BioCryst Presents New ORLADEYO® (berotralstat) Data at 7th Bradykinin Symposium

September 6, 2024

RESEARCH TRIANGLE PARK, N.C., Sept. 06, 2024 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (Nasdaq: BCRX) today announced the presentation of six posters, including the first interim real-word evidence from the APeX-N trial, and new data highlighting the value of shared decision making (SDM) between healthcare providers (HCPs) and their hereditary angioedema (HAE) patients to provide optimal patient outcomes.

The company is presenting the posters at the 7th Bradykinin Symposium in Berlin from September 5-6, 2024.

- ***APeX-N interim results: Oral berotralstat for HAE prophylaxis in Europe***
- ***HCP and patient perspectives: HAE long-term prophylaxis and shared decision-making***
- ***Adverse health outcomes and perspectives of androgen use in HAE***
- ***Evaluation of adherence to berotralstat in patients with hereditary angioedema***
- ***Tolerability and effectiveness of berotralstat for long-term prophylaxis in HAE***
- ***Effectiveness and safety of berotralstat in HAE with normal C1-inhibitor***

APeX-N interim results

APeX-N is a European multi-center observational study assessing the safety (primary objective), effectiveness and quality of life (secondary objectives) of berotralstat 150 mg in routine clinical use. This interim analysis included 56 patients from the United Kingdom, France, Germany and Sweden.

Non-serious gastrointestinal adverse events were reported in 12.5 percent of patients. Seven percent (n=4) of patients discontinued treatment (three due to unsatisfactory response, one to participate in a clinical trial). One patient had a severe HAE attack but continued treatment.

“These initial data from APeX-N in Europe reinforce and closely replicate the clinical trial and real-world evidence of berotralstat as the first oral prophylaxis for HAE,” said Dr. Sorena Kiani, consultant immunologist at Royal Free London NHS Foundation.

HCP and patient perspectives: HAE long-term prophylaxis and shared decision-making

This study, conducted in Germany, explored the dynamics between HCPs and patients in HAE management, identifying barriers to SDM and strategies to improve it. Ten HCPs participated in 60 minute interviews and simulated patient consultations. Eight HAE patients participated in 30 minute interviews. Participants then convened in structured focus groups to discuss their findings.

The participants identified a need for enhanced HCP awareness of patient perspectives, more comprehensive HCP-patient conversations and improved education about HAE treatment management.

“The findings from this study support the need for further insights to develop future guidance and HAE management strategies to facilitate successful shared decision making and improved patient quality of life,” said Dr. Emel Aygören-Pürsün, department for children and adolescents, University Hospital Frankfurt, Germany.

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).

Berotralstat is a substrate of P-glycoprotein (P-gp) and breast cancer resistance protein. 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 is a global biotechnology company with a deep commitment to improving the lives of people living with complement-mediated and other rare diseases. BioCryst leverages its expertise in structure-guided drug design to develop first-in-class or best-in-class oral small-molecule and protein therapeutics to target difficult-to-treat diseases. BioCryst has commercialized ORLADEYO® (berotralstat), the first oral, once-daily plasma kallikrein inhibitor, and is advancing a pipeline of small-molecule and protein therapies. For more information, please visit www.biocryst.com or follow us on LinkedIn.

Forward-Looking Statements

This press release contains forward-looking statements, including statements regarding BioCryst's plans and expectations for ORLADEYO. 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: BioCryst's ability to successfully implement or maintain its commercialization plans for ORLADEYO, which could take longer or be more expensive than planned; the commercial viability of ORLADEYO, including its ability to achieve sustained 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; and risks related to the international expansion of BioCryst's business. 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.

BCRXW

Contacts:
John Bluth
+1 919 859 7910
jbluth@biocryst.com

Niamh Lyons
+353 87 639 7083
nlyons@biocryst.com