BioCryst Reports First Quarter 2025 Financial Results and Provides Business Update

May 5, 2025

—Q1 2025 ORLADEYO net revenue of $134.2 million (+51 percent y-o-y)—

—Full year 2025 ORLADEYO revenue guidance increased to $580 million to $600 million—

—Company now expects to be profitable for full year 2025, a year ahead of schedule—

—Company pays down $75 million of debt; saves approximately $23.5 million over life of loan—

—NDA submitted for ORLADEYO oral granules for children with HAE aged 2-11 —

—U.S. IND open for BCX17725 for Netherton syndrome—

RESEARCH TRIANGLE PARK, N.C., May 05, 2025 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (Nasdaq:BCRX) today reported financial results for the first quarter ended March 31, 2025, and provided a corporate update.

“We started 2025 with another quarter of outstanding performance. ORLADEYO revenue growth was driven by moving ORLADEYO patients from free drug to paid at a much faster rate than we expected, resulting in a substantial increase to our annual guidance as we also move closer to peak sales of $1 billion. This increased financial strength accelerates our path to profitability and enables us to start paying down our debt, while continuing to invest in and advance our pipeline,” said Jon Stonehouse, president and chief executive officer of BioCryst.

ORLADEYO® (berotralstat): Oral, Once-daily Treatment for Prevention of Hereditary Angioedema (HAE) Attacks

- ORLADEYO net revenue in the first quarter of 2025 was $134.2 million (+51 percent year-over-year (y-o-y)).
- The total percentage of all ORLADEYO patients on paid drug has increased to approximately 84 percent (compared to 73.5 percent at end of 2024), generating higher than expected ORLADEYO revenue that the company expects will continue through the full year.
- First quarter prescriptions were strong, above the quarterly average in 2024, and the percentage of U.S. HAE patients who describe a strong preference for an oral prophylaxis therapy increased to 70 percent, up from 50 percent in 2023, in the company’s latest market survey of HAE patients.
- Sales from the U.S. contributed 89.5 percent of global ORLADEYO net revenues in the first quarter. The number of patients treated with ORLADEYO outside the U.S. continued to grow in new and existing markets.

“By driving a dramatic increase in the rate of paid ORLADEYO patients through the prescription reauthorization period, on top of continued very strong new patient demand, our team has achieved in four months what we had expected would take three years, getting the rate of paid patients nearly to our long-term goal of 85 percent. As a result, we will capture significantly more revenue opportunity in 2025 and subsequent years and our path to peak sales of $1 billion is even more profitable than we had projected,” said Charlie Gayer, chief commercial officer of BioCryst.

Rare Disease Pipeline

The goal with our pipeline is to build on our success with ORLADEYO by bringing additional selected, highly differentiated rare disease products to patients.

- The company has submitted a new drug application (NDA) to the U.S. Food and Drug Administration (FDA) to expand the ORLADEYO label to children with HAE aged 2 to 11 using an oral granule formulation. The company also expects to submit regulatory filings in 2025 in global territories, including Europe, Japan and Canada. ORLADEYO would be the first targeted oral prophylactic therapy for children with HAE.
- The FDA has cleared the company’s investigational new drug application (IND) which will enable its clinical trial of BCX17725, an investigational KLK5 inhibitor for the treatment of Netherton syndrome, to enroll patients in the United States. This phase 1 trial is also open in Australia. The company expects initial data from the program in 2025.
    - Netherton syndrome is a serious, rare, lifelong genetic disorder causing disruption of the skin barrier with premature separation of the skin layers, chronic inflammation and vulnerability to serious infections, caused by lack of normal function of a natural inhibitor of KLK5. People with Netherton syndrome often have itchy, red, scaly, inflamed skin,

fragile hair, and are more likely to develop severe food allergies, asthma and eczema. Netherton syndrome can be life threatening, especially during infancy when patients are vulnerable to dehydration and recurrent infections. Currently, there are no approved treatments that target the underlying cause of Netherton syndrome. BCX17725 is designed to replace missing functions of the natural KLK5 inhibitor, which could restore the normal skin barrier and result in improved skin function, including protection from severe inflammatory and infectious complications of the disease.

- The first clinical trial with suprachoroidal delivery of avoralstat, the company's investigational plasma kallikrein inhibitor for the treatment of diabetic macular edema (DME), has been granted authorization to proceed in Australia. The company expects initial data from DME patients in 2025.
    - DME is an important cause of vision loss in diabetes and is due to leakage of fluid from the blood vessels in the retina. While current treatments focus on vascular endothelial growth factor (VEGF) inhibition, DME can develop from other mechanisms, such as the kallikrein-bradykinin pathway. This is supported by observations that many DME patients have an incomplete response to intravitreal anti-VEGF therapies that are administered every four to eight weeks. Avoralstat targets the kallikrein-bradykinin system on the retinal vascular endothelial cells and may result in less vascular leakage and less edema. Avoralstat, delivered to the suprachoroidal space, is designed to provide long-lasting exposure to the retinal vessels, which could result in less frequent injections and a reduced burden on patients and the healthcare system.

