BioCryst Reports First Quarter 2020 Financial Results and Upcoming Key Milestones

—Berotralstat approval/launch timelines in U.S., Japan, EU remain on track—

—Early clinical data supports BCX9930 as oral monotherapy for complement-mediated diseases—

—Patient dosing underway in galidesivir trial in COVID-19 patients in Brazil—

RESEARCH TRIANGLE PARK, N.C. – May 6, 2020 – BioCryst Pharmaceuticals, Inc. (Nasdaq:BCRX) today announced financial results for the first quarter ended March 31, 2020, and provided a corporate update.

"This is a transformational year for BioCryst as we prepare to launch berotralstat in multiple territories to bring our oral, once-daily prophylactic medicine to HAE patients, and begin generating significant revenue," said Jon Stonehouse, president and chief executive officer of BioCryst.

"We are excited to see the clinical response in treatment-naïve PNH patents at the 50 mg and 100 mg twice-daily dose levels, and more than 98 percent suppression of complement in both alternative pathway assays at the 200 mg and 400 mg twice-daily levels in healthy volunteers," said Dr. William Sheridan, chief medical officer of BioCryst.

"This profile provides strong support for BCX9930 as an oral monotherapy. We look forward to studying 200 mg and 400 mg twice-daily in PNH patients and advancing this program to treat multiple complement-mediated diseases," Sheridan added.

Program Updates and Key Milestones

Hereditary Angioedema (HAE) Program – Berotralstat (BCX7353): Oral, once-daily treatment for prevention of HAE attacks

- BioCryst expects three regulatory approvals for berotralstat in 2020 and early 2021. These timelines remain on track.
    - The U.S. Food and Drug Administration (FDA) is currently reviewing a new drug application for berotralstat and has set an action date of December 3, 2020, under the Prescription Drug User Fee Act (PDUFA).
    - In Japan, the Pharmaceuticals and Medical Devices Agency (PMDA) is reviewing a new drug application (JNDA) for berotralstat under the Sakigake timeline, and the company expects approval in Japan in the second half of 2020.

- On March 30, 2020, the company announced that the European Medicines Agency (EMA) had validated its marketing authorization application (MAA) submission for berotralstat and begun their formal review of the MAA under the centralized procedure. An opinion from the Committee for Medicinal Products for Human Use (CHMP) is expected within approximately 12 months from MAA validation.

- Ongoing commercial launch preparations are on track in the U.S., EU and Japan. The company does not expect delays due to COVID-19.

- On May 5, 2020, the company announced that the United States Patent and Trademark Office issued a notice of allowance for a new composition of matter patent which extends patent protection for berotralstat in the U.S. market by four years through October 2039.

Complement Oral Factor D Inhibitor Program – BCX9930

- Low dose cohort (50 mg and 100 mg twice-daily) data in three treatment-naïve paroxysmal nocturnal hemoglobinuria (PNH) patients who completed 28 days of therapy shows BCX9930 inhibited complement and was safe and generally well tolerated.

    - Patients were severely ill with pre-treatment LDH from 3.7 to 11× the upper limit of normal (ULN) and low hemoglobin of 6.0 to 8.2 g/dL.

    - All patients had dose-dependent reductions in LDH and increases in hemoglobin.

    - No drug-related serious adverse events were observed.

    - No PNH patients experienced rash.

    - Based on the investigators' assessment of clinical benefit, all three patients continued on therapy with BCX9930 (100 mg twice-daily) following the 28-day study window.

- With the recent enrollment of a fourth patient with PNH, enrollment is now complete in treatment-naïve cohort 1 (50 mg and 100 mg twice-daily). Treatment-naïve cohort 2 (200 mg and 400 mg twice-daily) is expected to begin enrollment upon completion of cohort 1, with data expected in Q3 2020.

- The company plans to begin enrolling PNH patients resistant to C5 inhibitors in Q3 2020 and expects to report data from these treatment-resistant patients by the end of 2020.

- Data from the 200 mg and 400 mg twice-daily multiple ascending dose (MAD) cohorts in healthy volunteers shows >98 percent suppression of the alternative pathway beyond 12 hours and no dose-limiting adverse events.

Given these data, the company expects to achieve its goal of monotherapy for PNH patients in cohort 2 (200 mg and 400 mg twice-daily).

Additional details can be found on slides, which can be accessed at the Investors' section of BioCryst's website at http://www.biocryst.com.

Coronavirus Antiviral Program – Galidesivir (BCX4430)

- Patient dosing is underway in Brazil in a randomized, double-blind, placebo-controlled clinical trial to assess the safety, clinical impact and antiviral effects of galidesivir in patients with COVID-19. The trial (NCT03891420) is being funded by the National Institute of Allergy and Infectious Diseases (NIAID), part of the National Institutes of Health.

    - Part 1 of the trial is enrolling 24 hospitalized adults diagnosed with moderate to severe COVID-19 confirmed by PCR. Three cohorts of eight patients will be randomized to receive intravenous (IV) galidesivir (n=6) or placebo (n=2) every 12 hours for seven days. Upon completion of part 1 of the trial, an optimized dosing regimen of galidesivir will be selected for part 2 of the trial, based on part 1 results. In part 2 of the trial, up to 42 hospitalized patients with COVID-19 will be randomized 2:1 to receive IV galidesivir or placebo.

- In vitro testing of galidesivir against SARS-CoV-2, the virus that causes COVID-19, is underway. Galidesivir has been shown to be active against more than 20 RNA viruses in nine different families, including coronaviruses.

- The company also is working closely with the government to increase the supply of galidesivir.

