BioCryst Reports Second Quarter 2020 Financial Results and Upcoming Key Milestones

August 6, 2020

—ORLADEYO™ (berotralstat) approvals expected in U.S. and Japan in Q4 2020, EU in early 2021—

—200 mg/400mg BCX9930 data from treatment-naïve PNH patients expected in Q3 2020, 200 mg/400mg data from poor-responders to C5 inhibitors expected by year end 2020 —

—Information from Part 1 of galidesivir trial in COVID-19 patients expected by end of Q3 2020—

RESEARCH TRIANGLE PARK, N.C., Aug. 06, 2020 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (Nasdaq:BCRX) today announced financial results for the second quarter ended June 30, 2020, and provided a corporate update.

“BioCryst is currently in an exciting transformation from a company primarily focused on R&D to one that is about to launch its first oral drug to patients with HAE (ORLADEYO) this year, generating meaningful revenue starting next year, with global peak sales potential of greater than half a billion dollars. In parallel, we plan to fill our pipeline with a single molecule, BCX9930, by investing to accelerate this program across multiple rare disease indications based on the proof of concept data we have,” said Jon Stonehouse, president and chief executive officer of BioCryst.

“We expect to end the year with ORLADEYO approved in the U.S. and Japan, additional data at 200 mg and 400 mg with BCX9930 in PNH patients, and clinical data from galidesivir in COVID-19 patients,” 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 an approval decision 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 significant preparations to support the launch of ORLADEYO in the U.S.
    - The company has attracted an accomplished U.S. rare disease sales team, which averages 20 years in pharmaceutical sales and nearly a decade of rare disease experience.
    - The company is well-positioned in terms of product supply and inventory on-hand to support the launch and anticipated global demand for ORLADEYO.
- On June 9, 2020, the company announced that it had established an expanded access program with ORLADEYO for patients with HAE in the United States. Through this program, physicians may be able to request ORLADEYO for HAE patients who do not have access to the product through a clinical trial.
- On June 6, at the European Academy of Allergy and Clinical Immunology (EAACI) Digital Congress, the company presented new data highlighting the burden of therapy faced by HAE patients taking currently available injectable prophylactic medication. Patients taking oral, once daily ORLADEYO experienced sustained decreases in their attack frequency and improvements in quality of life scores over 48 weeks. ORLADEYO was also safe and generally well-tolerated over 48 weeks in both the APeX-2 and APeX-S clinical trials.

“Our ongoing dialogue and research with HAE patients and physicians continue to reinforce their strong demand for an oral, once-daily medicine that is safe and provides the significant and sustained attack reduction we are seeing with ORLADEYO in our clinical program. Nearly half of patients in our APeX-2 and APeX-S trials have prior experience with injectable or infused therapies and most have chosen to remain on ORLADEYO,” said Charlie Gayer, chief commercial officer of BioCryst.

Complement Oral Factor D Inhibitor Program – BCX9930

- On August 3, 2020, the company announced that the FDA has granted Fast Track designation for BCX9930 for the treatment of paroxysmal nocturnal hemoglobinuria (PNH). According to the FDA, the purpose of the Fast Track designation is to get important new drugs to the patient earlier by facilitating the development, and expediting the review of, drugs to treat serious conditions and fill an unmet medical need.

- On May 6, 2020, the company reported proof of concept data for BCX9930 in three treatment-naïve PNH patients in the lowest dose cohort of 50 mg (14 days) and 100 mg (14 days) twice-daily. BCX9930 inhibited complement and was safe and generally well tolerated.

- Based on the investigators’ assessment, three patients receiving 50 mg and 100 mg twice daily experienced clinical benefit, continued on therapy and are now receiving BCX9930 therapy at higher doses. An additional treatment-naïve PNH cohort, which starts at 200 mg (14 days) followed by 400 mg (14 days) twice-daily, has also begun enrollment. The company expects to report data from treatment-naïve PNH patients receiving 200 mg / 400 mg twice daily in Q3 2020.

- The company plans to report data from PNH patients who are poor responders to C5 inhibitors receiving 200 mg / 400 mg twice daily by the end of 2020.

- Based on the safety and proof-of-concept data generated to date in PNH patients, the company is working closely with key opinion leaders in hematology and nephrology to map the development strategy across a broad set of indications.

