BioCryst Reports Second Quarter 2021 Financial Results and Upcoming Key Milestones

August 5, 2021

—2Q 2021 revenue of $50.0 million—

—ORLADEYO® (berotralstat) net revenue of $28.5 million—

RESEARCH TRIANGLE PARK, N.C., Aug. 05, 2021 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (Nasdaq:BCRX) today announced financial results for the second quarter ended June 30, 2021, and provided a corporate update.

“BioCryst is in an outstanding position, both near-term and long-term, with growing revenue from a strong ORLADEYO launch in the U.S., more approvals and launches of ORLADEYO around the globe and a pipeline in a molecule with our oral Factor D inhibitor, BCX9930, entering pivotal trials this year in the first of many indications,” said Jon Stonehouse, president and chief executive officer of BioCryst.

Program Updates and Key Milestones

ORLADEYO® (berotralstat): Oral, Once-daily Treatment for Prevention of Hereditary Angioedema (HAE) Attacks

U.S. Launch

“The ORLADEYO launch is off to an excellent start because HAE patients want a safe and effective oral medicine to control their attacks and reduce their burden of therapy, and switching to ORLADEYO meets these needs for them,” said Charlie Gayer, chief commercial officer of BioCryst.

- ORLADEYO net revenue in the second quarter of 2021 was $28.5 million.
- Patient switches continue to drive the launch with 60 percent of patients who were new to ORLADEYO in the second quarter switching from other prophylactic medicine to ORLADEYO and the remainder from acute-only treatment.
- The number of physicians prescribing ORLADEYO grew by approximately 50 percent in second quarter.
- The majority (approximately 70 percent) of HAE patients in the U.S. now have access to ORLADEYO through insurance policies.
- Through the launch thus far, patient retention on therapy remains consistent with the one-year patient retention rate observed in the APeX-2 clinical trial.

ORLADEYO: Global Updates

- On July 10, 2021, the company announced data presented at the European Academy of Allergy and Clinical Immunology Hybrid Congress 2021. HAE patients who were randomized to receive 150 mg of oral, once-daily ORLADEYO at the start of the APeX-2 trial had an 80 percent average reduction in their mean attack rate per month during weeks 25-96 of the trial, compared to baseline. Median attack rates also decreased from 2.7 attacks/month at baseline to 0.0 attacks per month in 16 of 17 months through the same period. ORLADEYO was generally well-tolerated during the treatment period with fewer drug-related adverse events reported in part 3 (weeks 49-96) as compared to part 1 (weeks 0-24) and part 2 (weeks 25-48). Eighty-one percent of the patients who entered part 3 completed the trial.
- On June 16, 2021, the company announced that the Israeli Ministry of Health has accepted the regulatory submission of ORLADEYO for the prevention of recurrent attacks in patients with hereditary angioedema (HAE) 12 years and older. In addition, BioCryst entered into a distribution and supply agreement granting Neopharm Ltd., the exclusive rights to commercialize ORLADEYO in Israel.
- On June 3, 2021, the company announced the launch of ORLADEYO in Germany.
- On May 12, 2021, the company announced that the United Kingdom’s Medicines and Healthcare products Regulatory Agency has granted marketing authorization for ORLADEYO for the routine prevention of HAE attacks in HAE patients 12 years and older.
- On April 30, 2021, the company announced that the European Commission (EC) has approved ORLADEYO for the

prevention of recurrent HAE attacks in HAE patients 12 years and older. The EC approval of ORLADEYO is applicable to all European Union member states plus Iceland, Norway and Liechtenstein.

- On April 14, 2021, the company announced that the Japanese National Health Insurance System (NHI) approved the addition of ORLADEYO to the NHI drug price list on April 21, 2021. This triggered a $15 million milestone payment to BioCryst from Torii Pharmaceutical Co., Ltd., the company's commercial partner in Japan, which BioCryst received and recognized in the second quarter.

Complement Oral Factor D Inhibitor Program – BCX9930

- On June 15, 2021, the company announced the designs for REDEEM-1 and REDEEM-2, two upcoming pivotal trials with its oral Factor D inhibitor, BCX9930, in patients with paroxysmal nocturnal hemoglobinuria (PNH). REDEEM-1 is a randomized, open-label, active, comparator-controlled comparison of the efficacy and safety of BCX9930 (500 mg bid) monotherapy in approximately 81 PNH patients with an inadequate response to a C5 inhibitor. REDEEM-2 is a randomized, placebo-controlled trial to evaluate the efficacy and safety of BCX9930 (500 mg bid) as monotherapy versus placebo in approximately 57 PNH patients not currently receiving complement inhibitor therapy. The primary endpoint for both trials is the change from baseline in hemoglobin, assessed at weeks 12 to 24 in REDEEM-1 and at week 12 in REDEEM-2. Trial site start-up activities are now underway at sites around the world and both pivotal trials are expected to begin enrolling patients in the second half of 2021.

- In the second half of 2021, the company also plans to initiate a proof of concept trial of oral BCX9930 (500 mg bid) in renal complement-mediated diseases. The trial will be a basket study including cohorts of patients with C3 glomerulopathy, IgA nephropathy and primary membranous nephropathy.

Additional Updates

- On July 28, 2021, the company announced the appointment of Vincent Milano to the BioCryst board of directors.

