BioCryst Reports Fourth Quarter and Full Year 2021 Financial Results and Upcoming Key Milestones

February 23, 2022

— Q4 2021 ORLADEYO net revenue of $46.2 million and $122.6 million for FY 2021 —

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

— Pivotal trials in PNH and proof-of-concept trial in three renal indications currently enrolling patients —

RESEARCH TRIANGLE PARK, N.C., Feb. 23, 2022 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (Nasdaq:BCRX) today reported financial results for the fourth quarter and full year ended December 31, 2021, and provided a corporate update.

“The successful launch of ORLADEYO, the rapid advancement of our pipeline and the additional capital we acquired last year have transformed BioCryst. With more than $500 million on our balance sheet and ORLADEYO revenue growing to no less than $250 million in 2022, and $1 billion in global peak sales, we are focused on compounding the value of the company by allocating capital to grow ORLADEYO and advancing our complement program to get our oral drugs to patients suffering from many different rare diseases,” 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

- ORLADEYO net revenue in the fourth quarter of 2021 was $46.2 million.
- New patient demand for ORLADEYO remains strong and consistent, with a similar number of new patients added in Q4 2021 as in each of the previous three quarters of the year. Patients switching from other prophylactic therapies and acute-only therapy continue to drive the launch. More than half of patients new to ORLADEYO since launch had a previous prophylactic medicine prior to ORLADEYO and most of the remainder were from acute-only treatment.
- Most patients are well-controlled on ORLADEYO and remain on therapy. Approximately 70 percent of patients starting ORLADEYO, including those switching from injectable prophylaxis, remain on ORLADEYO in the first year.
- The ORLADEYO prescriber base continues to grow significantly as physicians gain real-world experience. In market research, 60 U.S. physicians, who treat an average of seven HAE patients each, reported that they expect to double their use of ORLADEYO, and that ORLADEYO will become their most prescribed prophylactic treatment in the next 12 months.
- ORLADEYO is now covered by all major payors and pharmacy benefit managers, which will lead to more patients moving quickly to paid product.
- ORLADEYO net revenue is expected to benefit from this wide coverage and continued new patient growth throughout 2022. Because of typical first quarter requirements from payors for prescription reauthorization of specialty products, like ORLADEYO, that can temporarily move patients from paid drug to free product, copayment assistance and Medicare D cost sharing dynamics, the company expects little to no ORLADEYO net revenue growth from Q4 2021 to Q1 2022. The company has accounted for this expected Q1 impact in its expectation that full year 2022 ORLADEYO global net revenues will double year over year to no less than $250 million.

“We are excited by the strong start to the ORLADEYO launch and the favorable experience most HAE patients are having controlling their HAE attacks with an oral, once-daily capsule. With a sizeable base of patients already on therapy, reimbursement in place for the full year in 2022, and more face-to-face opportunities for our commercial team to engage directly with patients and physicians, we are looking forward to more than doubling our revenue this year as we continue on our trajectory to become the market leader in HAE prophylaxis,” said Charlie Gayer, chief commercial officer of BioCryst.

ORLADEYO: Global Updates

- ORLADEYO has been launched in 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

- BioCryst is currently enrolling patients in two global pivotal trials, REDEEM-1 and REDEEM-2, with the company's oral Factor D inhibitor, BCX9930 (500 mg bid), 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 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 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.

- The company also has begun patient enrollment for RENEW, a proof-of-concept (PoC) basket trial of oral BCX9930 (500 mg bid) in complement-mediated renal diseases. The trial is being conducted in patients with C3 glomerulopathy (C3G), IgA nephropathy (IgAN), and primary membranous nephropathy (PMN).

- BioCryst plans to further advance and expand its Factor D program over the next two years by achieving the following by the end of 2023:

    - Complete and report data from REDEEM-1 and REDEEM-2
    - Prepare to submit regulatory approval filings in PNH
    - Complete the renal PoC basket trial and advance to pivotal trials in C3G, IgAN and PMN
    - Commence PoC trials in other complement-mediated diseases

Additional Updates

- On November 22, 2021, the company announced financing transactions totaling $350 million in new funding for BioCryst from Royalty Pharma and OMERS Capital Markets.

- On November 1, 2021, the company announced the appointment of Jinky Ang Rosselli as Chief Data and Insights Officer.

