BioCryst Presents New Long-term Data Demonstrating Consistently High Attack-free Status Among Hereditary Angioedema Patients with ORLADEYO® (berotralstat)

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Long-term prophylaxis with ORLADEYO continues to help patients achieve consistently high attack-free days regardless of gender, age and prior HAE therapy after 96 weeks of treatment

RESEARCH TRIANGLE PARK, N.C., April 27, 2023 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (Nasdaq: BCRX) today announced new data from the APeX-S clinical trial, which evaluated oral, once-daily ORLADEYO® (berotralstat) for the prophylactic treatment of hereditary angioedema (HAE), showing sustained reduction in disease burden for patients across multiple subgroups through 96 weeks of treatment. The data are being presented at the 13th C1-inhibitor Deficiency & Angioedema Workshop, which is being held in Budapest, Hungary, from May 4-7, 2023.

"We continue to generate evidence that further strengthens confidence in ORLADEYO as a safe, effective and more convenient therapeutic option for people living with HAE. These data demonstrate how our oral, once-daily prophylactic treatment can consistently help patients spend more of their days without disruption from HAE attacks, regardless of their age, gender and experience with prior prophylactic treatment, including among pediatric patients aged 12-17. These additional analyses of long-term data reflect our continued commitment to define the potential benefits and bring ORLADEYO to as many HAE patients around the world as possible," said Dr. Ryan Arnold, chief medical officer of BioCryst.

BioCryst C1-inhibitor Deficiency & Angioedema Workshop Presentation Highlights

The oral presentation at the C1-inhbitor Deficiency & Angioedema Workshop will include post-hoc analyses from the APeX-S clinical study. APeX-S was a Phase 2, open label, international study evaluating the safety and effectiveness of ORLADEYO 110 mg and 150 mg once daily (QD) in patients with HAE Type I or Type II for up to 96 weeks in the United States and 240 weeks in all other countries. Overall, treatment-emergent adverse events (TEAEs) reported in APeX-S were mild and transient, indicating that ORLADEYO was generally well tolerated.

- ***Attack-free status across subgroups of patients with hereditary angioedema after 96 weeks of berotralstat treatment: results from the APeX-S trial*; Saturday, May 6, 8:00-8:15 am CEST**
    - This analysis assessed the attack-free status of patients receiving ORLADEYO 150 mg through 96 weeks in APeX-S (n=287), stratified by baseline age, gender, and prior HAE prophylaxis treatment. The three subgroups included patients who were 12-17 (n=23), 18-64 (n=253) and ≥ 65 years of age (n=11), female (n=180) and male (n=107) and had prior experience with androgens (n=142) or C1-inhibitors (n=105).
    - Overall, a reduction in mean adjusted HAE attack rates was observed compared to Weeks 0-24 in patients treated with ORLADEYO 150 mg QD. Mean adjusted HAE attack rates decreased from 1.08 from Weeks 0-24 (n=287) to 0.69 and 0.59 from Weeks 25-48 (n=214) and Weeks 49-96 (n=158), respectively.
    - Attack-free status was consistently high through 96 weeks of treatment with ORLADEYO 150 mg regardless of patients' age, gender and prior prophylactic treatment.
        - Patients aged 12-17, 18-64 and ≥ 65 remained attack free an average of 97, 94 and 98 percent of days, with a mean (SEM) of 196.8 (30.4), 99.3 (9.5) and 275.9 (104.4) days and a maximum duration of 461, 1,101 and 1,182 days between attacks, respectively.
        - Female patients remained attack free an average of 94 percent of days, with a mean (SEM) of 106 (11.8) days and a maximum duration of 1,182 days between attacks. Male patients remained attack free an average of 94 percent of days, with a mean (SEM) of 127.2 (17.4) days and a maximum duration of 1,101 days between attacks.
        - Patients who had prior treatment with androgens remained attack free an average of 93 percent of days, with a mean (SEM) of 87.1 (12.7) days and a maximum duration of 1,026 days between attacks. Patients who had prior treatment with C1-inhibitors remained attack free an average of 91 percent of days, with a mean (SEM) of 75.8 (9.8) days and a maximum duration of 584 days between attacks.
    - Long-term prophylaxis with ORLADEYO 150 mg led to a durable treatment effect and sustained reduction in disease burden through 96 weeks of treatment regardless of baseline characteristics including patients' age, gender and prior prophylactic treatment.

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% 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 and many global markets. BioCryst has active programs to develop oral medicines for multiple targets across the complement system, including BCX10013, an oral Factor D inhibitor 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 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 periodically files 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 commercial viability of ORLADEYO, including its ability to achieve market acceptance; 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; 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 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, which identify important factors that could cause actual results to differ materially from those contained in BioCryst's forward-looking statements.

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