

May 8, 2018



### **Forward Looking Statements**

These materials contain forward-looking statements, including statements regarding future results, performance or achievements of BioCryst, and statements regarding the expected benefits of the transactions contemplated by the Agreement and Plan of Merger dated as of January 21, 2018 by and among BioCryst, Idera Pharmaceuticals, Inc. ("Idera") and the other parties thereto (the "merger agreement" and such transactions, the "merger"). These statements involve known and unknown risks, uncertainties and other factors which may cause BioCryst's actual results, performance or achievements to be materially different from any future results, performances or achievements expressed or implied by the forward-looking statements. These statements reflect our current views with respect to future events and are based on assumptions and are subject to risks and uncertainties. Given these uncertainties, you should not place undue reliance on these forward-looking statements. Some of the factors that could affect the forward-looking statements contained herein include: that developing any drug candidate may take longer or may be more expensive than planned; that ongoing and future preclinical and clinical development of candidates may not have positive results; that BioCryst may not be able to enroll the required number of subjects in planned clinical trials of product candidates; that the Company may not advance human clinical trials with product candidates as expected; that the FDA and EMA may require additional studies beyond the studies planned for product candidates, or may not provide regulatory clearances which may result in delay of planned clinical trials, or may impose a clinical hold with respect to such product candidate, or withhold market approval for product candidates; that the Company may not be able to continue development of ongoing and future development programs; that such development programs may never result in future products; that the merger may not be completed on the terms set forth in the merger agreement within the expected time period; that the merger may involve unexpected costs or liabilities; that the announcement of the merger may result in disruption to our business or affect our ability to retain and hire key personnel and maintain business relationships; or that the anticipated benefits of the merger or other commercial opportunities may not be fully realized or may take longer than expected to realize. Please refer to the documents BioCryst files periodically with the Securities and Exchange Commission, specifically BioCryst's most recent Annual Report on Form 10-K, Quarterly Reports on Form 10-Q, and Current Reports on Form 8-K, all of which identify important factors that could cause the actual results to differ materially from those contained in BioCryst's projections and forward-looking statements.



### Agenda

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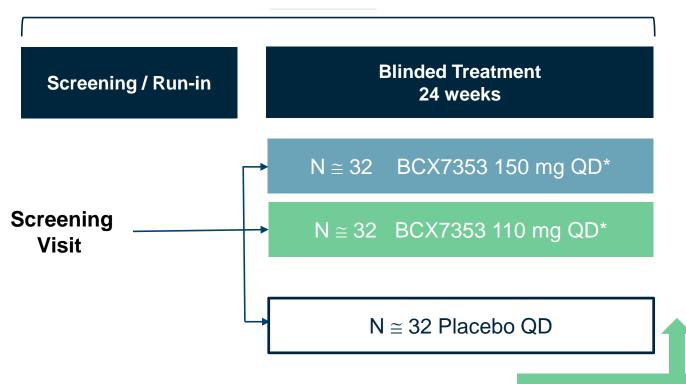
- ◆ Introduction and Program Updates: Executing our Plan Jon Stonehouse – President, Chief Executive Officer
- ◆ Financial Update: Resources to Achieve our Plan
   Thomas Staab Senior Vice President, Chief Financial Officer
- ◆ Update on Proposed Merger with Idera Pharmaceuticals, Inc.
   Jon Stonehouse President, Chief Executive Officer
- ◆ Summary and Q&A



# Introduction and Program Updates: Executing our Plan

## **APeX-2: Phase 3 Trial Design**







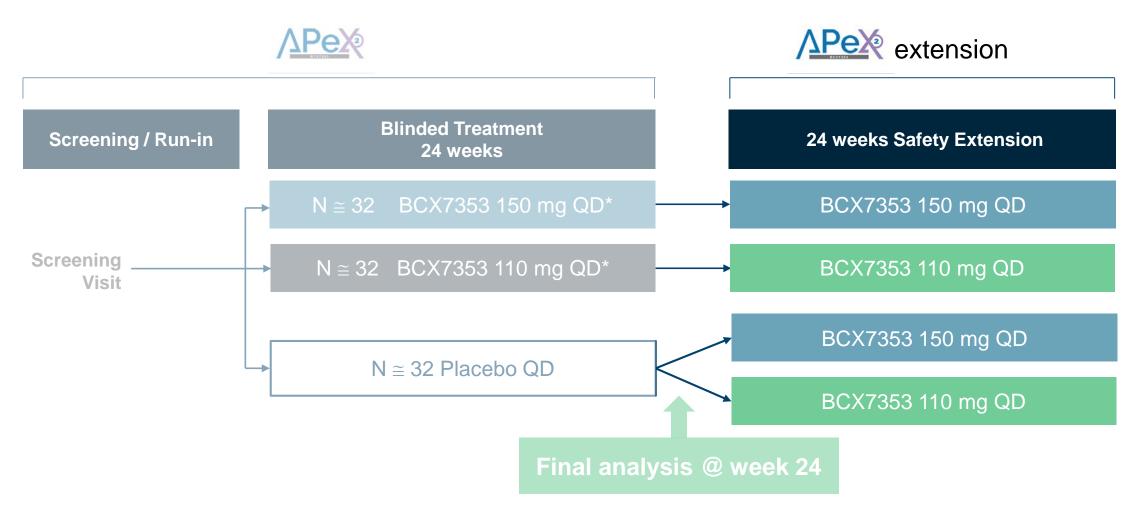
- Primary endpoint at Week 24:
  - Rate of Investigator-confirmed HAE attacks through entire treatment period
- Study powered at >90% to detect a ≥50% reduction in attack rate over placebo

