BioCryst Reports First Quarter 2021 Financial Results and Upcoming Key Milestones

May 6, 2021

—Q1 2021 ORLADEYO™ (berotralstat) net revenue of $10.9 million—

—ORLADEYO now approved in U.S., Japan and EU—

—BioCryst reaches agreement with FDA that change from baseline in hemoglobin is the primary endpoint for pivotal PNH trials of oral BCX9930 set to begin in second half of 2021—

RESEARCH TRIANGLE PARK, N.C., May 06, 2021 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (Nasdaq:BCRX) today announced financial results for the first quarter ended March 31, 2021, and provided a corporate update.

“Our commercial team is off to an outstanding start with the U.S. launch of ORLADEYO. In this highly competitive market, we are demonstrating what we have known for some time now, HAE patients have been waiting to switch to an oral, once-daily therapy to reduce their attacks and burden of therapy,” said Jon Stonehouse, president and chief executive officer of BioCryst.

“Our early launch performance is the latest piece of evidence that BioCryst’s differentiated strategy to discover, develop and, now, successfully commercialize unique oral medicines for rare diseases has the potential to create greater and greater value. We are doing this first in HAE and will next apply what we have learned to patients suffering from complement-mediated diseases,” Stonehouse added.

Program Updates and Key Milestones

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

- ORLADEYO net revenue in the first quarter of 2021, the first full quarter of launch in the United States, was $10.9 million.
- The majority of ORLADEYO revenue in the first quarter of 2021 came from new patients who switched to ORLADEYO from either injectable/infused prophylactic medications or from acute-only treatment. The remainder came from patients transitioning from clinical trials and the company’s early access program.

European Approvals and Launches

- On April 30, 2021, the company announced that the European Commission (EC) had approved oral, once-daily ORLADEYO for the prevention of recurrent hereditary angioedema (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.
- BioCryst has its European commercial team in place and expects to launch ORLADEYO in the second quarter in Germany, with launches in other European markets to follow. HAE patients in France currently have access to ORLADEYO through an Autorisation Temporaire d'Utilisation de cohorte (cohort ATU).
- On March 2, 2021, the company announced the submission of a marketing authorization application (MAA) to the United Kingdom’s Medicines and Healthcare products Regulatory Agency (MHRA) seeking approval of ORLADEYO for the prevention of recurrent HAE attacks in HAE patients 12 years and older. The MAA was submitted under the MHRA’s new European Commission Decision Reliance Procedure. If approved, ORLADEYO would be the first oral, once-daily therapy in the United Kingdom to treat patients with HAE.

Japanese Approval and Launch

- On January 22, 2021, the company announced that the Ministry of Health, Labor and Welfare (MHLW) in Japan had granted marketing and manufacturing approval for oral, once-daily ORLADEYO 150 mg for prophylactic treatment of HAE in adults and pediatric patients 12 years and older.
- On April 14, 2021, the company announced that the Japanese National Health Insurance System (NHI) approved the addition of oral, once-daily ORLADEYO™ (berotralstat) 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.
- ORLADEYO is the first and only prophylactic HAE medication approved in Japan. Torii launched ORLADEYO in Japan on April 23, 2021. BioCryst will receive tiered royalties ranging from 20 percent to 40 percent of Japanese net sales.

Complement Oral Factor D Inhibitor Program – BCX9930

- BioCryst has reached agreement with the U.S. Food and Drug Administration (FDA) that the primary endpoint for the upcoming pivotal trials in paroxysmal nocturnal hemoglobinuria (PNH) is change from baseline in hemoglobin. On March 22, 2021 the company announced that BCX9930 increased hemoglobin from baseline by a mean of 3.3 g/dL in C5 inadequate response (no prior treatment with C5 inhibitors) patients and 3.5 g/dL in treatment-naïve patients and reduced transfusions in an ongoing dose-ranging trial in PNH patients. BCX9930 was safe and generally well-tolerated in the trial.

