

August 7, 2018



Forward-Looking Statements

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Agenda



- Update on Strategy and Pipeline: Focused on Executing our Strategic Plan
 Jon Stonehouse President, Chief Executive Officer
- ◆ Clinical Update: ZENITH-1, APeX-2 and APeX-S Trials On-track Dr. Bill Sheridan – Chief Medical Officer
- Commercial Update: Significant Commercial Opportunity
 Lynne Powell Chief Commercial Officer
- Financial Update: Strong Financial Position with Cash Runway into 2020
 Thomas Staab Chief Financial Officer
- Summary and Q&A

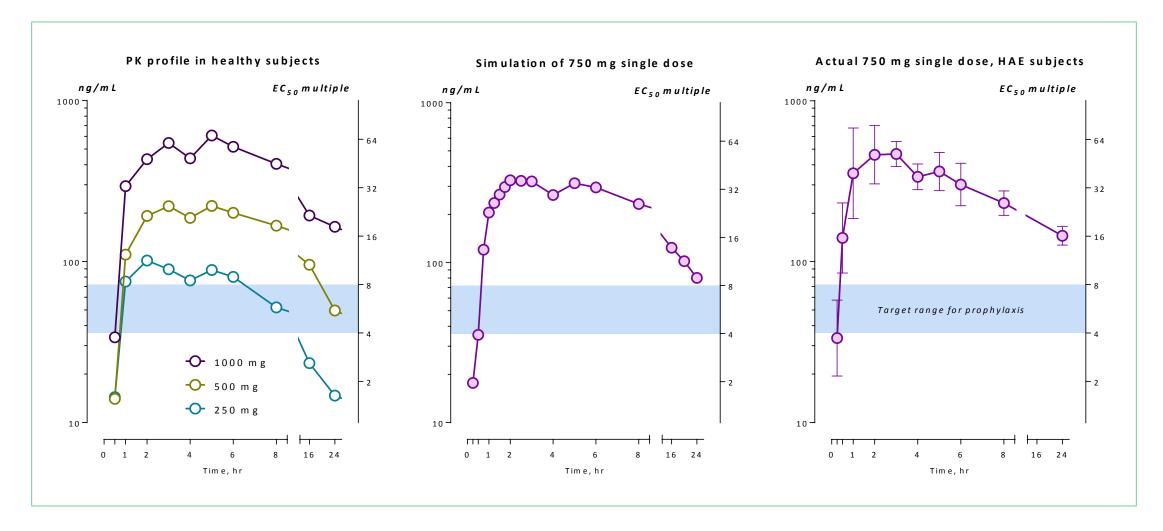


Update on Strategy and Pipeline: Focused on Executing our Strategic Plan



Clinical Update: ZENITH-1, APeX-2, APeX-S Trials On-track

PK profile of single oral dose of BCX7353 supports evaluation as an acute treatment in HAE





Variable design of previous registration trials for acute treatments

| Drug Study | Cinryze¹ CHANGE | Berinert IMPACT-1 | Kalbitor <i>EDEMA-3</i> | Firazyr FAST-3 | Ruconest C-1310 Trial |
|--------------------------------------------|--------------------------------------------------|-------------------------------------------------------------------------------------------------------------------------------------------------------|----------------------------|---------------------|-----------------------------------------------|
| Years subjects enrolled | 2005-2007 | 2005-2007 | 2005-2007 | 2009-2010 | 2011-2012 |
| Route | IV infusion | IV infusion | SC injection | SC injection | IV infusion |
| Duration of symptoms prior to Rx | ≤ 4 hours | ≤ 5 hours | ≤8 hours | 6 to 12 hours | ≤ 5 hours |
| Minimum severity of attack | Moderate | Moderate | Moderate | Moderate | VAS 50mm |
| Location of treatment | Clinic | Clinic | Clinic | Clinic | Clinic |
| Duration of observation by HCP | ≥ 4 hours | ≥ 4 hours | ≥ 4 hours | ≥ 8 hours | 6 hours |
| Treatment administration | НСР | НСР | НСР | НСР | НСР |
| Outcome measure | Symptom severity assessed every 15 minutes | Symptom improvement "Taking into account all of the symptoms you experienced with this HAE attack, are you confident that it is starting to improve?" | Symptom score (TOS) | Symptom score (VAS) | Treatment effect questionnaire (TEQ) |
| Availability of HCP-administered rescue Rx | 2 nd dose of blinded study drug | 2 nd dose of blinded study drug | Opiates, antiemetics | icatibant pdC1NH | rhC1INH icatibant pdC1NH ecallantide |