Final analysis @ week 24

\*Doses in Phase 2 APeX-1 were shown as the dihydrochloride salt: 150 mg = 175 mg dihydrochloride salt; 110 mg = 125 mg dihydrochloride salt



# **APeX-2: Phase 3 Trial Design – Safety Extension**



\*Doses in Phase 2 APeX-1 were shown as the dihydrochloride salt: 150 mg = 175 mg dihydrochloride salt; 110 mg = 125 mg dihydrochloride salt



**APeX-S: Long-term Safety Study Design** 





 $N \cong 80 \text{ BCX7353 150 mg QD}$ 

 $N \cong 80 BCX7353 110 mg QD$ 

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Analyses as needed for regulatory submissions

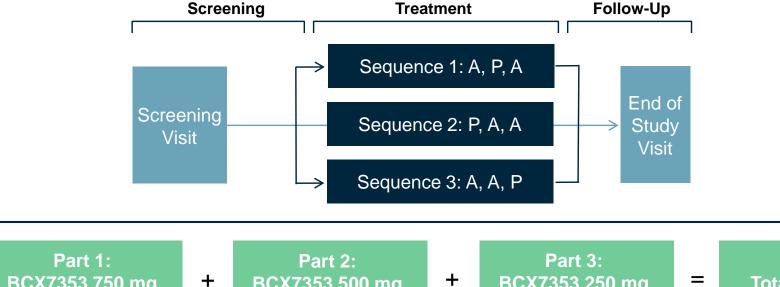




- Endpoints:
  - Long term safety of BCX7353
  - Durability of response
  - Quality of Life
- APeX-1 subjects eligible
- Safety database:
  - Up to 100 subjects at each dose level
  - Combination of APeX-2 extension and APeX-S



### **ZENITH-1: Phase 2 Trial Design – Oral Liquid**



Part 1: BCX7353 750 mg single doses n = 36 Part 2:
BCX7353 500 mg
single doses
n = 12

Part 3:
BCX7353 250 mg
single doses
n = 12

Total n = 60

#### **Primary Efficacy Endpoint:**

 Proportion of subjects with either improved or stable composite visual analog scale (VAS) score at 4 hours post-dose.



# Fibrodysplasia Ossificans Progressiva (FOP) **Devastating Disease; No Treatments Available**



Rare disease that affects approximately 1 in 2 million people worldwide



Irregular formation of bone or ossification in muscles, tendons or soft tissue

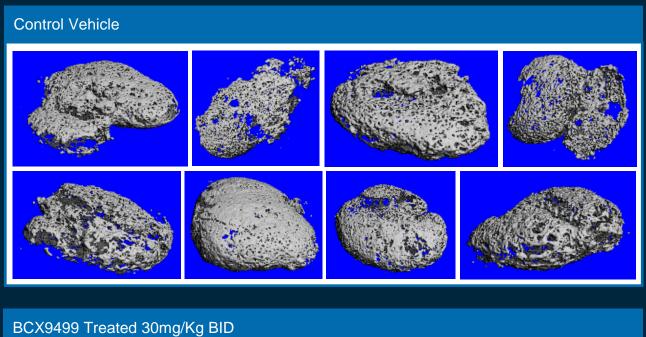


Currently no approved treatments for FOP



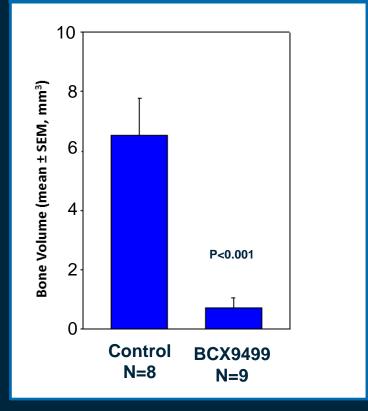
Results in loss of function, deformities and a severely disabling condition