Fourth Quarter 2021 Financial Results

For the three months ended December 31, 2021, total revenues were $47.2 million, compared to $4.0 million in the fourth quarter of 2020. The increase was primarily due to $46.2 million in ORLADEYO net revenue in the fourth quarter of 2021.

Research and development (R&D) expenses for the fourth quarter of 2021 increased to $63.5 million from $35.4 million in the fourth quarter of 2020, primarily due to increased investment in the development of our Factor D program and other research, preclinical and development costs, offset by a slight reduction in spend on the ORLADEYO program following our commercial launch in December 2020.

Selling, general and administrative (SG&A) expenses for the fourth quarter of 2021 increased to $35.4 million, compared to $21.0 million in the fourth quarter of 2020. The increase was primarily due to increased investment to support the commercial launch of ORLADEYO and expanded international operations.

Interest expense was $18.8 million in the fourth quarter of 2021, compared to $5.6 million in the fourth quarter of 2020. The increase was due to service on the royalty and debt financings, which were completed in December 2020 and November 2021.

A one-time non-cash gain of $55.8 million related to the extinguishment of debt was recognized during the fourth quarter of 2021 to write-off the non-recourse PhaRMA Notes and related accrued interest payable.

Net loss for the fourth quarter of 2021 was $17.8 million, or $0.10 per share, compared to a net loss of $60.5 million, or $0.34 per share, for the fourth quarter of 2020. Non-GAAP net loss for the fourth quarter of 2021 was $73.6 million, or $0.40 per share when excluding the one-time non-cash gain on the extinguishment of the non-recourse PhaRMA Notes. A reconciliation between GAAP and non-GAAP net loss is provided in the table below.

Cash, cash equivalents, restricted cash and investments totaled $517.8 million as of December 31, 2021, compared to $302.6 million as of December 31, 2020. Operating cash use for the fourth quarter of 2021 was $29.0 million.

Full Year 2021 Financial Results

For the full year ended December 31, 2021, total revenues were $157.2 million, compared to $17.8 million in the full year ended December 31, 2020. The increase was primarily due to $122.6 million of ORLADEYO net revenue following our commercial launch in December 2020, recognition of a $15.0 million milestone payment from Torii related to the Japanese National Health Insurance System approval of ORLADEYO in Japan, and increased RAPIVAB revenues. These increases in revenue were partially offset by a reduction in royalty revenue (excluding those associated with ORLADEYO sales) of $4.2 million, a reduction in contract revenue of $3.3 million and the recognition of $1.9 million of deferred revenue in the prior year period compared to none in the current year period.

R&D expenses in full year 2021 increased to $208.8 million from $123.0 million in full year 2020, primarily due to increased investment in our Factor D program, and an increase in other research, preclinical and development activities, partially offset by a ramp down of clinical investment related to ORLADEYO, which launched commercially in the U.S. during December 2020.

SG&A expenses in full year 2021 increased to $118.8 million, compared to $67.9 million in full year 2020. The increase was primarily due to increased investment to support the U.S. commercial launch of ORLADEYO and expanded international operations.

Interest and other income was $0.1 million in full year 2021, compared to $9.4 million in full year 2020. The decrease was primarily due to the one-time settlement of arbitration proceedings related to our Seqirus dispute in the first quarter of 2020.

Interest expense was $59.3 million in full year 2021, compared to $14.5 million in full year 2020. The increase was associated with the $125.0 million Term A Loan under the Credit Agreement and Royalty financing obligations which were completed in December 2020 and November 2021.

A one-time non-cash gain of $55.8 million on extinguishment of debt was recognized in the full year 2021 related to the write-off of the non-recourse PhaRMA notes and related accrued interest payable.

Net loss for full year 2021 was $184.1 million, or $1.03 per share, compared to a net loss of $182.8 million, or $1.09 per share, for full year 2020. Non-GAAP net loss for full year 2021 was $239.9 million, or $1.34 per share when excluding the one-time gain on the extinguishment of the non-recourse PhaRMA Notes. A reconciliation between GAAP and non-GAAP net loss is provided in the table below.

Non-GAAP Pro forma Financial Measures

The information furnished in this release includes non-GAAP pro forma financial measures that differ from measures calculated in accordance with generally accepted accounting principles in the United States of America ("GAAP"), including financial measures labeled as "non-GAAP" or "adjusted."

