BioCryst Reports First Quarter 2022 Financial Results and Upcoming Key Milestones

May 5, 2022

—Q1 2022 ORLADEYO net revenue of $49.7 million—

—ORLADEYO net revenue in 2022 expected to be no less than $250 million—

—Company provides update on BCX9930 investigation—

RESEARCH TRIANGLE PARK, N.C., May 05, 2022 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (Nasdaq:BCRX) today reported financial results for the first quarter ended March 31, 2022, and provided a corporate update.

"We are now over a year into the ORLADEYO launch and are excited to see strong and continuing patient demand and steady expansion in our prescriber base among both new and existing prescribers. These trends continued in the first quarter of 2022 and reinforce our confidence that we will achieve no less than $250 million in net ORLADEYO revenue in 2022 and peak ORLADEYO sales of $1 billion," said Jon Stonehouse, president and chief executive officer of BioCryst.

"We also have made substantial progress in our investigation with BCX9930. Based on our initial findings, we believe that both dose and dosing regimen could be contributing factors to the safety signal we have observed. By the end of the third quarter, we plan to discuss our proposed approach to resume the REDEEM trials, under a revised dosing protocol, with regulators," Stonehouse added.

Program Updates and Key Milestones

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

U.S. Launch

- ORLADEYO net revenue in the first quarter of 2022 was $49.7 million.
- Approximately 50 percent of patients currently on ORLADEYO have switched from another prophylactic therapy, and half of those patients have come from lanadelumab. These trends continued with new patients starting ORLADEYO in Q1 2022.
- In the first quarter, new patient prescriptions were evenly split between repeat prescribers and new prescribers, with an approximately equal number of new prescriptions coming from the top 500 HAE treaters and the broader set of other HAE treaters.
- The company has completed its latest quarterly survey of another 60 allergists who treat an average of eight HAE patients. These physicians were already using ORLADEYO on 13 percent of their patients and predicted growth to 23 percent over the next 12 months. These findings were in line with previously reported market research from August 2021.
- Approximately 80 percent of HAE patients in the U.S. are insured by payors and pharmacy benefit managers that cover ORLADEYO. Despite prior authorization headwinds early in 2022, approximately 80 percent of patients on ORLADEYO were receiving paid product by the end of the first quarter, a substantial increase from approximately 66 percent of patients in the second half of 2021.
- Most patients are well-controlled on ORLADEYO and remain on therapy. Once payor prior authorization is complete, 78 percent of patients switching from lanadelumab and 73 percent of patients switching from subcutaneous C1 inhibitor remain on ORLADEYO for at least six months. About 70 percent of all patients who receive reimbursed product stay on ORLADEYO for at least 12 months, compared to 60 percent of patients who remain on long-term free product.
- The company expects steady quarterly ORLADEYO net revenue growth throughout the remainder of 2022, with total 2022 ORLADEYO net revenue of no less than $250 million.

"We are very pleased that strong patient demand continued to drive growth in the first quarter and overcome the traditional Q1 reimbursement headwinds. With outstanding reimbursement in place, patients continuing to enjoy an excellent experience on ORLADEYO and the expansion of both our new and existing prescriber bases, we expect steady growth throughout the remainder of 2022 as we achieve no less than $250 million in ORLADEYO net revenues for the year," said Charlie Gayer, chief commercial officer of BioCryst.

ORLADEYO: Global Updates

- ORLADEYO has been launched in Denmark, France, Germany, Japan, Norway, Sweden, the United Arab Emirates and

the United Kingdom. The company expects launches in additional countries throughout the year.

Complement Oral Factor D Inhibitor Program – BCX9930

On April 8, 2022, BioCryst announced that the company was voluntarily pausing enrollment in BCX9930 clinical trials while it investigated observed elevations in serum creatinine seen in some patients.

Patients in the REDEEM-1, REDEEM-2 and RENEW clinical trials randomized to BCX9930 began those trials by starting immediately at a dose of 500 mg twice-daily. Patients receiving BCX9930 in the long-term extension trial started at lower doses as part of the proof-of-concept dose escalation regimen and were ultimately moved up to 500 mg twice-daily.

Preliminary evidence from the investigation points to both the 500 mg twice-daily dosing level and the immediate start of that dose, without a period at a lower dose first, as plausible contributory factors for the observed increases in serum creatinine.

Based on the initial results of the investigation, and the safety and efficacy data observed in the BCX9930 clinical program at 400 mg twice-daily, the company plans to discuss with regulators whether clinical trials with amended protocols could resume using stepped dosing to 400 mg twice-daily. The company expects to have discussions with regulators by the end of the third quarter.

During the ongoing investigation the company has observed the following:

- Three patients with PNH receiving BCX9930 in the REDEEM trials had early onset, and moderate or severe, elevations in their serum creatinine (2-4 xULN) after several weeks of dosing with 500 mg twice-daily. Two of these patients have been discontinued from therapy and one patient (who had the smallest increase in serum creatinine) continues on BCX9930 at this time.
- The company estimates that one-third of subjects randomized to BCX9930 in the REDEEM studies have had early increases in serum creatinine.
- The company also found a different pattern of slowly evolving, late onset, mild to moderate increases in serum creatinine in approximately 40 percent of patients in the long-term extension of the proof-of-concept trial, after those patients switched to the 500 mg twice-daily dose. This pattern was not observed during treatment with doses lower than 500 mg.

