BioCryst Reports Third Quarter 2020 Financial Results and Upcoming Key Milestones

November 5, 2020

—ORLADEYO ™ (berotralstat) PDUFA date December 3, 2020—

—Approval decisions expected in Japan in December 2020, EU in early 2021 —

RESEARCH TRIANGLE PARK, N.C., Nov. 05, 2020 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (Nasdaq:BCRX) today announced financial results for the third quarter ended September 30, 2020, and provided a corporate update.

“We are 28 days from our PDUFA date and we are ready to launch ORLADEYO to bring HAE patients the oral, once-daily medicine they have been waiting for to prevent attacks, reduce their burden of therapy and live a normal life,” said Jon Stonehouse, president and chief executive officer of BioCryst.

“In addition to this commercial transformation of the company, and the revenue it brings, our pipeline of BioCryst-discovered molecules continues to advance, with several upcoming near-term data readouts,” Stonehouse added.

Program Updates and Key Milestones

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

- BioCryst expects three regulatory approvals for ORLADEYO in Q4 2020 and early 2021.
    - The U.S. Food and Drug Administration (FDA) is reviewing a new drug application for ORLADEYO and has set an action date of December 3, 2020, under the Prescription Drug User Fee Act (PDUFA).
    - In Japan, ORLADEYO is being reviewed under Sakigake designation. The Pharmaceutical and Medical Devices Agency (PMDA) has confirmed their regulatory review schedule and the company expects a decision on approval in December 2020.
    - On March 30, 2020, the company announced that the European Medicines Agency (EMA) had validated its marketing authorization application (MAA) submission for ORLADEYO and begun its formal review of the MAA under the centralized procedure. The company expects an opinion from the Committee for Medicinal Products for Human Use (CHMP) within approximately 12 months from MAA validation.
- BioCryst has completed the build-out of the commercial infrastructure to support the successful launch of ORLADEYO in the U.S.
    - The company has hired and trained accomplished U.S. rare disease sales and market access teams and has deployed a robust patient services support hub.
    - The company is well-positioned in terms of product supply and inventory on-hand to support the launch and anticipated demand for ORLADEYO.
- On October 30, 2020, the company announced that the United Kingdom’s Medicines and Healthcare Products Regulatory Agency (MHRA) has granted oral, once-daily berotralstat a positive scientific opinion through the Early Access to Medicines Scheme (EAMS). Under the EAMS, HAE patients in the UK aged 12 years and older can gain access to berotralstat for the routine prevention of recurrent attacks of HAE before the drug is granted marketing authorization by the European Commission. Medicines included in the EAMS are those that have a high unmet need, are intended to treat, diagnose or prevent seriously debilitating or life-threatening conditions where there are no adequate treatment options, and are likely to offer significant advantage over methods currently used in the UK.
- On October 28, 2020, the company announced it will present five abstracts and one Distinguished Industry Oral Abstract, including 48-week results from the APeX-2 trial and new data on quality of life and the treatment burden of injectable medication administration, at the upcoming (virtual) Annual Scientific Meeting of the American College of Allergy, Asthma & Immunology on November 13-15.

- On October 22, 2020, the company announced that data from the first 24 weeks of the APeX-2 trial of oral, once-daily berotralstat in patients with HAE have been published online by the *Journal of Allergy and Clinical Immunology*.

Complement Oral Factor D Inhibitor Program – BCX9930

- The company is completing an ongoing dose ranging trial in treatment-naïve paroxysmal nocturnal hemoglobinuria (PNH) patients, and PNH patients with an inadequate response to C5 inhibitors.
    - Seven treatment-naïve PNH patients are currently receiving BCX9930, with four beyond 12 weeks of therapy, including two with more than 32 weeks on therapy. All seven treatment-naïve patients are continuing to benefit from BCX9930 treatment.
    - Based on the excellent results observed to-date at 400 mg bid and 500 mg bid, the company plans to add patients at these dose levels.
    - Because the acceleration of COVID-19 in the EU has slowed start-up of the inadequate responder cohorts, the company expects to report data from treatment-naïve and inadequate C5 responders dosed up to 500 mg bid in the first quarter of 2021.
- On September 30, 2020, the company announced new data from treatment-naïve (no prior treatment with C5 inhibitors) PNH patients receiving doses of oral BCX9930 through 400 mg bid. Oral BCX9930 is driving rapid and dose-dependent reductions in key biomarkers, including LDH, and increasing hemoglobin levels in all PNH patients in the trial. Increases in hemoglobin levels were maintained without transfusions. BCX9930 has been safe and well tolerated at all doses in the trial. No drug-related serious adverse events have been reported.
- On August 31, 2020, the company announced that the FDA has granted Orphan Drug designation for BCX9930, for the treatment of PNH. Orphan Drug designation qualifies BCX9930 for various development incentives, including tax credits for certain clinical costs, a waiver of the new drug application fee and a designated period of market exclusivity following approval.

Coronavirus Antiviral Program – Galidesivir (BCX4430)

- Part 1 of a clinical trial of galidesivir in COVID-19 patients in Brazil has completed enrollment and the company expects to report results in the fourth quarter.
- The primary endpoint of part 1 is safety. Data is also being collected on secondary endpoints, including clinical outcomes and virology. Based on recent conversations with the National Institute of Allergy and Infectious Diseases (NIAID), part of the National Institutes of Health, the major funding partner for the program, the company understands that data from part 1 is a gating item for the program and some evidence of clinical and/or antiviral activity is important for the program to advance.

