

**UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION  
Washington, D.C. 20549**

**FORM 8-K**

**CURRENT REPORT**

**Pursuant to Section 13 or 15(d)  
of the Securities Exchange Act of 1934**

**Date of Report (Date of earliest event reported): February 25, 2021**

**BIOCRYS T PHARMACEUTICALS, INC.**  
(Exact name of registrant as specified in its charter)

**Delaware**  
(State or Other Jurisdiction of Incorporation)

**000-23186**  
(Commission File Number)

**62-1413174**  
(I.R.S. Employer Identification No.)

**4505 Emperor Blvd., Suite 200  
Durham, North Carolina 27703**  
(Address of Principal Executive Offices) (Zip Code)

**(919) 859-1302**  
(Registrant's telephone number, including area code)

(Former name or former address, if changed since last report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock	BCRX	Nasdaq Global Select Market

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

**Item 7.01. Regulation FD Disclosure.**

On February 25, 2021, BioCryst Pharmaceuticals, Inc. (the "Company") issued a press release announcing that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) has adopted a positive opinion recommending the approval of ORLADEYO™ (berotralstat) for routine prevention of recurrent attacks of hereditary angioedema (HAE) in adult and adolescent patients aged 12 years and older. A copy of the press release is furnished as Exhibit 99.1 hereto and is incorporated herein by reference.

The information in this Current Report on Form 8-K shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference into any filing made by the Company under the Securities Act of 1933, as amended, or the Exchange Act, except as expressly set forth by specific reference in such filing.

**Item 9.01. Financial Statements and Exhibits.**

(d) Exhibits

<u>Exhibit</u> <u>No.</u>	<u>Description</u>
<u>99.1</u>	<u><a href="#">Press release dated February 25, 2021 entitled "BioCryst Receives Positive CHMP Opinion for ORLADEYO™ (berotralstat), an Oral, Once-daily Therapy to Prevent Attacks in Patients with Hereditary Angioedema"</a></u>
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

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**SIGNATURE**

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

**BioCryst Pharmaceuticals, Inc.**

Date: February 25, 2021

By: /s/ Alane Barnes

Alane Barnes

Senior Vice President and Chief Legal Officer

## **BioCryst Receives Positive CHMP Opinion for ORLADEYO™ (berotralstat), an Oral, Once-daily Therapy to Prevent Attacks in Patients with Hereditary Angioedema**

*For investors and media only*

RESEARCH TRIANGLE PARK, N.C., Feb. 25, 2021 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (Nasdaq: BCRX) today announced that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) has adopted a positive opinion recommending the approval of ORLADEYO™ (berotralstat) for routine prevention of recurrent attacks of hereditary angioedema (HAE) in adult and adolescent patients aged 12 years and older.

The European Commission (EC) will review the CHMP recommendation and a final approval decision from the EC on the marketing authorization application (MAA) for ORLADEYO is expected in the second quarter.

If approved, ORLADEYO would be the first oral, once-daily therapy in the European Union to treat patients with HAE by preventing recurrent attacks. The CHMP positive opinion is based on data from the pivotal APeX-2 clinical trial and supporting data from the APeX-S trial. In APeX-2, ORLADEYO met its primary endpoint ( $p < 0.001$ ) for ORLADEYO 150 mg compared to placebo. ORLADEYO showed a positive safety profile and was generally well-tolerated over 48 weeks in both APeX-2 and APeX-S.

“Following the recent approvals in the U.S. and Japan, we continue to focus on bringing our oral, once-daily treatment to HAE patients around the world,” said Jon Stonehouse, president and chief executive officer of BioCryst. “The positive CHMP opinion for ORLADEYO is an important step closer to delivering a new option to HAE patients across Europe and our commercial team is in place and ready to launch quickly upon final EC approval.”

In December 2020, the U.S. Food & Drug Administration (FDA) approved ORLADEYO in the U.S. In January 2021, ORLADEYO was approved by the Ministry of Health, Labour and Welfare in Japan. An Early Access to Medicines Scheme (EAMS) for HAE patients has been approved by the Medicines & Healthcare products Regulatory Agency in the United Kingdom.

### **About ORLADEYO™ (berotralstat)**

ORLADEYO™ (berotralstat) is the first and only oral therapy designed specifically to prevent attacks of hereditary angioedema (HAE) in adults 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 ( $\geq 10\%$  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](http://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 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](http://www.biocryst.com).

### **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, performances 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, 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; 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 forward-looking statements.

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