"Today we are reporting significant milestones for each of the next three programs in our pipeline, demonstrating our focus on advancing medicines with the potential to provide differentiated outcomes for patients of all ages. As we move closer to delivering an urgently needed oral prophylactic therapy to children with HAE, we are simultaneously progressing two clinical-stage programs in Netherton syndrome and DME towards the first patient data later this year," said Dr. Helen Thackray, chief research and development officer of BioCryst.

First Quarter 2025 Financial Results

For the three months ended March 31, 2025, total revenues were $145.5 million (+$52.7 million y-o-y), compared to $92.8 million in the first quarter of 2024 (+56.8 percent y-o-y). The increase was primarily due to $134.2 million (+$45.3 million y-o-y) in ORLADEYO net revenue in the first quarter of 2025, compared to $88.9 million in ORLADEYO net revenue in the first quarter of 2024 (+51.0 percent y-o-y).

Research and development expenses for the first quarter of 2025 decreased to $37.3 million from $46.5 million in the first quarter of 2024 (-19.8 percent y-o-y), primarily due to decreased expenses driven by the discontinuation and close-out of the Factor D programs, BCX10013 and BCX9930. These reductions were partially offset by an increase in other research, preclinical and development costs, comprised of avoralstat and other early-phase pipeline programs, and a change in general and administrative expense allocations.

Selling, general and administrative expenses for the first quarter of 2025 increased to $82.5 million, compared to $59.5 million in the first quarter of 2024 (+38.7 percent y-o-y). The increase was primarily due to increased commercial investment to support our growing ORLADEYO revenue, our newly launched regions, expanded international operations and global commercial support activities. Additionally, there was an increase to general and administrative expenses, and an offsetting reduction to research and development expenses, due to a change in the general and administrative expense allocations in 2025.

Operating income for the first quarter of 2025 was $21.2 million, compared to an operating loss of $14.5 million for the first quarter of 2024. Non-GAAP operating income, excluding stock-based compensation expense, was $42.6 million for the first quarter of 2025, compared to a non-GAAP operating loss of $0.8 million for the first quarter of 2024.

Interest expense was $23.5 million in the first quarter of 2025, compared to $24.5 million in the first quarter of 2024 (-4.1 percent y-o-y). The decrease was primarily due to a decrease in interest expense associated with the interest accrued under the Pharmakon Loan Agreement.

Net income for the first quarter of 2025 was $32 thousand, or $0.00 per share, compared to a net loss of $35.4 million, or $0.17 per share, for the first quarter of 2024.

Cash, cash equivalents, restricted cash and investments totaled $317.3 million at March 31, 2025, compared to $338.4 million at March 31, 2024. Net cash utilization for the first quarter of 2025 was $25.5 million, which was driven by debt service, royalty payments and one-time first quarter compensation expense.

Early in the second quarter, the company paid down $75 million of the outstanding Pharmakon debt, which will result in approximately $23.5 million in interest savings over the life of the loan.

Financial Outlook for 2025

The company is increasing its outlook for full year 2025 global net ORLADEYO revenue to be between $580 million and $600 million (previously $535 million to $550 million).

The company now expects full year 2025 operating expenses will be $440 million to $450 million (previously $425 million to $435 million). This operating expense outlook does not reflect non-cash stock compensation expense.

The company is accelerating its expectation for sustainable profitability and positive cash flows by a year. The company now expects to deliver net income and positive cash flows for full year 2025 (previously expected both for full year 2026). Positive cash flow refers to the improvement in cash, cash equivalents, restricted cash and investments from year end 2024 to year end 2025, not including the impact of the $75 million Pharmakon prepayment made in April 2025.

Conference Call and Webcast
BioCryst management will host a conference call and webcast at 8:30 a.m. ET today to discuss the financial results and provide a corporate update. The live call may be accessed by dialing 1-844-481-2942 for domestic callers and 1-412-317-1866 for international callers. A live webcast and replay of the call will be available online in the investors section of the company website at www.biocryst.com.

