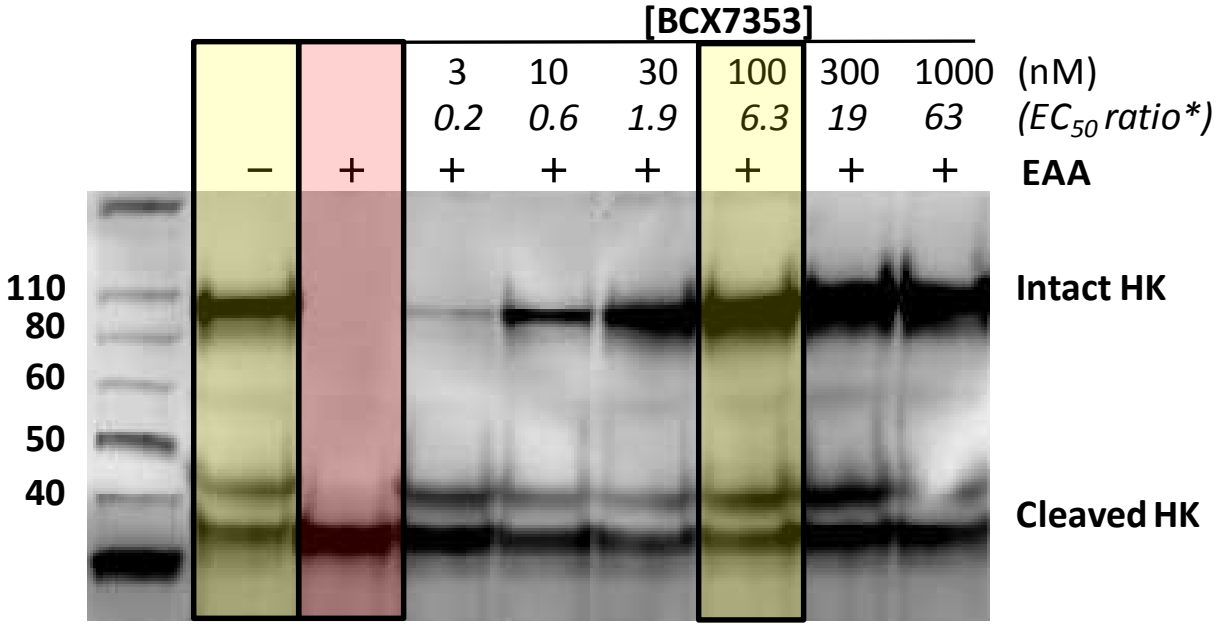




Data and PK population simulations presented at AAAAI 2016. Cornpropst, M. *et al. in AAAAI Vol. 137 AB-401 (Los Angeles, 2016).*

In Vitro Activity: BCX7353 blocks HK cleavage in HAE plasma

100nM of BCX7353 (6.3 X EC_{50} ratio) inhibits essentially all EAA-stimulated HK cleavage in HAE plasma



multiples of EC_{50} in fluorometric plasma kallikrein inhibition assay (15.9 nM)

Methods

94 μL of fresh HAE patient plasma + 0.15 μL of ellagic acid (EAA) + 1 μL of 7353, incubated for 5 minutes to activate contact pathway. Reaction was stopped and applied on the gel.

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BCX7353 was generally safe and well tolerated over the range of doses and durations tested in Phase I

Single doses of 10 mg through 1000 mg

- No SAEs
- No clinically significant laboratory abnormalities
- 31 of 34 AEs were mild (grade 1)
- Three grade 2 events:
  - 1 subject in 100 mg cohort with moderate (grade 2) nausea and vomiting (2 AEs)
  - 1 subject in 100 mg cohort with moderate (grade 2) hay fever

Once daily doses of 125 mg, 250 mg and 500 mg for 7 days; 350 mg for 14 days

- No SAEs
- No clinically significant laboratory abnormalities
- 48 of 54 AEs were mild (grade 1)
- Five grade 2 events and 1 grade 3 event:
  - 350 mg QD x 14d cohort: 1 subject grade 2 upper abdominal pain (discontinued from study)
  - 500 mg QD x 7d cohort: 1 subject grade 2 syncope, 1 subject grade 2 headache, 1 subject grade 2 diarrhea and upper abdominal pain (2 AEs, discontinued from study), 1 subject grade 3 hypersensitivity reaction

