BioCryst Reports Third Quarter 2022 Financial Results and Upcoming Key Milestones

November 1, 2022

—$66.0 million in ORLADEYO Q3 2022 net revenue; on-track to more than double sales in 2022 vs 2021—

—Underlying patient trends remain strong with nine percent paid patient growth in Q3 2022—

—Company expands complement pipeline by advancing second oral Factor D inhibitor, BCX10013, into clinical development with goal of once-daily dosing, and adding discovery targets—

—BCX9930 patient screening underway, data expected from approximately 15 newly-enrolled patients in mid-2023—

RESEARCH TRIANGLE PARK, N.C., Nov. 01, 2022 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (Nasdaq:BCRX) today reported financial results for the third quarter ended September 30, 2022, and provided a corporate update.

“With ORLADEYO on a trajectory to more than double sales in its second year of launch after a very strong first year, we continue to demonstrate that what we are offering HAE patients is unique and fills their need for a therapy that offers both low burden of disease and low burden of treatment. We believe that our ability to successfully pursue challenging targets like plasma kallikrein is just the start, and we expect we will bring rare disease patients many more oral therapies to offer these patients the unique treatment options they are waiting for,” 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 total number of patients on paid therapy grew by nine percent in the third quarter.
- New patient starts remained strong in the third quarter, in line with the running six quarter average.
- The ORLADEYO prescriber base grew by 11 percent in the third quarter.
- These strong underlying growth trends were masked by accelerated direct shipments requested by patients late in the second quarter in June prior to the July 4 holiday weekend, plus a negative out of period reimbursement-related charge that was accounted for in the third quarter.

ORLADEYO: Global Updates

- In the third quarter, ORLADEYO was approved in Saudi Arabia. The company expects continued approvals and launches in additional countries.

“With the strong underlying growth fundamentals we continued to see from patients and physicians in the third quarter, and the further growth dynamics we expect to see in the fourth quarter, we are on track to more than double ORLADEYO revenue in 2022 compared to 2021,” said Charlie Gayer, chief commercial officer of BioCryst.

Complement Program

- **BCX9930 – Oral, Twice-daily Factor D Inhibitor**
  - On August 4, 2022, the company announced that the U.S. Food and Drug Administration (FDA) had lifted its partial clinical hold on the BCX9930 program and that the company will resume enrollment in global clinical trials under revised protocols at a reduced dose of 400 mg twice daily of BCX9930. This includes the REDEEM-1 and REDEEM-2 pivotal trials in patients with paroxysmal nocturnal hemoglobinuria (PNH) and the RENEW proof-of-concept trial in patients with C3 glomerulopathy (C3G), immunoglobulin A nephropathy (IgAN) and primary membranous nephropathy (PMN).
  - Screening has begun for new patients to participate in the trials and the company expects to have data from approximately 15 newly-enrolled patients by the middle of 2023 to inform its decision to either fully invest in the pivotal program, or to discontinue the BCX9930 program.

- **BCX10013 – Oral, Once-daily Factor D Inhibitor with Best-in-class Potential**
    - The company has begun a clinical program with BCX10013, a novel, potent and specific Factor D inhibitor, and expects to report preliminary data from healthy volunteers in the first quarter of 2023. The preclinical and early clinical profile from approximately 90 healthy volunteers to date suggests BCX10013 could have the properties of a once-daily oral therapy. A goal of the ongoing clinical program is to confirm this once-daily profile with healthy volunteer and patient data.

- **Additional Complement Targets**
    - In addition to BCX9930 and BCX10013, which target the alternative pathway of complement, BioCryst is pursuing oral medicines directed at other targets across the classical, lectin and terminal pathways of the complement system. The goal of the company's overall complement program is to advance several oral compounds across multiple pathways in the complement system to treat many complement-mediated diseases.

BCX9250 – Oral ALK-2 Inhibitor for Fibrodysplasia ossificans progressiva (FOP)

- The company believes that patients with FOP, an ultra-rare disease, are likely to benefit from other oral ALK-2 inhibitors that currently are substantially ahead of BCX9250 in development. Considering the expectation that patients will be well-served by these other products, and the approximately $100 million in additional investment that would be required to advance BCX9250 to approval, the company is stopping the BCX9250 program and redirecting this investment to the other opportunities it has to serve patients with complement-mediated diseases.