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 future results, performance or achievements, and expectations regarding pipeline development timing. These statements involve known and unknown risks, uncertainties and other factors which may cause BioCryst's actual results, performance, achievements or pipeline development timing to be materially different from any future results, performance, achievements, or timing 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; BioCryst's ability to successfully progress its pipeline development plans as described herein, including meeting the expected timelines; the results of BioCryst's partnerships with third parties 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 sustained market acceptance and demand; ongoing and future preclinical and clinical development of product candidates may take longer than expected and may not have positive results; the outcome of preclinical testing and early clinical trials may not be predictive of the success of later clinical trials, and interim results of a clinical trial do not necessarily predict final 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 not review regulatory filings on our expected timeline, 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 commercialize its 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; timing for achieving and sustainability of profitability and positive cash flow may not meet management's expectations; statements and projections regarding financial guidance and goals and the attainment of such goals may differ from actual results based on market factors and BioCryst's ability to execute its operational and budget plans; 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 projections and forward-looking statements.

Non-GAAP Financial Measures
The information furnished in this release includes non-GAAP financial measures that differ from measures calculated in accordance with generally accepted accounting principles in the United States of America ("GAAP"), including financial measures labeled as "non-GAAP" or "adjusted."

We believe providing these non-GAAP measures, which show our results with these items adjusted, is valuable and useful since they allow management and investors to better understand the company's financial performance in the absence of certain non-cash items such as stock-based compensation and allow investors to more accurately understand our current and past period results and more easily compare them to future results. These non-GAAP measures also correspond with the way we expect investors and financial analysts to compare our results. Our non-GAAP measures should be considered only as supplements to, and not as substitutes for or in isolation from, our other measures of financial information prepared in accordance with GAAP, such as GAAP revenue, operating income, net income, and earnings per share.

Our reference to our first quarter 2024 and 2025 "non-GAAP" financial measure of non-GAAP operating income (loss) constitutes a non-GAAP financial measure. It refers to our GAAP results, adjusted to show the results without including non-cash stock compensation expense. A reconciliation between GAAP and non-GAAP operating income (loss) is provided in the table below.

We also provide our non-GAAP operating expense outlook for full year 2025, which refers to our expected GAAP operating expense, excluding stock-based compensation expense. We have not provided a reconciliation against the comparable forward-looking GAAP measure because we are unable to predict with reasonable certainty the full amount of stock-based compensation expense for full year 2025 without unreasonable effort. Stock-based compensation expense is uncertain and depends on various factors, including our future hiring and retention needs, as well as the future fair market value of our common stock, which is difficult to predict and subject to change. The actual amount of stock-based compensation expense for the full year 2025 could have a material impact on GAAP reported results for the guidance period.

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BIOCRYST PHARMACEUTICALS, INC.
CONSOLIDATED FINANCIAL SUMMARY
(In thousands, except per share)

Statements of Operations (unaudited)

	Three Months Ended March 31,	
	2025	2024
Revenues:		
ORLADEYO	$ 134,243	$ 88,867
Other	11,291	3,894
Total revenues	145,534	92,761
Expenses:		
Cost of product sales	4,568	1,265
Research and development	37,270	46,493
Selling, general and administrative	82,469	59,491
Total operating expenses	124,307	107,249
Income (loss) from operations	21,227	(14,488)
Other income (expense):		
Interest income	3,024	4,031
Interest expense	(23,494)	(24,506)
Foreign currency gains (losses), net	1	(51)
Total other expense	(20,469)	(20,526)
Income (loss) before income taxes	758	(35,014)
Income tax expense	726	365
Net income (loss)	$ 32	$ (35,379)
Net income (loss) per common share: basic	$ 0.00	$ (0.17)
Weighted average shares of common stock outstanding: basic	208,882	206,064
Net income (loss) per common share: diluted	$ 0.00	$ (0.17)
Weighted average shares of common stock outstanding: diluted	215,261	206,064

Balance Sheet Data (in thousands)

	March 31, 2025 (unaudited)	December 31, 2024 Note 1
Cash, cash equivalents and investments	$ 315,640	$ 341,173
Restricted cash	1,691	1,610
Receivables	93,394	79,069
Total assets	480,047	490,420
Secured term loan	315,413	314,869
Royalty financing obligation	500,918	513,729
Accumulated deficit	(1,770,008)	(1,770,040)
Stockholders' deficit	(451,927)	(475,934)
Shares of common stock outstanding	209,208	208,543

Note 1: Derived from audited financial statements.

Reconciliation of Adjusted Income (Loss) from Operations (in thousands)

	Three Months Ended March 31,	
	2025	2024
GAAP income (loss) from operations	$ 21,227	$ (14,488)
Less: Stock-based compensation expense	(21,368)	(13,652)
Adjusted income (loss) from operations	$ 42,595	$ (836)