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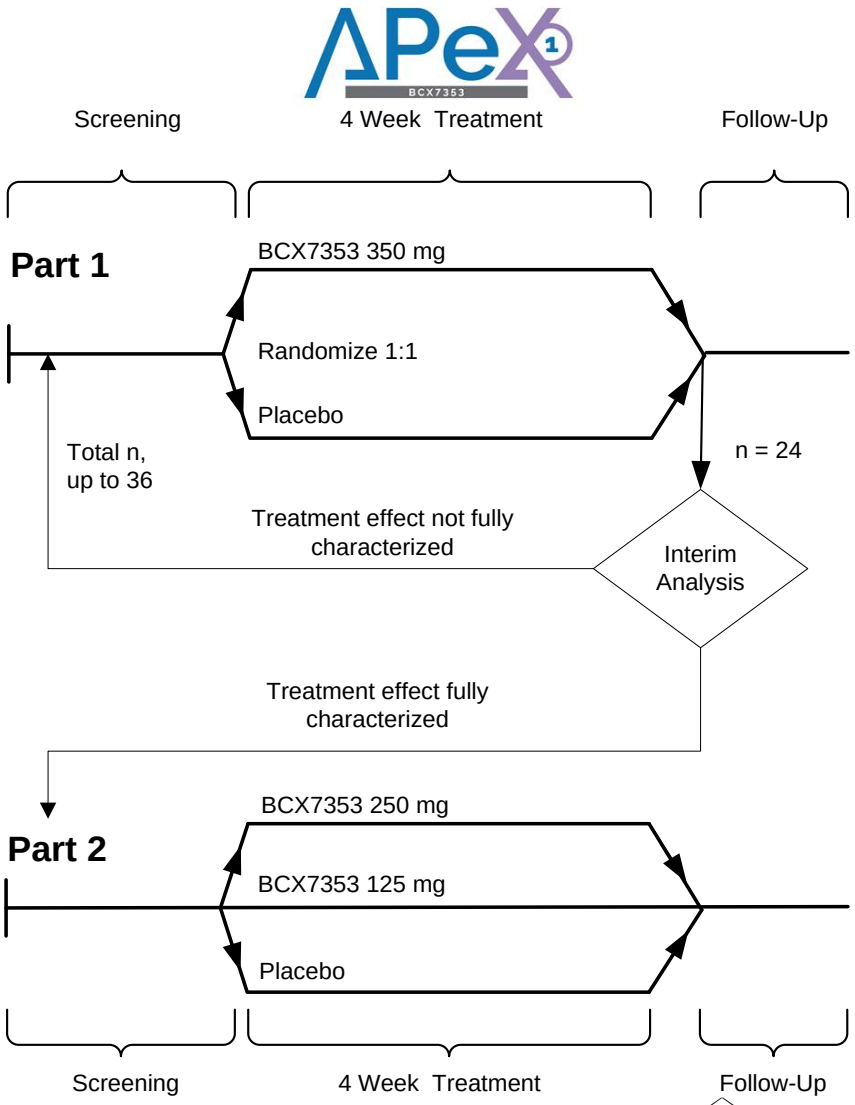
Phase 2 placebo-controlled BCX7353 trial enrolling HAE patients

