BioCryst Reports Fourth Quarter and Full Year 2020 Financial Results and Upcoming Key Milestones

February 25, 2021

—ORLADEYO™ (berotralstat) now approved in U.S. and Japan; EU approval expected in Q2—

—Dose-ranging trial of BCX9930 has fully enrolled 16 PNH patients; Data to be presented at R&D day March 22 —

—Company adds $325 million in financing in Q4 2020; Year-end cash of $303 million provides cash runway into 2023—

RESEARCH TRIANGLE PARK, N.C., Feb. 25, 2021 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (Nasdaq:BCRX) today announced financial results for the fourth quarter and full year ended December 31, 2020, and provided a corporate update.

“With two approvals for ORLADEYO, our launch in the U.S., proof of concept data for BCX9930 in complement-mediated diseases, a successful phase 1 trial of BCX9250 for FOP and the addition of more than $425 million through our May and December financings, 2020 was a transformational year for BioCryst,” said Jon Stonehouse, president and chief executive officer of BioCryst.

“Using our strong balance sheet as a foundation, we expect to continue this transformation in 2021 with ORLADEYO generating revenue in the U.S., Japan and Europe, and the opportunity to significantly advance the 9930 program as an oral monotherapy for both PNH, and renal complement-mediated diseases,” Stonehouse added.

Program Updates and Key Milestones

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

- BioCryst launched ORLADEYO in the United States following U.S. Food and Drug Administration (FDA) approval on December 3, 2020, and product shipments began on December 16, 2020.
- 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 hereditary angioedema (HAE) in adults and pediatric patients 12 years and older.
    - ORLADEYO is the first and only prophylactic HAE medication approved in Japan and will be commercialized in Japan by BioCryst’s partner, Torii Pharmaceutical Co., Ltd. OrphanPacific, Inc. is BioCryst’s representative partner in Japan and holds the marketing authorization.
    - Torii will launch ORLADEYO in Japan following the successful completion of BioCryst’s pricing negotiations with the Japanese National Health Insurance System (NHI).
    - BioCryst is eligible to receive an additional milestone payment of $15 million from Torii upon receipt of a reimbursement price from the NHI in excess of the threshold specified in the agreement with Torii. In addition, BioCryst will receive tiered royalties ranging from 20 percent to potentially 40 percent of Japanese net sales.
- In Europe, the Committee for Medicinal Products for Human Use (CHMP) is scheduled to review the ORLADEYO marketing authorization application this week. The company expects approval from the European Commission (EC) approximately 60 days following a positive opinion from the CHMP.
    - On October 30, 2020, the company announced that the United Kingdom’s Medicines and Healthcare products Regulatory Agency (MHRA) had granted ORLADEYO a positive scientific opinion through the Early Access to Medicines Scheme (EAMS). Under the EAMS, HAE patients in the UK aged 12 years and older can gain access to ORLADEYO for the routine prevention of recurrent attacks of HAE before the drug is granted marketing authorization by the EC.
- In the fourth quarter of 2020, the company presented data in several manuscripts and abstracts.
    - On October 22, 2020, the company announced that data from the first 24 weeks of the Phase 3 APeX-2 trial of ORLADEYO in 121 HAE patients ages 12 years or older had been published online by the *Journal of Allergy and Clinical Immunology*.

- On November 13, 2020, the company presented data in several abstracts at the 2020 Annual Scientific Meeting of the American College of Allergy, Asthma & Immunology across HAE patients, caregivers and treating physicians showing many patients experience a significant treatment burden associated with current prophylactic HAE therapies.

- On November 30, 2020, the company announced that the journal *Allergy* had published data from the APeX-J trial, a randomized, placebo-controlled trial conducted in Japan evaluating ORLADEYO for the prophylactic treatment of HAE.

Complement Oral Factor D Inhibitor Program – BCX9930

- The company has completed the enrollment of its ongoing dose ranging trial in treatment-naïve (no prior treatment with C5 inhibitors) paroxysmal nocturnal hemoglobinuria (PNH) patients, and PNH patients with an inadequate response to C5 inhibitors. The company plans to present data from the 16 enrolled PNH patients (10 treatment naïve and six inadequate C5 responders) at its upcoming R&D day on March 22.

- In previously reported data from treatment-naïve (no prior treatment with C5 inhibitors) PNH patients receiving doses of oral BCX9930 through 400 mg bid, oral BCX9930 drove rapid and dose-dependent reductions in key biomarkers, including LDH, and increasing hemoglobin levels in all PNH patients in the trial. Increases in hemoglobin levels were maintained without transfusions. BCX9930 has been safe and well tolerated at all doses in the trial. No drug-related serious adverse events have been reported.