- In the second half of 2021, the company plans to advance directly into PNH pivotal trials with oral BCX9930, at a dose of 500 mg bid, in patients naïve to C5 inhibitors, and patients with an inadequate response to C5 inhibitors. The goal of the pivotal trials is to achieve a broad indication for BCX9930 to treat PNH as oral monotherapy. Also in the second half of 2021, the company plans to initiate a proof of concept trial of oral BCX9930 (500 mg bid) in renal complement-mediated diseases.

Additional Updates

- On February 3, 2021, the company announced that the FDA had approved a supplemental new drug application for RAPIVAB® (peramivir injection) expanding the patient population of RAPIVAB for the treatment of acute uncomplicated influenza to include patients six months and older who have been symptomatic for no more than two days. Prior to this approval, RAPIVAB had been indicated for patients two years and older.

- On March 19, 2021, the company announced the appointment of Helen Thackray, M.D., FAAP, to the newly created position of chief research and development officer.

First Quarter 2021 Financial Results

For the three months ended March 31, 2021, total revenues were $19.1 million, compared to $4.8 million in the first quarter of 2020. The increase was primarily due to $10.9 million in ORLADEYO net revenue in the first quarter of 2021.

Research and development (R&D) expenses for the first quarter of 2021 increased to $42.4 million from $29.9 million in the first 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 (SG&A) expenses for the first quarter of 2021 increased to $22.1 million, compared to $15.9 million in the first quarter of 2020. The increase was primarily due to increased investment in to support the U.S. commercial launch of ORLADEYO and expanded international operations.

Interest and other income in the first quarter of 2021 was $6.4 million lower than the first quarter of 2020, primarily due to the partial arbitration award in the first quarter of 2020 related to our Seqirus dispute.

Interest expense was $12.9 million in the first quarter of 2021, compared to $3.0 million in the first quarter of 2020. The increase was due to service on the royalty and debt financings which were completed in December 2020.

Net loss for the first quarter of 2021 was $64.3 million, or $0.36 per share, compared to a net loss of $37.6 million, or $0.24 per share, for the first quarter of 2020.

Cash, cash equivalents, restricted cash and investments totaled $244.4 million at March 31, 2021, compared to $114.6 million at March 31, 2020. Operating cash use for the first quarter of 2021 was $60.0 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 # 2660434. 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 # 2660434.

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, European Union, and Japan for the prevention of HAE attacks in adults and pediatric patients 12 years and older, and under regulatory review for approval in the United Kingdom. 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.

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

Statements of Operations (Unaudited)

	Three Months Ended March 31,	
	2021	2020
Revenues:		
Product sales	$ 17,871	$ 218
Royalty revenue	(897)	1,945
Collaborative and other research and development	2,085	2,660
Total revenues	19,059	4,823
Expenses:		
Cost of product sales	5,923	-
Research and development	42,435	29,867
Selling, general and administrative	22,114	15,865
Royalty	(36)	69
Total operating expenses	70,436	45,801
Loss from operations	(51,377)	(40,978)

Interest and other income	26	6,446
Interest expense	(12,904)	(3,047)
Loss on foreign currency	(29)	(20)
Net loss	$ (64,284)	$ (37,599)
Basic and diluted net loss per common share	$ (0.36)	$ (0.24)
Weighted average shares outstanding	177,343	154,156

Balance Sheet Data (in thousands)

	March 31, 2021 (Unaudited)	December 31, 2020 (Note 1)
Cash, cash equivalents and investments	$ 240,356	$ 300,366
Restricted cash	4,008	2,221
Receivables	18,386	8,646
Total assets	284,431	334,715
Non-recourse notes payable	30,000	30,000
Secured term loan	123,039	119,735
Royalty financing obligation	131,296	124,717
Accumulated deficit	(1,087,726)	(1,023,442)
Stockholders' deficit	(74,988)	(19,262)
Shares of common stock outstanding	177,670	176,883

Note 1: Derived from audited financial statements.