¹Not approved for the treatment of attacks in the US



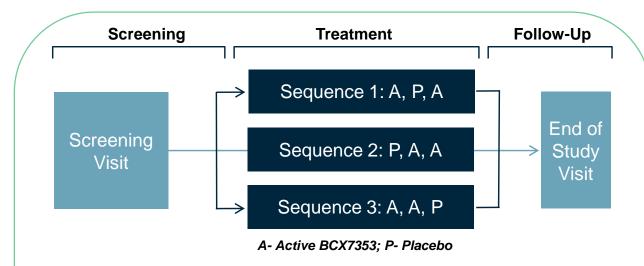
Marketed injectables achieved ~ 16-43% improvement compared with placebo

| Trial Drug, dose | Therapy or Medical Intervention Active - | | L. | Proportion mee at 4 h | % Difference Active - | |
|------------------------------------------------------------|------------------------------------------|-----------------|---------|--------------------------|--------------------------|---------|
| | Active | Placebo | Placebo | Active | Placebo | Placebo |
| CHANGE Cinryze 1000 U IV infusion ¹ (Shire) | 23/35 (66%) | 28/33 (85%) | -19% | 21/35 (60%) | 14/33 (42%) | 18% |
| IMPACT-1 Berinert 20 U/kg IV infusion (CSL) | 8/43 (19%) | 24/42 (57%) | -38% | ~86% | ~59% | ~27% |
| EDEMA-3 Kalbitor 30 mg SC injection (Shire) | 5/36* (14%) | 13/36* (36%) | -22% | ~51% | ~35% | ~16% |
| FAST-3 Firazyr 30 mg SC injection (Shire) | 3/43* (7%) | 18/45* (40%) | -33% | 32/43* (74%) | 14/45* (31%) | ~43% |
| C-1310 Trial Ruconest 50 U/kg IV infusion (Pharming) | 5/44* (13%) | 13/31* (43%) | -30% | ~80% | ~53% | ~27% |

¹Not approved for the treatment of attacks in the US



ZENITH-1 exploratory phase 2 trial aligned with the current guidelines for on-demand treatment^{1,2}



| Trial part | Dose level | N of subjects |
|------------|------------|---------------|
| Part 1 | 750 mg | 36 |
| Part 2 | 500 mg | 12 |
| Part 3 | 250 mg | 12 |
| Total | | 60 |

ZENITH-1 protocol instructions

- Subjects are to call the site PI and treat attacks within 1 hour of symptom onset
- Study drug treatment must be approved by telephone by the site PI
- Subjects wait 4 hours if possible before using HAE medicines, if they feel additional treatment is needed

"Whenever possible and allowed by drugspecific summary product characteristics, patients should have the on-demand medicine to treat acute attacks at home and should be trained to self-administer these medicines."²



¹Zuraw, B. L. et al 2013 <u>J Allergy Clin Immunol Pract</u> **1**(5): 458-467 ²Cicardi, M. et al 2012). <u>Allergy</u> **67**(2): 147-157.

ZENITH-1 is unique

| Drug Study | Cinryze ¹ CHANGE | Berinert IMPACT-1 | Kalbitor EDEMA-3 | Firazyr FAST-3 | Ruconest C-1310 Trial | BCX7353 ZENITH-1 |
|-------------------------------------------------|-----------------------------------------|-----------------------------------------|-------------------------|---------------------|-----------------------------------------------|---------------------------------|
| Years subjects enrolled | 2005-2007 | 2005-2007 | 2005-2007 | 2009-2010 | 2011-2012 | 2017-2018 |
| Route | IV infusion | IV infusion | SC injection | SC injection | IV infusion | PO (liquid) |
| Duration of symptoms prior to Rx | ≤ 4 hours | ≤ 5 hours | ≤8 hours | 6 to 12 hours | ≤ 4 hours | ≤1 hour |
| Location of treatment | Clinic | Clinic | Clinic | Clinic | Clinic | Home |
| Duration of observation by HCP | ≥ 4 hours | ≥ 4 hours | ≥ 4 hours | ≥8 hours | 6 hours | none |
| Treatment administration | НСР | НСР | НСР | НСР | НСР | Patient |
| Availability of self- administered rescue Rx | None | None | None | None | None | icatibant pdC1INH rhC1INH |
| Availability of HCP- administered rescue Rx | Second dose of blinded study drug | Second dose of blinded study drug | Opiates, antiemetics | icatibant pdC1NH | rhC1INH icatibant pdC1NH ecallantide | icatibant pdC1INH rhC1INH |