We believe providing these non-GAAP measures, which show our pro forma results with these items adjusted, is valuable and useful since they allow the company and investors to better understand the company's financial performance in the absence of these one-time events and will allow investors to more accurately understand our 2021 results and more easily compare them to future results. These non-GAAP pro forma measures also correspond with the way we expect Wall Street analysts to compare our results. Our non-GAAP pro forma measures should be considered only as supplements to, and not as substitutes for or in isolation from, our other measures of financial information prepared in accordance with GAAP, such as GAAP revenue, operating income, net income, and earnings per share.

Our references to our fourth quarter 2021 "non-GAAP pro forma" financial measures of adjusted net loss and adjusted earnings per share constitute non-GAAP financial measures. They refer to our GAAP results, adjusted to show the results without the one-time gain realized by the extinguishment of the debt from our PhaRMA notes.

Financial Outlook for 2022

Based on the strength of the ORLADEYO launch, and continued 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. Operating expenses for full year 2022, not including non-cash stock compensation, are expected to be in the range of $440 million to $480 million. The increase year over year is predominantly driven by additional investment in advancing the Factor D program across multiple indications.

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 # 6365545. 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 # 6365545.

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, including Torii Pharmaceutical Co., Ltd. ("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 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 December 31,		Twelve Months Ended December 31,	
	2021	2020	2021	2020
Revenues:				
Product sales	$ 45,908	$ 605	$ 136,350	$ 3,301
Royalty revenue	347	1,138	(100)	3,381
Milestone revenue	-	-	15,000	-
Collaborative and other research and development	903	2,273	5,920	11,130
Total revenues	47,158	4,016	157,170	17,812
Expenses:				
Cost of product sales	418	33	7,229	1,550
Research and development	63,529	35,354	208,808	122,964
Selling, general and administrative	35,387	20,986	118,818	67,929
Royalty	1	48	35	126
Total operating expenses	99,335	56,421	334,890	192,569
Loss from operations	(52,177)	(52,405)	(177,720)	(174,757)
Interest and other income	14	528	62	9,420
Interest expense	(18,780)	(5,609)	(59,294)	(14,501)
Gain (loss) on extinguishment of debt	55,838	(2,011)	55,838	(2,011)
Foreign currency (losses) gains, net	(421)	(996)	(695)	(965)
Loss before income taxes	$ (15,526)	$ (60,493)	$ (181,809)	$ (182,814)
Income tax expense	2,253	-	2,253	-
Net loss	$ (17,779)	$ (60,493)	$ (184,062)	$ (182,814)
Basic and diluted net loss per common share	$ (0.10)	$ (0.34)	$ (1.03)	$ (1.09)
Weighted average shares outstanding	181,843	176,618	179,117	167,267

Balance Sheet Data (in thousands)

	December 31, 2021 (Unaudited)	December 31, 2020 (Note 1)
Cash, cash equivalents and investments	$ 514,430	$ 300,366
Restricted cash	3,345	2,221
Receivables	29,413	8,646
Total assets	588,151	334,715
Non-recourse notes payable	-	30,000

Secured term loan	136,082	119,735
Royalty financing oblgation	449,375	124,717
Accumulated deficit	(1,207,504)	(1,023,442)
Stockholders' deficit	(106,986)	(19,262)
Shares of common stock outstanding	184,350	176,883

Note 1: Derived from audited financial statements.

Reconciliation of Adjusted Net Income and Adjusted Diluted Earnings Per Share (in thousands)

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2021	2020	2021	2020
GAAP net loss	**$ (17,779)**	**$ (60,493)**	**$ (184,062)**	**$ (182,814)**
Less : One-time Gain on extinguishment of PhaRMA notes	55,838	-	55,838	-
Adjusted net loss	**$ (73,617)**	**$ (60,493)**	**$ (239,900)**	**$ (182,814)**
GAAP basic and diluted net loss per common share	**$ (0.10)**	**$ (0.34)**	**$ (1.03)**	**$ (1.09)**
Adjusted basic and diluted net loss per common share	**$ (0.40)**	**$ (0.34)**	**$ (1.34)**	**$ (1.09)**