Subsequent to BioCryst voluntarily pausing trial enrollments, the U.S. Food and Drug Administration (FDA) informed the company that it has placed the clinical program for BCX9930 on a partial clinical hold. Consistent with BioCryst's voluntary action, the company may not enroll new patients in its BCX9930 clinical trials, however patients already enrolled who are receiving clinical benefit from BCX9930 treatment, and have no other available treatment options, can continue to be dosed and remain in the trials.

"As we complete our investigation, we will continue to be comprehensive and deliberate, with a primary focus on patient safety. After consultation with regulators, we will determine the next step for the BCX9930 program," said Dr. William Sheridan, Chief Medical Officer of BioCryst.

The company does not plan to provide additional updates on the BCX9930 program until it completes additional regulatory discussions and has more clarity on the next steps for the program.

Additional Updates

- On February 8, 2022, the company announced the appointment of Machelle Sanders to its board of directors.

First Quarter 2022 Financial Results

For the three months ended March 31, 2022, total revenues were $49.9 million, compared to $19.1 million in the first quarter of 2021 (+161.3 percent year-over-year (y-o-y)). The increase was primarily due to $49.7 million in ORLADEYO net revenue in the first quarter of 2022, compared to $10.9 million in ORLADEYO net revenue in the first quarter of 2021 (+356.0 percent y-o-y).

Research and development (R&D) expenses for the first quarter of 2022 increased to $65.4 million from $42.4 million in the first quarter of 2021 (+54.2 percent y-o-y), primarily due to increased investment in the development of our Factor D program, including BCX9930, as well as other research, preclinical and development costs.

Selling, general and administrative (SG&A) expenses for the first quarter of 2022 increased to $34.3 million, compared to $22.1 million in the first quarter of 2021 (+55.2 percent y-o-y). The increase was primarily due to increased investment to support the commercial launch of ORLADEYO and expanded international operations.

Interest expense was $23.8 million in the first quarter of 2022, compared to $12.9 million in the first quarter of 2021 (+84.5 percent y-o-y). The increase was due to service on the royalty financings, which were completed in November 2021.

Net loss for the first quarter of 2022 was $74.2 million, or $0.40 per share, compared to a net loss of $64.3 million, or $0.36 per share, for the first quarter of 2021.

Cash, cash equivalents, restricted cash and investments totaled $446.8 million at March 31, 2022, compared to $244.4 million at March 31, 2021. Operating cash use for the first quarter of 2022 was $71.0 million.

Financial Outlook for 2022

Based on the strength of the ORLADEYO launch, and continued steady growth from new patient demand anticipated throughout the year, the company expects full year 2022 net ORLADEYO revenue to be no less than $250 million.

The company had previously expected operating expenses for full year 2022, not including non-cash stock compensation, to be between $440 million to $480 million. Once the company completes its investigation into BCX9930 and has clarity on the next step for the program it expects to provide an updated outlook on full year 2022 operating expenses. If BCX9930 program enrollment resumes, then operating expenses are likely to be at the lower end of the previously provided range. If we discontinue the BCX9930 program, then operating expenses for the year would be lower than that.

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 # 9498023. 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 # 9498023.

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, the United Kingdom and the United Arab Emirates. 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) 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 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; the timing and results of the ongoing investigation described in this press release regarding BCX9930 and of any related discussions with regulators; ongoing and future preclinical and clinical development of BioCryst's Factor D program, 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 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 commercialize its products and 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 revenue, 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,	
	2022	2021
Revenues:		
Product sales	$ 49,546	$ 17,871
Royalty revenue	347	(897)
Collaborative and other research and development	30	2,085
Total revenues	49,923	19,059
Expenses:		
Cost of product sales	236	5,923
Research and development	65,360	42,435
Selling, general and administrative	34,282	22,114
Royalty	2	(36)
Total operating expenses	99,880	70,436
Loss from operations	(49,957)	(51,377)
Interest and other income	54	26
Interest expense	(23,837)	(12,904)
Foreign currency (losses) gains, net	(177)	(29)
Loss before income taxes	(73,917)	(64,284)
Income tax expense	279	-
Net loss	$ (74,196)	$ (64,284)
Basic and diluted net loss per common share	$ (0.40)	$ (0.36)
Weighted average shares outstanding	184,898	177,343

Balance Sheet Data (in thousands)

	March 31, 2022 (Unaudited)	December 31, 2021 (Note 1)
Cash, cash equivalents and investments	$ 443,468	$ 514,430
Restricted cash	3,354	3,345
Receivables	36,415	29,413
Total assets	527,720	588,151
Secured term loan	140,236	136,082
Royalty financing obligation	464,225	449,375
Accumulated deficit	(1,281,700)	(1,207,504)
Stockholders' deficit	(164,215)	(106,986)
Shares of common stock outstanding	185,572	184,350

Note 1: Derived from audited financial statements.