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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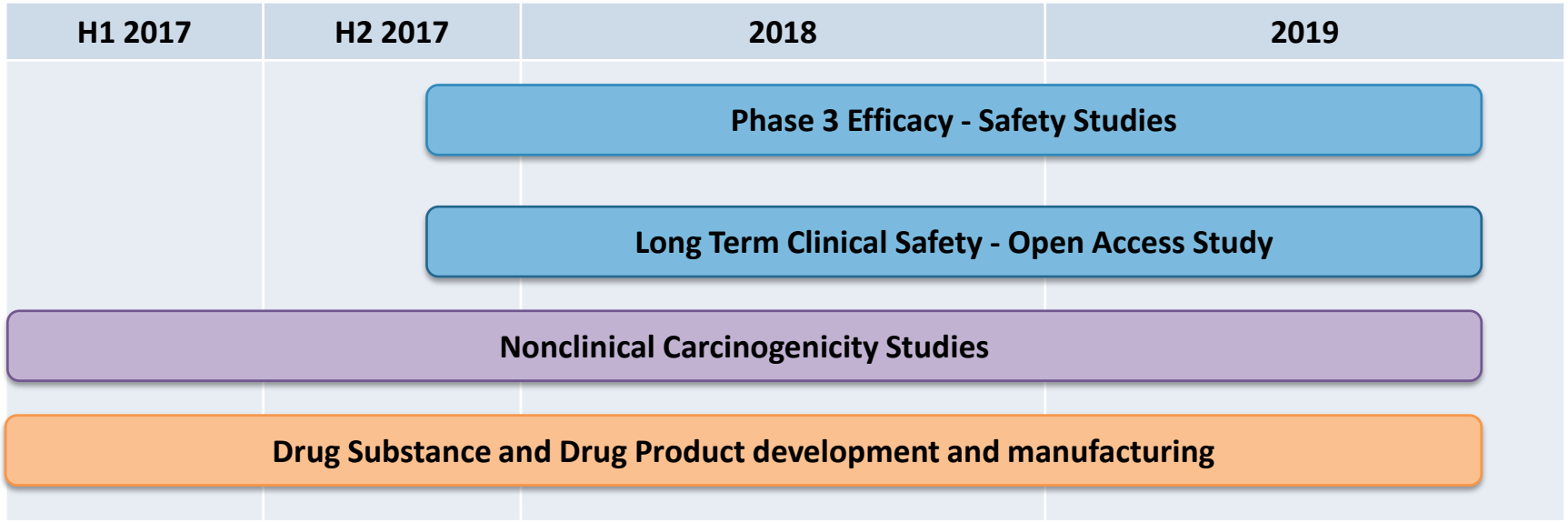
BCX7353 Update

APeX-1 Update*

- 44 patients screened with 5 screen failures
- 34 patients randomized
- Recruiting continues, trial remains blinded, part 1 on track to report results in 1Q17

Estimated timing of key activities to support NDA/MAA filing



*As of January 6, 2017

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Antiviral programs are externally funded, generating $400M to date with the potential for significant future capital infusions

Antiviral Program	Indication	Development funding	Additional capital infusions
	First and only one-dose IV treatment for influenza	Over $200M US Government funding to support development and approval	• Over $90M in milestones and royalty monetization • Over $25M in Government stockpiling (Japan/US)
Galidesivir (BCX4430)	• Ebola is lead indication • Broad-spectrum activity in Zika, Marburg and several other virus families	Approximately $80M US Government contract development funding	• Potential for Government stockpiling prior to FDA approval • Eligible for FDA priority review voucher upon approval

Broad-spectrum activity increases attractiveness of Galidesivir for Government stockpiling

