# BioCryst Announces Preliminary Full Year 2021 ORLADEYO® (berotralstat) Net Revenue and Provides Full Year 2022 ORLADEYO Net Revenue and Peak Sales Guidance

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***—ORLADEYO preliminary net revenue of $45.6 million for Q4 2021 and $122 million for FY 2021—***

***—ORLADEYO net revenue expected to more than double in 2022 to no less than $250 million; Company expects ORLADEYO to become the market leader in HAE prophylaxis therapy with peak sales of $1 billion—***

***—Enrollment of PNH patients has begun in REDEEM-1 and REDEEM-2 pivotal trials of BCX9930, oral Factor D inhibitor for complement-mediated diseases—***

RESEARCH TRIANGLE PARK, N.C., Jan. 10, 2022 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (Nasdaq:BCRX) today announced preliminary, unaudited ORLADEYO® (berotralstat) revenue for the fourth quarter and full year 2021 and provided new guidance for full year 2022 ORLADEYO net revenue and expected peak ORLADEYO sales.

“Following 12 months of a successful launch through a global pandemic, we have a clear picture of the continued commercial trajectory for ORLADEYO based on a very attractive product profile, leading to strong patient demand to switch from injectable therapies to our oral, once-daily medicine, with 70 percent patient retention through the first year. Building on our substantial 2021 patient base, we are confident that ORLADEYO will achieve no less than $250 million of net revenue in 2022 and that ORLADEYO will become the market leader as the most prescribed prophylactic therapy with peak sales of $1 billion,” said Jon Stonehouse, president and chief executive officer of BioCryst.

**Fourth Quarter 2021 (Q4 2021) ORLADEYO Launch Dynamics**

- Preliminary, unaudited ORLADEYO net revenue in Q4 2021 was $45.6 million. Preliminary, unaudited ORLADEYO net revenue for full year 2021 (FY 2021) was $122 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.
- ORLADEYO is now covered by all major payors and national and regional pharmacy benefit managers, which will lead to more patients being reimbursed quickly.
- The ORLADEYO prescriber base continues to grow significantly. The number of new physicians prescribing ORLADEYO in Q4 2021 was similar to the number added in Q3 2021. 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.

“We expect ORLADEYO revenues in 2022 to more than double in our second year of launch as we benefit from a full year of reimbursement and continued strong demand from patients and physicians. ORLADEYO is transforming the lives of HAE patients, which is why ORLADEYO is on a trajectory to become the market leader in HAE prophylaxis,” said Charlie Gayer, chief commercial officer of BioCryst.

**Pipeline Update: BCX9930 Pivotal Trials in PNH Now Enrolling**

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). The company also has begun screening patients in a proof of concept (PoC) basket trial of BCX9930 (500 mg bid) 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:

- 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

“Following the discovery, development and commercialization of ORLADEYO, the BioCryst team plans to repeat this success as we leverage our platform to bring new oral medicines to patients suffering from other rare diseases. BCX9930 is especially exciting because the clinical data we have reported so far provides confidence that we can help patients in PNH, and across many complement-mediated diseases, with this pipeline in a molecule,” said Dr. Helen Thackray, chief research and development officer of BioCryst.

**Presentation Today at 40th Annual J.P. Morgan Healthcare Conference**

Today at 9:45 a.m. ET, the company will present at the 40th Annual J.P. Morgan Healthcare Conference, which is being conducted as a virtual event. Links to a live audio webcast and replay of the presentation may be accessed in the Investors section of BioCryst’s website at https://www.biocryst.com/.

**About ORLADEYO® (berotralstat)**

ORLADEYO® (berotralstat) is the first and only oral therapy designed specifically to prevent attacks of hereditary angioedema (HAE) in adult and pediatric patients 12 years and older. One capsule of ORLADEYO per day works to prevent HAE attacks by decreasing the activity of plasma kallikrein.

**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 percent 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.**

**Please see full Prescribing Information.**

**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 preliminary, unaudited net revenue results and 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 preliminary, unaudited net revenue results and 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: BioCryst’s completion of its customary closing, review and audit procedures for the fourth quarter and full year 2021, which may cause actual net revenue results for these periods to differ materially from the preliminary, unaudited net revenue results; 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 NewBridge Pharmaceuticals and 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 BCX9930 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 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 future 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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