# Advancing ALK2 Inhibitor Program for FOP











# Financial Update: Resources to Achieve our Plan

# First Quarter Operating Results

(in thousands, except per share amounts)	Q1 2018	Q1 2017	Change Q1 2018 vs Q1 2017
Revenues:			
Royalty revenue	\$ 3,661	\$ 6,321	(42%)
Collaborative and other R&D	315	3,116	(90%)
Total revenues	3,976	9,437	(58%)
Expenses:			
Research and development	18,441	16,770	10%
General and administrative	7,609	3,058	149%
Royalty	140	294	(52%)
Total operating expenses	26,190	20,122	30%
Loss from operations	(22,214)	(10,685)	108%
Interest and other income, net	462	109	324%
Interest expense	(2,221)	(2,100)	6%
Loss on foreign currency derivative	(1,804)	(1,543)	17%
Net loss	\$ (25,777)	\$ (14,219)	81%
Net loss per share - Basic & Diluted	\$ (0.26)	\$ (0.19)	37%
Net operating cash utilization	\$ 22,901	\$ 11,376	101%
Weighted average shares outstanding	98,592	75,167	



# Cash Position & 2018 Guidance (In millions)

Cash & investments at December 31, 2017	\$159				
Cash & investments at March 31, 2018	\$138				
Senior Credit Facility	\$22				
FY 2018 GUIDANCE(on a stand-alone basis)					
Operating cash utilization <sup>#</sup>	\$67 – 90				
Operating expenses#	\$85 – 110				

<sup>#</sup> Expected to be in upper-end of range and excludes equity-based compensation.





# Update on Proposed Merger with Idera Pharmaceuticals, Inc.

### **Update on Idera Merger**

#### Combination Creates Substantial Value

Maximizing Value and Market Potential

Robust, Rare Disease Focused Pipeline

Synergistic Discovery Engines

Complementary Leadership

**Financial Strength** 

- Creates a unique player in rare diseases, with scale and strengthened competitive position
- More opportunities for success through diversified late-stage pipeline, variety of early stage programs and supporting assets

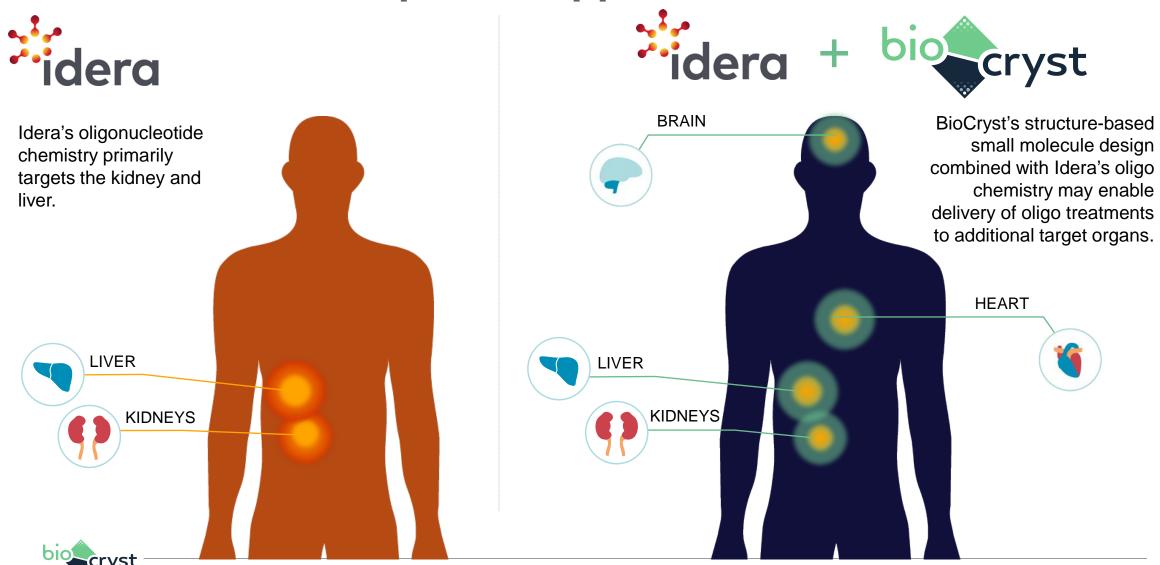
New clinical data on Idera's IMO-2125 Program to be presented at the American Society of Clinical Oncology meeting June 4, 2018

- Synergistic discovery engines with enhanced development opportunities, including through joint small molecule and oligo treatments
- Best-in-class people, facilities and commercial know-how in rare diseases
- Increased financial strength and flexibility through significant cost synergies and opportunities to generate non-dilutive capital

Complementary Assets and Platforms Enhance Market Opportunities and Accelerate Value Creation



# Synergistic Discovery Engines with Enhanced Development Opportunities



# Thank you... Questions and Answers

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