



## BioCryst Provides Update on Strategy, Pipeline and Outlook

July 11, 2018

RESEARCH TRIANGLE PARK, N.C.--(BUSINESS WIRE)--Jul. 11, 2018-- BioCryst Pharmaceuticals, Inc. (NASDAQ:BCRX) ("BioCryst" or the "Company"), a pharmaceutical company focused on the development and commercialization of treatments for rare diseases, today provided an update on the Company's strategic and financial outlook as a standalone company following the recent termination of its merger agreement with Idera Pharmaceuticals, Inc. (NASDAQ:IDRA) ("Idera").

Jon P. Stonehouse, President and Chief Executive Officer of BioCryst, said, "We are moving forward executing our standalone strategy with a great deal of confidence in our programs, our pipeline and our ability to drive value for our stockholders. We have been, and will continue to be, focused on advancing our early- and late-stage programs toward commercial launch and getting new and valuable medicines into the hands of physicians as they treat serious conditions."

Mr. Stonehouse continued, "We are making excellent progress on enrollment in the APeX-2 pivotal trial of BCX7353. We remain on track to read out the results of efficacy and safety from the first 24 weeks of APeX-2 in the first half of next year. Also, we are on track for a first half of 2019 initiation of a Phase 1 clinical trial for our ALK-2 inhibitor program for treating fibrodysplasia ossificans progressiva, or FOP. In addition, we have completed enrollment in all three dose cohorts of the ZENITH-1 proof-of-concept Phase 2 clinical trial. We continue to expect to report top-line results from the 750 mg cohort in the third quarter of 2018 and look forward to the continued progress of each of our ongoing trials."

### Executing a Strategic Plan to Deliver Significant Value for Stockholders

BioCryst continues to execute on its plans to drive growth and create significant value for stockholders by discovering, developing and commercializing novel therapeutics for patients with rare and serious diseases. Through its established expertise in drug discovery and clinical development, and plan to continue to build a highly competitive commercial team, BioCryst is well-positioned to continue advancing its clinical programs toward approval and successful launch.

BioCryst is also focused on generating new compounds from its differentiated small-molecule, rare-disease discovery engine. Over the long-term, the Company will also continue exploring and developing additional complementary organic and strategic opportunities while building on the initiatives underway.

BioCryst has significant near-term milestones representing significant value creation opportunities:

1. Third Quarter of 2018: ZENITH-1 first cohort (750 mg) trial data, a clinical trial to evaluate BCX7353 as an acute treatment of hereditary angioedema attacks ("HAE");
2. First Half of 2019: 24 week efficacy and safety data read out of APeX-2, a Phase 3 clinical trial evaluating two dosage strengths of BCX7353 administered orally once-daily ("QD") as a preventive treatment to reduce the frequency of attacks in patients with HAE; and
3. First Half of 2019: Initiation of ALK-2 inhibitor Phase I clinical trial to treat fibrodysplasia ossificans progressiva.

### 2018 Revised Financial Outlook Due to Merger Costs

Based upon development plans, merger-related incurred costs from the recently terminated merger agreement with Idera and awarded government contracts, BioCryst has revised its stand-alone 2018 guidance and expects its 2018 net operating cash use to be in the range of \$85 to \$105 million, revised from previously issued guidance of \$67 to \$90 million, and its 2018 operating expenses to be in the range of \$90 to \$110 million, revised from previously issued guidance of \$85 to \$110 million. The Company's operating expense range excludes equity-based compensation expense due to the difficulty in reliably projecting this expense, as it is impacted by the volatility and price of the Company's stock, as well as by the vesting of the Company's outstanding performance-based stock options.

### About BioCryst Pharmaceuticals

BioCryst Pharmaceuticals designs, optimizes and develops novel small-molecule medicines that address both common and rare conditions. BioCryst has several ongoing development programs including BCX7353, an oral treatment for hereditary angioedema, galidesivir, a potential treatment for filoviruses, and a preclinical program to develop oral ALK-2 inhibitors for the treatment of fibrodysplasia ossificans progressiva ("FOP"). RAPIVAB® (peramivir injection), a viral neuraminidase inhibitor for the treatment of influenza, is BioCryst's first approved product and has received regulatory approval in the U.S., Canada, Australia, Japan, Taiwan, Korea and the European Union. Post-marketing commitments for RAPIVAB are ongoing. For more information, please visit the Company's website at [www.BioCryst.com](http://www.BioCryst.com).

### Forward-Looking Statements

This press release contains forward-looking statements, including statements regarding future results, performance or achievements. 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 HAE product candidate may take longer or may be more

expensive than planned; that ongoing and future preclinical and clinical development of HAE second generation drug candidates (including ZENITH-1, APeX-2, APeX-J and APeX-S) 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, EMA or other applicable regulatory agency 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 and that actual financial results may not be consistent with expectations, including that 2018 operating expenses and cash usage may not be within management's expected ranges. 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.

BCRXW

View source version on businesswire.com: <https://www.businesswire.com/news/home/20180711005390/en/>

Source: BioCryst Pharmaceuticals, Inc.

BioCryst Pharmaceuticals  
Thomas Staab, 919-859-7910  
Senior Vice President, Chief Financial Officer  
[tstaab@biocryst.com](mailto:tstaab@biocryst.com)