Additional Updates

- The company remains on track to report data in Q4 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.
- On September 3, 2020, the company announced that the U.S. Department of Health and Human Services (HHS) has exercised its option to purchase an additional 10,000 doses of BioCryst's approved antiviral influenza therapy, RAPIVAB ® (peramivir injection), for approximately $7 million. The order is part of a $34.7 million contract (Contract No. 75D301-18-C-02984) the Centers for Disease Control and Prevention awarded in 2018 for the procurement of up to 50,000 doses of RAPIVAB® (peramivir injection) over a five-year period for the strategic national stockpile.

Third Quarter 2020 Financial Results

For the three months ended September 30, 2020, total revenues were $6.1 million, compared to $1.8 million in the third quarter of 2019. The increase was primarily due to an increase in collaboration revenue under U.S. government development contracts and higher peramivir product sales to our commercial partners.

Research and development (R&D) expenses for the third quarter of 2020 increased to $30.2 million from $25.1 million in the third quarter of 2019, primarily due to increased spending on our complement-mediated diseases and galidesivir programs.

Selling, general and administrative (SG&A) expenses for the third quarter of 2020 increased to $17.2 million, compared to $11.7 million in the third quarter of 2019. The increase was primarily due to increased spending on commercial and medical affairs activities to support the U.S. commercial

launch of ORLADEYO.

Interest expense was $2.9 million in the third quarter of 2020, compared to $3.0 million in the third quarter of 2019.

Net loss for the third quarter of 2020 was $46.1 million, or $0.26 per share, compared to a net loss of $37.6 million, or $0.34 per share, for the third quarter of 2019.

Cash, cash equivalents, restricted cash and investments totaled $148.5 million at September 30, 2020, and reflect an increase from $137.8 million at December 31, 2019. Operating cash use for the third quarter of 2020 was $43.1 million. Net operating cash use for the first nine months of 2020 was $98.0 million, as compared to $77.9 million for the first nine months of 2019.

Financial Outlook for 2020

BioCryst continues to expect full year 2020 net operating cash use to be in the range of $150 to $165 million, and its operating expenses to be in the range of $180 to $195 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 # 3766784. 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 # 3766784.

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 ORLADEYO™ (berotralstat), 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 are identified by use of terms such as "expect," "plan," "anticipate," "will," "may," "project," and similar words, although some forward-looking statements may be expressed differently. 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: the ongoing COVID-19 pandemic, which could create challenges in all aspects of BioCryst's business, including without limitation delays, stoppages, difficulties and increased expenses with respect to BioCryst's and its partners' development, regulatory processes and supply chains, negatively impact BioCryst's ability to access the capital or credit markets to finance its operations, or have the effect of heightening many of the risks described below or in the documents BioCryst files periodically with the Securities and Exchange Commission; developing and commercializing ORLADEYO or any HAE product candidate may take longer or may be more expensive than planned; ongoing and future preclinical and clinical development of BCX9930, BCX9250 and galidesivir may not have positive results; BioCryst may not be able to enroll the required number of subjects in planned clinical trials of product candidates; BioCryst may not advance human clinical trials with product candidates as expected; government contracts contain certain terms and conditions, including termination provisions, that subject BioCryst to additional risks; the FDA, EMA, PMDA or other applicable regulatory agency may require additional studies beyond the studies planned for product candidates, may not provide regulatory clearances which may result in delay of planned clinical trials, may impose certain restrictions, warnings, or other requirements on product candidates, may impose a clinical hold with respect to such product candidates, or may withhold market approval for product candidates; product candidates, if approved, may not achieve market acceptance; BioCryst's ability to successfully commercialize its product candidates, manage its growth, and compete effectively; risks related to the international expansion of BioCryst's business; and 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 September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Revenues:				
Product sales	$ 2,478	$ 335	$ 2,696	$ 2,014
Royalty revenue	254	508	2,243	3,526
Collaborative and other research and development	3,370	932	8,857	3,570
Total revenues	6,102	1,775	13,796	9,110
Expenses:				
Cost of product sales	1,517	-	1,517	1,399
Research and development	30,245	25,120	87,610	80,294
Selling, general and administrative	17,195	11,735	46,943	26,632
Royalty	9	18	78	131
Total operating expenses	48,966	36,873	136,148	108,456
Loss from operations	(42,864)	(35,098)	(122,352)	(99,346)
Interest and other income and expense	(312)	402	8,892	1,545
Interest expense	(2,927)	(3,044)	(8,892)	(8,805)
(Loss) gain on foreign currency derivative	(12)	148	31	331
Net loss	$ (46,115)	$ (37,592)	$ (122,321)	$ (106,275)
Basic and diluted net loss per common share	$ (0.26)	$ (0.34)	$ (0.75)	$ (0.96)
Weighted average shares outstanding	176,521	110,416	164,127	110,308

Balance Sheet Data (in thousands)

	September 30, 2020 (Unaudited)	December 31, 2019 (Note 1)
Cash, cash equivalents and investments	$ 146,321	$ 136,226
Restricted cash	2,213	1,551
Receivables from collaborations	5,422	22,146
Total assets	176,226	175,282
Non-recourse notes payable	29,890	29,561
Senior credit facility	46,041	50,309
Accumulated deficit	(962,949)	(840,628)
Stockholders' equity	33,637	38,252
Shares of common stock outstanding	176,566	154,082

Note 1: Derived from audited financial statements.