Additional Updates

- On December 7, 2020, the company announced transactions totaling $325 million in funding for BioCryst, with $250 million available at closing, to support the launch of ORLADEYO in HAE and the development of BCX9930. Royalty Pharma provided BioCryst with an upfront cash payment of $125 million and will receive royalties on direct annual net sales of ORLADEYO up to $550 million, and a tiered percentage of sublicense revenue for ORLADEYO in certain territories. In addition, Royalty Pharma will receive a 1.0% royalty on global net sales of BCX9930, if approved. A fund managed by Athyrium Capital Management provided BioCryst with a $200 million credit facility, of which BioCryst drew $125 million at closing. The additional capital will be available in two tranches at BioCryst's option, upon reaching defined revenue milestones. The credit facility bears interest at LIBOR + 8.25% (with a LIBOR floor of 1.75%) and is interest-only for the entire five-year term, with all outstanding principal due at maturity.

- On December 21, 2020, the company announced that in a Phase 1 clinical trial with BCX9250, an oral activin receptor-like kinase-2 (ALK-2) inhibitor discovered and developed by BioCryst for the treatment of fibrodysplasia ossificans progressiva, BCX9250 was safe and well tolerated at all doses studied, with linear and dose-proportional exposure supporting once-daily dosing.

- 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.

Fourth Quarter 2020 Financial Results

For the three months ended December 31, 2020, total revenues were $4.0 million, compared to $39.7 million in the fourth quarter of 2019. In the fourth quarter of 2019, we recognized revenue of $20.1 related to a one-time upfront milestone per the Torii agreement. Additionally, in that period we recognized $13.9 million of RAPIVAB product sales under our U.S. Department of Health and Human Services (HHS) contract, while in the fourth quarter 2020 we had no RAPIVAB product sales under our HHS contract. ORLADEYO revenues in the fourth quarter of 2020 were $0.1 million.

Research and development (R&D) expenses for the fourth quarter of 2020 increased to $35.4 million from $26.8 million in the fourth quarter of 2019, primarily due to increased investment in our Factor D and galidesivir programs, partially offset by a ramp down of clinical investment related to ORLADEYO, which launched commercially in the U.S. during December 2020.

Selling, general and administrative (SG&A) expenses for the fourth quarter of 2020 increased to $21.0 million, compared to $10.5 million in the fourth quarter of 2019. The increase was primarily due to increased investment in commercial activities to support the U.S. launch of ORLADEYO.

Interest expense was $5.6 million in the fourth quarter of 2020, compared to $3.1 million in the fourth quarter of 2019. This increase was due to service on the royalty and debt financings which were completed in December 2020. As part of those financings, there was also a loss on debt extinguishing related to the closing of our secured credit facility with MidCap Financial.

Net loss for the fourth quarter of 2020 was $60.5 million, or $0.34 per share, compared to a net loss of $2.6 million, or $0.02 per share, for the fourth quarter of 2019.

Cash, cash equivalents, restricted cash and investments totaled $302.6 million at December 31, 2020, and reflect an increase from $137.8 million at

December 31, 2019. Cash, cash equivalents, restricted cash and investments include $250 million in cash received through transactions with Royalty Pharma and Athyrium Capital Management in December 2020. Operating cash use for the fourth quarter of 2020 was $49.9 million, and for the full year of 2020 was $147.9 million.

Full Year 2020 Financial Results

For the full year ended December 31, 2020, total revenues were $17.8 million, compared to $48.8 million in the full year ended December 31, 2019. In the fourth quarter of 2019 we recognized revenue of $20.1 related to a one-time upfront milestone per the Torii agreement, with the remaining amount of $1.9 million recognized in 2020. Additionally, in the fourth quarter of 2019 we recognized $13.9 million of RAPIVAB product sales under our HHS contract, while in the fourth quarter 2020 we had no RAPIVAB product sales under our HHS contract.

R&D expenses in full year 2020 increased to $123.0 million from $107.1 million in full year 2019, primarily due to increased investment in our Factor D and galidesivir programs, 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 2020 increased to $67.9 million, compared to $37.1 million in full year 2019. The increase was primarily due to increased investment in commercial activities to support the U.S. launch of ORLADEYO.

Interest and other income was $9.4 million in full year 2020, compared to $1.9 million in full year 2019. The increase was primarily due to the settlement of arbitration proceedings related to our Seqirus dispute in the first quarter of 2020.

Interest expense was $14.5 million in full year 2020, compared to $11.9 million in full year 2019. This was due to service on the royalty and debt financings which were completed in December 2020. As part of those financings, there was also a loss on debt extinguishing related to the closing of our secured credit facility with MidCap Financial.

Net loss for full year 2020 was $182.8 million, or $1.09 per share, compared to a net loss of $108.9 million, or $0.94 per share, for full year 2019.