¹Not approved for the treatment of attacks in the US

ZENITH-1: Multiple efficacy endpoints to allow selection of primary endpoint for phase 3



- At 4 hours:
 - Improved or stable 3-symptom composite visual analogue scale (VAS) score
 - Improved or stable symptoms on patient global assessment
 - No or mild symptoms
- At 24 hours: requiring standard of care attack treatment

Time to:

- Use of standard of care acute attack treatment
- Improved or stable symptoms by composite VAS score
- Symptom relief
- Almost complete symptom relief
- Initial symptom relief
- Complete symptom relief

Multiple opportunities for ZENITH-1 to show clinically meaningful endpoint



BCX7353 phase 2 APeX-1 trial published, phase 3 trial well underway

The NEW ENGLAND JOURNAL of MEDICINE

ORIGINAL ARTICLE

Oral Plasma Kallikrein Inhibitor for Prophylaxis in Hereditary Angioedema

E. Aygören-Pürsün, A. Bygum, V. Grivcheva-Panovska, M. Magerl, J. Graff, U.C. Steiner, O. Fain, A. Huissoon, T. Kinaciyan, H. Farkas, R. Lleonart, H.J. Longhurst, W. Rae, M. Triggiani, W. Aberer, M. Cancian, A. Zanichelli, W.B. Smith, M.L. Baeza, A. Du-Thanh, M. Gompels, T. Gonzalez-Quevedo, J. Greve, M. Guilarte, C. Katelaris, S. Dobo, M. Cornpropst, D. Clemons, L. Fang, P. Collis, W. Sheridan, M. Maurer, and M. Cicardi

Aygoren-Pursun, E. et al 2018 N Engl J Med 379(4): 352-362



Blinded Treatment 24 weeks

 $N \cong 32$ BCX7353 150 mg QD*

N ≅ 32 BCX7353 110 mg QD*

 $N \cong 32$ Placebo QD

Final analysis @ week 24





Commercial Update: Significant Commercial Opportunity

Significant commercial opportunity

Potential to launch new era of oral therapy for HAE

- Large and growing HAE market opportunity
- Prophylactic treatment will drive market growth with acute remaining important
- In a market of predominately injectable therapies, BioCryst is building a highly differentiated portfolio of oral treatments that patients and providers want

Over \$2B global market by launch of BCX7353







Financial Update: Strong Financial Position with Cash Runway into 2020

Second quarter operating results

| (in thousands, except per share amounts) | Q2 2018 | | Q2 2017 | Change Q2 2018 vs Q2 2017 |
|------------------------------------------|----------------|----|----------|------------------------------------|
| Revenues: | | | | |
| Royalty revenue | \$ 142 | \$ | 489 | (71%) |
| Collaborative and other R&D | 12,352 | | 2,610 | 373% |
| Total revenues | 12,494 | - | 3,099 | 303% |
| Expenses: | | | | |
| Research and development | 21,010 | | 15,759 | 33% |
| General and administrative | 9,492 | | 2,834 | 235% |
| Royalty | 243 | | 22 | 1005% |
| Total operating expenses | 30,745 | | 18,615 | 65% |
| Loss from operations | (18,251) | | (15,516) | 18% |
| Interest and other income, net | 493 | | 203 | 143% |
| Interest expense | (2,195) | | (2,094) | 5% |
| Gain on foreign currency derivative | 1,507 | | 521 | 189% |
| Net loss | \$ (18,446) | \$ | (16,886) | 9% |
| Net loss per share - Basic & Diluted | \$ (0.19) | \$ | (0.21) | (10%) |
| Net operating cash utilization | \$ 18,421 | \$ | 12,209 | 51% |
| Weighted average shares outstanding | 98,787 | | 80,418 | |



Cash position & 2018 guidance (in millions)

| Cash & investments at December 31, 2017 | \$159 | |
|-------------------------------------------------|------------------|--|
| Cash & investments at June 30, 2018 | \$122 | |
| Pro-forma cash & investments at June 30, 2018 A | \$186 | |
| Senior Credit Facility ^B | \$30 | |
| FY 2018 GUIDANCE(stand-alone, as revised or | n July 11, 2018) | |
| Operating cash utilization | \$85 — 105 | |
| Operating expenses ^c | \$90 – 110 | |

A – Includes proceeds from the July credit facility enhancement and the August public offering.

C - Excludes equity-based compensation.



B - Credit Facility was enhanced in July 2018.

Thank you... Questions and Answers

August 7, 2018

