

**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): December 21, 2020**

**BIOCRYSST 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 December 21, 2020, BioCryst Pharmaceuticals, Inc. (the “Company”) issued a press release announcing that in a Phase 1 clinical trial with BCX9250, an oral activin receptor-like kinase-2 (ALK-2) inhibitor discovered and developed by the Company for the treatment of fibrodysplasia ossificans progressiva (FOP), BCX9250 was safe and well tolerated at all doses studied, with linear and dose-proportional exposure supporting once-daily dosing. A copy of the press release is furnished as Exhibit 99.1 hereto and is incorporated herein by reference.

The information in this Item 7.01, including Exhibit 99.1, 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 Exchange Act or the Securities Act of 1933, as amended, except as expressly set forth by specific reference in such filing.

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

(d) Exhibits

**Exhibit****No.****Description**

<a href="#">99.1</a>	<a href="#">Press release dated December 21, 2020 entitled “BioCryst Announces Positive Phase 1 Results with BCX9250, an Oral ALK-2 Inhibitor for Treatment of Fibrodysplasia Ossificans Progressiva”</a>
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: December 21, 2020

By: /s/ Alane Barnes

Alane Barnes

Senior Vice President and Chief Legal Officer

## BioCryst Announces Positive Phase 1 Results with BCX9250, an Oral ALK-2 Inhibitor for Treatment of Fibrodysplasia Ossificans Progressiva

RESEARCH TRIANGLE PARK, N.C., Dec. 21, 2020 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (Nasdaq: BCRX) today 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 (FOP), BCX9250 was safe and well tolerated at all doses studied, with linear and dose-proportional exposure supporting once-daily dosing.

FOP is an ultra-rare, severely disabling condition characterized by the irregular formation of bone outside the normal skeleton, also known as heterotopic ossification (HO). HO can occur in muscles, tendons and soft tissue. Patients with FOP become bound by this irregular ossification over time, with restricted movement and fused joints, resulting in deformities and premature mortality. There are currently no approved treatments for FOP.

The randomized, double-blind, placebo-controlled dose-ranging trial evaluated safety, tolerability and pharmacokinetics of single ascending doses (SAD) and multiple ascending doses (MAD) of BCX9250 in healthy subjects.

The SAD study was designed to randomize four cohorts of eight subjects each to receive oral BCX9250 (n=6) or placebo (n=2) at dose levels of 5 mg, 10 mg, 15 mg and 25 mg. Subjects in the 15 mg cohort also received a second single dose to evaluate food effect on absorption of BCX9250.

The MAD study was designed to randomize four cohorts of 12 subjects each to receive oral BCX9250 (n=10) or placebo (n=2) at dose levels of 5 mg, 10 mg, 15 mg and 20 mg once daily (QD) for seven days.

Drug exposure increased with dose in an approximately linear and dose-proportional manner. Drug levels after a high fat meal were similar to those after dosing on an empty stomach. Drug exposure (area under the curve) at 20 mg QD in the MAD was similar to that achieved with doses that suppressed HO in a nonclinical model of activity of orally dosed BCX9250. Additional data can be found in slides in the investors section of the company's website at <https://ir.biocryst.com/>.

In both the SAD and the MAD studies, oral BCX9250 was safe and well tolerated, with no serious adverse events, no study discontinuations due to adverse events, no grade 3 or 4 adverse events and no clinically significant changes in vital signs, electrocardiograms or safety laboratory parameters. No safety signals were seen.

"FOP is a devastating condition with no approved treatments. These encouraging Phase 1 results provide hope for patients and their families, and we look forward to speaking with expert physicians, patient advocates and regulators about the next steps to advance the program," said Dr. Bill Sheridan, chief medical officer of BioCryst.

In preclinical studies, BCX9250 demonstrated potency for the target kinase, selectivity, safety and strong suppression of HO in animal models.

### About BCX9250

Discovered by BioCryst, BCX9250 is a novel, oral, inhibitor of the ALK-2 enzyme. The ALK-2 enzyme is a part of the normal signaling pathway for bone formation and responds to binding its specific ligands (bone morphogenic proteins, BMPs) by stimulating normal bone growth and renewal in healthy children and adults. Specific activating mutations of the ALK-2 gene are seen in all cases of FOP. An activating mutation in ALK-2 is necessary for the disease to occur, making the ALK-2 enzyme an ideal drug target for treatment of FOP.

### 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 for the prevention of HAE attacks in adults and pediatric patients 12 years and older, and under regulatory review for approval in Japan and the European Union. BioCryst has several ongoing development programs including BCX9930, an oral Factor D inhibitor for the treatment of complement-mediated diseases, galidesivir, a potential treatment for COVID-19, Marburg virus disease and Yellow Fever, and BCX9250, an ALK-2 inhibitor for the treatment of fibrodysplasia ossificans progressiva. RAPIVAB® (peramivir injection), a viral neuraminidase inhibitor for the treatment of influenza, is BioCryst's first approved product and 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 for its BCX9250 program. These statements involve known and unknown risks, uncertainties and other factors which may cause actual results and developments of such program to be materially different from those 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; ongoing and future preclinical and clinical development of BCX9250 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 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 product candidates, may impose a clinical hold with respect to such product candidates, or may withhold market approval for product candidates; product candidates, if approved, may not achieve market acceptance; BioCryst's ability to successfully develop and commercialize its product candidates, 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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