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 # 6779206. 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 # 6779206.

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 Japan for the prevention of HAE attacks in adults and pediatric patients 12 years and older, and under regulatory review for approval in the European Union. 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 may be more expensive than planned; the results of BioCryst's partnerships with Torii and OrphanPacific may not meet BioCryst's current expectations; risks related to government actions, including that decisions and other actions relating to pricing and exclusivity of ORLADEYO in Japan 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 December 31,		Twelve Months Ended December 31,	
	2020	2019	2020	2019
Revenues:				
Product sales	$ 605	$ 15,519	$ 3,301	$ 17,533
Royalty revenue	1,138	2,777	3,381	6,303
Collaborative and other research and development	2,273	21,429	11,130	24,999
Total revenues	4,016	39,725	17,812	48,835
Expenses:				
Cost of product sales	33	2,327	1,550	3,726
Research and development	35,354	26,774	122,964	107,068
Selling, general and administrative	20,986	10,489	67,929	37,121
Royalty	48	244	126	375
Total operating expenses	56,421	39,834	192,569	148,290
Loss from operations	(52,405)	(109)	(174,757)	(99,455)
Interest and other income	528	388	9,420	1,933
Interest expense	(5,609)	(3,087)	(14,501)	(11,892)
Loss on debt extinguishment	(2,011)	-	(2,011)	-
(Loss) gain on foreign currency	(996)	186	(965)	517
Net loss	$ (60,493)	$ (2,622)	$ (182,814)	$ (108,897)
Basic and diluted net loss per common share	$ (0.34)	$ (0.02)	$ (1.09)	$ (0.94)
Weighted average shares outstanding	176,618	131,303	167,267	115,600

Balance Sheet Data (in thousands)

	December 31, 2020 (Unaudited)	December 31, 2019 (Note 1)
Cash, cash equivalents and investments	$ 300,366	$ 136,226
Restricted cash	2,221	1,551
Receivables	8,646	22,146
Total assets	334,715	175,282

Non-recourse notes payable	30,000	29,561
Senior credit facility	-	50,309
Secured term loan	119,735	-
Royalty financing obligation	124,717	-
Accumulated deficit	(1,023,442)	(840,628)
Stockholders' (deficit) equity	(19,262)	38,252
Shares of common stock outstanding	176,883	154,082

Note 1: Derived from audited financial statements.

U.S. Indication and Important Safety Information

INDICATION
ORLADEYO™ (berotralstat) is a plasma kallikrein inhibitor indicated for prophylaxis to prevent attacks of hereditary angioedema (HAE) in adults and pediatric patients 12 years and older.

Limitations of use
The safety and effectiveness of ORLADEYO for the treatment of acute HAE attacks have not been established. ORLADEYO should not be used for the treatment of acute HAE attacks. Additional doses or dosages of ORLADEYO higher than 150 mg once daily are not recommended due to the potential for QT prolongation.

IMPORTANT SAFETY INFORMATION
An increase in QT prolongation was observed at dosages higher than the recommended 150 mg once-daily dosage and was concentration dependent.

The most common adverse reactions (≥10% and higher than placebo) in patients receiving ORLADEYO were abdominal pain, vomiting, diarrhea, back pain, and gastroesophageal reflux disease.

A reduced dosage of 110 mg taken orally once daily with food is recommended in patients with moderate or severe hepatic impairment (Child-Pugh B or C) and in patients taking chronically administered P-glycoprotein (P-gp) or breast cancer resistance protein (BCRP) inhibitors (eg, cyclosporine).

Berotralstat is a substrate of P-gp and BCRP. P-gp inducers (eg, rifampin, St. John's wort) may decrease berotralstat plasma concentration, leading to reduced efficacy of ORLADEYO. The use of P-gp inducers is not recommended with ORLADEYO.

ORLADEYO at a dose of 150 mg is a moderate inhibitor of CYP2D6 and CYP3A4. For concomitant medications with a narrow therapeutic index that are predominantly metabolized by CYP2D6 or CYP3A4, appropriate monitoring and dose titration is recommended. ORLADEYO at a dose of 300 mg is a P-gp inhibitor. Appropriate monitoring and dose titration is recommended for P-gp substrates (eg, digoxin) when coadministering with ORLADEYO.

The safety and effectiveness of ORLADEYO in pediatric patients <12 years of age have not been established.

There are insufficient data available to inform drug-related risks with ORLADEYO use in pregnancy. There are no data on the presence of berotralstat in human milk, its effects on the breastfed infant, or its effects on milk production.

To report SUSPECTED ADVERSE REACTIONS, contact BioCryst Pharmaceuticals, Inc. at 1-833-633-2279 or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.