Second Quarter 2021 Financial Results

For the three months ended June 30, 2021, total revenues were $50.0 million, compared to $2.9 million in the second quarter of 2020. The increase was primarily due to $28.5 million in ORLADEYO net revenue in the second quarter of 2021, the recognition of a $15 million milestone payment to BioCryst from Torii Pharmaceutical Co., Ltd., the company's commercial partner in Japan, following approval and successful pricing negotiations in Japan, and $4.6 million for RAPIVAB® (peramivir injection) stockpile sales to the government, all realized in the second quarter of 2021.

Research and development expenses for the second quarter of 2021 increased to $52.9 million from $27.5 million in the second quarter of 2020, primarily due to increased investment in the development of BCX9930 as well as other research, preclinical and development costs, offset by a reduction in spend on the ORLADEYO program following our commercial launch in December 2020.

Selling, general and administrative expenses for the second quarter of 2021 increased to $26.3 million, compared to $13.9 million in the second quarter of 2020. The increase was primarily due to increased investment to support the U.S. commercial launch of ORLADEYO and expanded international operations.

Interest expense was $13.5 million in the second quarter of 2021, compared to $2.9 million in the second quarter of 2020. The increase was due to service on the royalty and debt financings which were completed in December 2020. The interest payment-in-kind (PIK) option on the Athyrium term loan has been exercised and $3.9 million has been added to the $125 million principal in the second quarter of 2021, and $7.5M since issuance.

Net loss for the second quarter of 2021 was $43.2 million, or $0.24 per share, compared to a net loss of $38.6 million, or $0.24 per share, for the second quarter of 2020.

Cash, cash equivalents, restricted cash and investments totaled $222.8 million at June 30, 2021, compared to $191.6 million at June 30, 2020. Operating cash use for the second quarter of 2021 was $22.0 million.

Financial Outlook for 2021

In the launch period for ORLADEYO, the company is not providing specific revenue or operating expense guidance. Based on our expectations for revenue, operating expenses, and our option to access an additional $75 million from our existing credit facility, we believe our current cash runway takes us into 2023.

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 # 9886913. 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 # 9886913.

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. Oral, once-daily ORLADEYO® (berotralstat) is approved in the United States, the

European Union, Japan and the United Kingdom for the prevention of HAE attacks in adults and pediatric patients 12 years and older. BioCryst has several ongoing development programs including BCX9930, an oral Factor D inhibitor for the treatment of complement-mediated diseases, BCX9250, an ALK-2 inhibitor for the treatment of fibrodysplasia ossificans progressiva, and galidesivir, a potential treatment for Marburg virus disease and Yellow Fever. RAPIVAB® (peramivir injection), a viral neuraminidase inhibitor for the treatment of influenza, has received regulatory approval in the U.S., Canada, Australia, Japan, Taiwan and Korea. 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, performance 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; BioCryst's ability to successfully implement its commercialization plans for, and to commercialize ORLADEYO, which could take longer or be more expensive than planned; the results of BioCryst's partnerships with third parties, including Torii, may not meet BioCryst's current expectations; risks related to government actions, including that decisions and other actions, including as they relate to pricing, may not be taken when expected or at all, or that the outcomes of such decisions and other actions may not be in line with BioCryst's current expectations; the commercial viability of ORLADEYO, including its ability to achieve market acceptance, which could also impact the amount of any related royalties BioCryst would be entitled to receive from Torii; 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 products and 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 products and product candidates, may impose a clinical hold with respect to product candidates, or may withhold, delay or withdraw market approval for products and product candidates; product candidates, if approved, may not achieve market acceptance; BioCryst's ability to successfully 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 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

Investors:
John Bluth
+1 919 859 7910
jbluth@biocryst.com

Media:
Catherine Collier Kyroulis
+1 917 886 5586
ckyroulis@biocryst.com

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,	
	2021	2020	2021	2020
Revenues:				
Product sales	$ 33,430	$ -	$ 51,301	$ 218
Royalty revenue	128	44	(769)	1,989
Collaborative and other research and development	16,401	2,827	18,486	5,487
Total revenues	49,959	2,871	69,018	7,694
Expenses:				
Cost of product sales	297	-	6,220	-
Research and development	52,873	27,498	95,308	57,365
Selling, general and administrative	26,325	13,883	48,439	29,748
Royalty	46	-	10	69

Total operating expenses	79,541	41,381	149,977	87,182
Loss from operations	(29,582)	(38,510)	(80,959)	(79,488)
Interest and other income	13	2,758	39	9,204
Interest expense	(13,495)	(2,918)	(26,399)	(5,965)
Gain (loss) on foreign currency derivative	(134)	63	(163)	43
Net loss	$ (43,198)	$ (38,607)	$ (107,482)	$ (76,206)
Basic and diluted net loss per common share	$ (0.24)	$ (0.24)	$ (0.60)	$ (0.48)
Weighted average shares outstanding	178,127	161,569	177,737	157,862

Balance Sheet Data (in thousands)

	June 30, 2021 (Unaudited)	December 31, 2020 (Note 1)
Cash, cash equivalents and investments	$ 218,428	$ 300,366
Restricted cash	4,348	2,221
Receivables	27,620	8,646
Total assets	277,284	334,715
Non-recourse notes payable	30,000	30,000
Secured term loan	126,796	119,735
Royalty financing oblgation	136,862	124,717
Accumulated deficit	(1,130,924)	(1,023,442)
Stockholders' deficit	(106,075)	(19,262)
Shares of common stock outstanding	178,725	176,883

Note 1: Derived from audited financial statements.