Third Quarter 2022 Financial Results

For the three months ended September 30, 2022, total revenues were $75.8 million, compared to $41.0 million in the third quarter of 2021 (+85 percent year-over-year (y-o-y)). The increase was primarily due to $66.0 million in ORLADEYO net revenue in the third quarter of 2022.

Research and development expenses for the third quarter of 2022 increased to $52.7 million from $50.0 million in the third quarter of 2021 (+6 percent y-o-y), primarily due to additional investment in the HAE program and expenses for BCX9250 prior to its discontinuation, partially offset by reduced costs related to the BCX9930 studies.

Selling, general and administrative expenses for the third quarter of 2022 increased to $36.9 million, compared to $35.0 million in the third quarter of 2021 (+6 percent y-o-y). The increase was primarily due to increased investment to support the commercial launch of ORLADEYO.

Interest expense was $24.8 million in the third quarter of 2022, compared to $14.1 million in the third quarter of 2021 (+76 percent y-o-y). The increase was due to service on the royalty financings, which were completed in November 2021. The interest payment-in-kind (PIK) option on the Athyrium term loan has been exercised and $6.5 million has been added in the third quarter of 2022 and $32.4 million since issuance, to the $200 million principal.

Net loss for the third quarter of 2022 was $42.5 million, or $0.23 per share, compared to a net loss of $58.8 million, or $0.33 per share, for the third quarter of 2021.

Cash, cash equivalents, restricted cash and investments totaled $462.6 million at September 30, 2022, compared to $203.9 million at September 30, 2021. Operating cash use for the third quarter of 2022 was $29.4 million.

Financial Outlook for 2022

Based on the strength of the ORLADEYO launch through the first three quarters of 2022, the company expects full year 2022 net ORLADEYO revenue to be $255 million.

Based on the reduced spending on the BCX9930 program in the first three quarters of the year, and lower than projected spending on the program for the remainder of the year, the company now expects operating expenses for full year 2022, not including non-cash stock compensation, to be between $365 million and $370 million.

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 866-374-5140 for domestic callers and 404-400-0571 for international callers and using conference ID 28663801#. 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 replay of the call will be available on the company website.

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 and many global markets. BioCryst has active programs to develop oral medicines for multiple targets across the complement system, including oral factor D inhibitors BCX9930 and BCX10013, which are in clinical development. RAPIVAB® (peramivir injection) is approved in the U.S. and multiple global markets, with post-marketing commitments 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; ongoing and future preclinical and clinical development of BCX9930, BCX10013 and other product candidates 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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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 September 30,		Nine Months Ended September 30,	
	2022	2021	2022	2021
Revenues:				
Product sales, net	$ 75,213	$ 39,141	$ 189,647	$ 90,442
Royalty revenue	600	322	1,487	(447)
Milestone revenue	-	-	-	15,000
Collaborative and other research and development	14	1,531	148	5,017
Total revenues	75,827	40,994	191,282	110,012
Expenses:				
Cost of product sales	3,543	591	4,025	6,811
Research and development	52,740	49,971	180,090	145,279
Selling, general and administrative	36,919	34,992	109,218	83,431
Royalty	70	24	73	34
Total operating expenses	93,272	85,578	293,406	235,555
Loss from operations	(17,445)	(44,584)	(102,124)	(125,543)
Interest and other income	1,760	9	2,423	48
Interest expense	(24,775)	(14,115)	(72,634)	(40,514)
Foreign currency losses, net	(538)	(111)	(583)	(274)

Loss before income taxes	(40,998)	(58,801)	(172,918)	(166,283)
Income tax expense	1,522	-	2,657	-
Net loss	$ (42,520)	$ (58,801)	$ (175,575)	$ (166,283)
Basic and diluted net loss per common share	$ (0.23)	$ (0.33)	$ (0.95)	$ (0.93)
Weighted average shares outstanding	186,180	179,106	185,566	178,199

Balance Sheet Data (in thousands)

	September 30, 2022 (Unaudited)	December 31, 2021 (Note 1)
Cash, cash equivalents and investments	$ 461,190	$ 514,430
Restricted cash	1,444	3,345
Receivables	42,610	29,413
Total assets	558,594	588,151
Secured term loan	223,867	136,082
Royalty financing obligation	489,781	449,375
Accumulated deficit	(1,383,079)	(1,207,504)
Stockholders' deficit	(242,659)	(106,986)
Shares of common stock outstanding	186,411	184,350

Note 1: Derived from audited financial statements.