Additional Updates

- On April 2, 2020, the company announced the appointment of Anthony Doyle as senior vice president and chief financial officer.

- The company remains on track to report data in 2H 2020 from its ongoing Phase 1 clinical trial of BCX9250, an oral ALK-2 kinase inhibitor for treatment of fibrodysplasia ossificans progressiva (FOP), in healthy subjects.

First Quarter 2020 Financial Results

For the three months ended March 31, 2020, total revenues were $4.8 million, compared to $5.9 million in the first quarter of 2019. The decrease was primarily due to reduced peramivir product sales and lower royalty revenues, partially offset by amortization of deferred revenue from the Torii Pharmaceutical, Co. commercialization agreement.

Research and development (R&D) expenses for the first quarter of 2020 increased to $29.9 million from $27.5 million in the first quarter of 2019, primarily due to due to increased spending on the company's complement-mediated diseases program and other preclinical development initiatives.

Selling, general and administrative (SG&A) expenses for the first quarter of 2020 increased to $15.9 million, compared to $6.2 million in the first quarter of 2019. The increase was primarily due to increased spending on commercial activities and medical affairs to support the U.S. commercial launch of berotralstat in 2020 and contingent legal costs associated with our Seqirus UK Limited (Seqirus) dispute.

Interest and other income were $6.4 million in the first quarter of 2020, compared to $0.6 million in the first quarter of 2019. The increase was primarily due to the partial arbitration award related to our Seqirus dispute.

Interest expense was $3.0 million in the first quarter of 2020, compared to $2.7 million in the first quarter of 2019 and was associated with an increase in the outstanding balance of the company's secured credit facility in February 2019 and increased interest expense associated with the company's non-recourse notes payable.

Net loss for the first quarter of 2020 was $37.6 million, or $0.24 per share, compared to a net loss of $31.1 million, or $0.28 per share, for the first quarter of 2019.

Cash, cash equivalents and investments totaled $114.6 million at March 31, 2020, and reflect a decrease from $137.8 million at December 31, 2019. Operating cash use for the first quarter of 2020 was $23.1 million.

Financial Outlook for 2020

BioCryst expects full year 2020 net operating cash use to be in the range of $125 to $150 million, and its operating expenses to be in the range of $135 to $160 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.

Conference Call and Webcast

BioCryst management will host a conference call and webcast at 8:30 a.m. ET today to discuss the financial results and provide a corporate update. The live call may be accessed by dialing 877-303-8027 for domestic callers and 760-536-5165 for international callers and using conference ID # 4679821. A live webcast of the call and any slides will be available online at the investors section of the company website at www.biocryst.com. A telephone replay of the call will be available by dialing 855-859-2056 for domestic callers or 404-537-3406 for international callers and entering the conference ID # 4679821.

About BioCryst Pharmaceuticals

BioCryst Pharmaceuticals discovers novel, oral, small-molecule medicines that treat rare diseases in which significant unmet medical needs exist and an enzyme plays a key role in the biological pathway of the disease. BioCryst has several ongoing development programs including berotralstat (BCX7353), an oral treatment for hereditary angioedema, BCX9930, an oral Factor D inhibitor for the treatment of complement-mediated diseases, galidesivir, a potential treatment for COVID-19, Marburg virus disease and Yellow Fever, and BCX9250, an ALK-2 inhibitor for the treatment of fibrodysplasia ossificans progressiva. 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.

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 the ongoing COVID-19 pandemic could create challenges in all

aspects of our business, including without limitation delays, stoppages, difficulties and increased expenses with respect to our and our partners' development, regulatory processes and supply chains, could negatively impact our ability to access the capital or credit markets to finance our operations, or could have the effect of heightening many of the risks described below or in the documents we file periodically with the Securities and Exchange Commission; 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 BCX9930, BCX9250 and galidesivir 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 BioCryst may not advance human clinical trials with product candidates as expected; that the FDA, EMA, PMDA 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 candidates, or withhold market approval for product candidates; that actual financial results may not be consistent with expectations, including that 2020 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.

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BIOCRYST PHARMACEUTICALS, INC.
CONSOLIDATED FINANCIAL SUMMARY
(in thousands, except per share)

Statements of Operations (Unaudited)

	Three Months Ended March 31,	
	2020	2019
Revenues:		
Product sales	$ 218	$ 1,679
Royalty revenue	1,945	2,322
Collaborative and other research and development	2,660	1,886
Total revenues	4,823	5,887
Expenses:		
Cost of product sales	-	1,399
Research and development	29,867	27,493
Selling, general and administrative	15,865	6,238
Royalty	69	87
Total operating expenses	45,801	35,217
Loss from operations	(40,978)	(29,330)
Interest and other income	6,446	596
Interest expense	(3,047)	(2,726)
(Loss) gain on foreign currency derivative	(20)	406
Net loss	$ (37,599)	$ (31,054)
Basic and diluted net loss per common share	$ (0.24)	$ (0.28)
Weighted average shares outstanding	154,156	110,167

Balance Sheet Data (in thousands)

	March 31, 2020 (Unaudited)	December 31, 2019 (Note 1)
Cash, cash equivalents and investments	$ 113,058	$ 136,226
Restricted cash	1,559	1,551
Receivables from collaborations	5,642	22,146
Total assets	136,589	175,282
Non-recourse notes payable	29,671	29,561
Senior credit facility	50,539	50,309
Accumulated deficit	(878,227)	(840,628)
Stockholders' equity	3,648	38,252
Shares of common stock outstanding	154,192	154,082

Note 1: Derived from audited financial statements.