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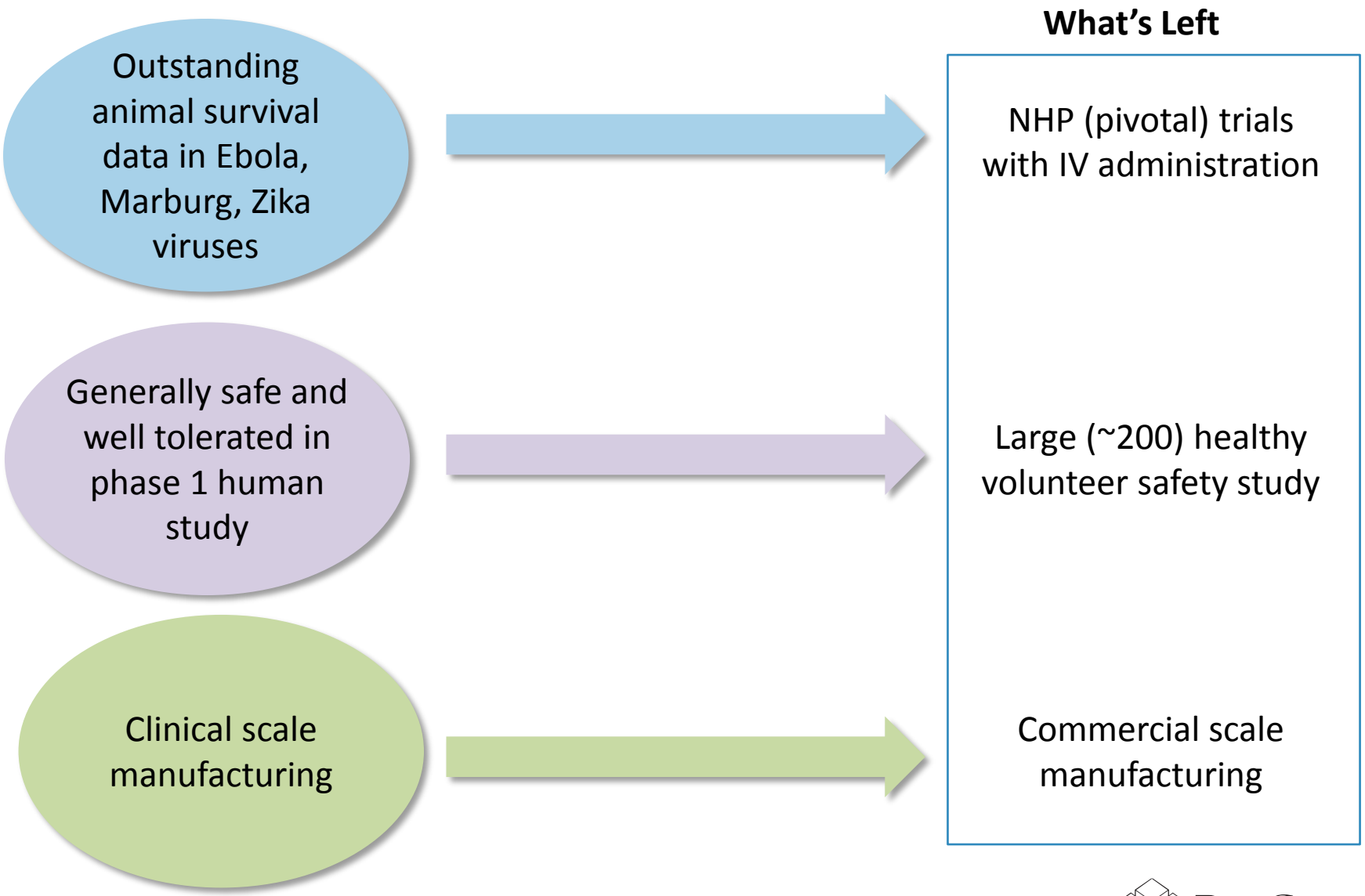
Galidesivir path to stockpiling and NDA



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Stockpiling and Voucher Comparables

Precedent highly pathogenic countermeasures

Product	Pathogen	Company	Doses	Cost
BioThrax vaccine	Anthrax	Emergent BioSolutions	29M	$691M
Raxibacumab antitoxin (CY '13)	Anthrax	GSK	60K	$193M
AbThrax antibody	Anthrax	HGS (now GSK)	65K	$326M
Botulimun antitoxin	Botulism	Cangene	200K	$427M
MVA vaccine	Smallpox	Bavarian Nordic	20M	$505M
ACAM2000 vaccine (CY '08)	Smallpox	Acambis	>72M	$425M-$660M
ST-246 antiviral	Smallpox	Siga	1.7M	$433M

Precedent voucher purchases

Disease	Drug	Seller (Buyer)	Price
Morquio A syndrome	Vimizim (elosulfase alfa)	BioMarin (Sanofi)	$67.5M
Leishmaniasis	Impavido (miltefosine)	Knight (Gilead)	$125M
High-risk neuroblastoma	Unituxin (dinutuximab)	United Therapeutics (Abbvie)	$350M
Rare bile acid synthesis disorders	Cholbam	Retrophin (Sanofi)	$245M

Stockpiling data from FY2014 Budget for Public Health and Social Services Emergency Fund, pg 116
http://www.hhs.gov/budget/fy2014/fy2014-phssef.pdf

Voucher data sourced from public reports

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Cash position & 2016 guidance (in millions)

Cash & investments at December 31, 2015	$101
Cash & investments at December 31, 2016 (unaudited)	**$65**
Senior Credit Facility	$23
Cash runway	Early 2018

Guidance for 2016*:

Operating cash utilization	$55 – 75
Operating expenses#	$68 – 80

* Guidance for 2017 will be provided with our 2016 audited financial results later in Q1 2017
# Excludes equity-based compensation, and represents a modification from the previous range of $78 - 98 million

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Summary: Building a company with the potential to generate expanding and sustainable value