“We plan to meet with regulators later this year to confirm our advanced development plan for BCX9930 in PNH. Because the alternative pathway of complement is also directly related to several complement-mediated diseases, we plan to broaden and accelerate the BCX9930 program to patients in multiple disease areas as quickly as possible,” said Dr. William Sheridan, chief medical officer of BioCryst.

Coronavirus Antiviral Program – Galidesivir (BCX4430)

- In response to the COVID-19 pandemic, the company is conducting a randomized, double-blind, placebo-controlled clinical trial (NCT03891420) in Brazil to assess the safety, clinical impact and antiviral effects of galidesivir in COVID-19 patients. The company expects to provide information from Part 1 of this ongoing clinical trial by the end of Q3 2020.

- The galidesivir program is being substantially funded by the National Institute of Allergy and Infectious Diseases (NIAID), part of the National Institutes of Health, and the Biomedical Advanced Research and Development Authority (BARDA). The goal of the galidesivir clinical program is to evaluate clinical activity that would support additional government investment and advanced development of galidesivir, including additional drug supply.

Additional Updates

- 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.

Second Quarter 2020 Financial Results

For the three months ended June 30, 2020, total revenues were $2.9 million, compared to $1.4 million in the second quarter of 2019. The increase was primarily due to an increase in collaboration revenue under U.S. government development contracts.

Research and development (R&D) expenses for the second quarter of 2020 were $27.5 million, similar to $27.7 million in the second quarter of 2019. During the second quarter of 2020, R&D spending increased on the company’s complement-mediated diseases and galidesivir programs, which was offset by a reduction in spend on the ORLADEYO program as the company approaches commercial launch.

Selling, general and administrative (SG&A) expenses for the second quarter of 2020 increased to $13.9 million, compared to SG&A expenses of $8.7 million in the second 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 ORLADEYO in 2020.

Interest and other income were $2.8 million in the second quarter of 2020, compared to $0.5 million in the second quarter of 2019. The increase was primarily due to recognition of income from our arbitration proceedings.

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

Net loss for the second quarter of 2020 was $38.6 million, or $0.24 per share, compared to a net loss of $37.6 million, or $0.34 per share, for the second quarter of 2019.

Cash, cash equivalents and investments and restricted cash totaled $191.6 million at June 30, 2020, and reflect an increase from $137.8 million at

December 31, 2019 and $114.6 million at March 31, 2020. Operating cash use for the second quarter of 2020 was $31.8 million.

Financial Outlook for 2020

With the additional capital raised in Q2 and the safety and proof-of concept data generated to-date with BCX9930 in PNH patients, the company is investing in accelerated development of BCX9930 and expects 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 # 5142479. 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 # 5142479.

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, could negatively impact BioCryst’s ability to access the capital or credit markets to finance its operations, or could 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; 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 June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
Revenues:				
Product sales	$ -	$ -	$ 218	$ 1,679

Royalty revenue	44	696	1,989	3,018
Collaborative and other research and development	2,827	752	5,487	2,638
Total revenues	2,871	1,448	7,694	7,335
Expenses:				
Cost of product sales	-	-	-	1,399
Research and development	27,498	27,681	57,365	55,174
Selling, general and administrative	13,883	8,659	29,748	14,897
Royalty	-	26	69	113
Total operating expenses	41,381	36,366	87,182	71,583
Loss from operations	(38,510)	(34,918)	(79,488)	(64,248)
Interest and other income	2,758	547	9,204	1,143
Interest expense	(2,918)	(3,035)	(5,965)	(5,761)
Gain (loss) on foreign currency derivative	63	(223)	43	183
Net loss	$ (38,607)	$ (37,629)	$ (76,206)	$ (68,683)
Basic and diluted net loss per common share	$ (0.24)	$ (0.34)	$ (0.48)	$ (0.62)
Weighted average shares outstanding	161,569	110,338	157,862	110,253

Balance Sheet Data (in thousands)

	June 30, 2020 (Unaudited)	December 31, 2019 (Note 1)
Cash, cash equivalents and investments	$ 189,403	$ 136,226
Restricted cash	2,188	1,551
Receivables from collaborations	3,997	22,146
Total assets	214,706	175,282
Non-recourse notes payable	29,780	29,561
Senior credit facility	50,771	50,309
Accumulated deficit	(916,834)	(840,628)
Stockholders' equity	76,507	38,252
Shares of common stock outstanding	176,429	154,082

Note 1: Derived from audited financial statements.