BioCryst and Clearside Biomedical Enter Partnership to Develop Avoralstat for Diabetic Macular Edema Using Clearside's Proprietary SCS Microinjector®

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– SCS Microinjector is the first and only FDA-approved approach to access the suprachoroidal space –

– BioCryst to host R&D Day today at 1:00 pm ET –

ALPHARETTA, Ga. and RESEARCH TRIANGLE PARK, N.C., Nov. 03, 2023 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (Nasdaq: BCRX) and Clearside Biomedical, Inc. (Nasdaq: CLSD) today announced the entry into a license agreement enabling BioCryst to develop its investigational plasma kallikrein inhibitor, avoralstat, with Clearside's SCS Microinjector ® to deliver avoralstat directly to the back of the eye through the suprachoroidal space to treat patients with diabetic macular edema (DME).

"Many DME patients are not effectively controlled with anti-VEGF therapy, so it is exciting to target plasma kallikrein by combining avoralstat with delivery into the suprachoroidal space using Clearside's proprietary SCS Microinjector. Our collaboration provides an opportunity for us to advance the development of avoralstat into a proof-of-concept trial as a potential best-in-class medicine that can offer new hope to patients living with DME," said Jon Stonehouse, president and chief executive officer of BioCryst.

Avoralstat has high potency and low solubility, which are two characteristics important to achieving potential efficacy with reduced dosing frequency in the eye for DME patients. Delivering avoralstat directly into the suprachoroidal space could allow avoralstat to inhibit plasma kallikrein at the sites of edema formation in DME disease, the retinal and choroidal vascular endothelium.

Avoralstat was previously evaluated in an oral formulation in a Phase 3 clinical trial in patients with hereditary angioedema (HAE). In the HAE clinical trial program in 276 individuals, oral avoralstat was safe and well tolerated with an adverse event profile similar to placebo.

"Treating patients with DME by delivering avoralstat directly to the suprachoroidal space using the SCS Microinjector expands the reach of our proprietary SCS injection platform to a greater number of patients. We are proud to partner with BioCryst to help bring a potential new treatment to patients who are underserved by current therapies for DME," said George Lasezkay, Pharm.D., J.D., president and chief executive officer of Clearside.

Under the terms of the agreement, Clearside will receive a $5 million upfront license fee from BioCryst. Clearside is eligible to receive up to an additional $30 million in clinical and regulatory milestone payments, and up to a total of $47.5 million in three post-approval sales-based milestone payments as annual global net sales progress to $2 billion.

BioCryst will pay Clearside tiered mid-single digit royalties on annual global net product sales, at three tiers, including a top tier of >$1.5 billion.

DME is the most common cause of vision loss in individuals with diabetes and at least one-third of patients have persistent DME despite treatment with anti-VEGF therapies, which are administered via monthly injection. Data have shown that elevated kallikrein may be a cause of non-response to anti-VEGF therapy.

BioCryst R&D Day
BioCryst will host a Research and Development (R&D) Day at 1:00 pm ET today at its Discovery Center of Excellence in Birmingham, AL. At the R&D Day, BioCryst plans to describe its drug discovery process and introduce additional therapies from its pipeline. The live webcast and replay of the R&D Day will be available online in the investors section of the BioCryst 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.

About Clearside Biomedical, Inc.
Clearside Biomedical, Inc. is a biopharmaceutical company revolutionizing the delivery of therapies to the back of the eye through the suprachoroidal space (SCS®). Clearside's SCS injection platform utilizes its patented SCS Microinjector ®, the first and only FDA-approved way to access the suprachoroidal space. Clearside's SCS Microinjector enables an in-office, repeatable, non-surgical procedure for the targeted and compartmentalized delivery of a wide variety of therapies to the macula, retina, or choroid to potentially preserve and improve vision in patients with sight-threatening eye diseases. Clearside developed and gained approval for its first product, XIPERE® (triamcinolone acetonide injectable suspension) for suprachoroidal use, which is available in the U.S. through a commercial partner. Clearside is developing its own pipeline of small molecule product candidates for administration via its SCS Microinjector. Clearside's lead suprachoroidal development program, CLS-AX (axitinib injectable suspension), is in Phase 2b clinical testing for the treatment of neovascular age-related macular degeneration (wet AMD). Clearside also strategically partners its SCS injection platform with companies utilizing other ophthalmic therapeutic innovations. For more information, please visit clearsidebio.com.

BioCryst Pharmaceuticals, Inc. Forward-Looking Statements

This press release contains forward-looking statements, including statements regarding BioCryst's plans and expectations for avoralstat. These

statements involve known and unknown risks, uncertainties and other factors which may cause BioCryst's 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 files periodically with the Securities and Exchange Commission; the results of BioCryst's partnerships with third parties may not meet BioCryst's current expectations; ongoing and future preclinical and clinical development of product candidates may take longer than expected and 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 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 product candidates, or may withhold or delay market approval for product candidates; product candidates, if approved, may not achieve market acceptance; BioCryst's ability to successfully commercialize its products and 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 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.

Clearside Biomedical, Inc. Cautionary Note Regarding Forward-Looking Statements

Any statements contained in this press release that do not describe historical facts may constitute forward-looking statements as that term is defined in the Private Securities Litigation Reform Act of 1995. These statements may be identified by words such as "believe", "expect", "may", "plan", "potential", "will", and similar expressions, and are based on Clearside's current beliefs and expectations. These forward-looking statements include statements regarding the potential benefits of product candidates delivered using Clearside's SCS Microinjector®. These statements involve risks and uncertainties that could cause actual results to differ materially from those reflected in such statements. Risks and uncertainties that may cause actual results to differ materially include uncertainties inherent in the conduct of clinical trials, Clearside's reliance on third parties over which it may not always have full control and other risks and uncertainties that are described in Clearside's Annual Report on Form 10-K for the year ended December 31, 2022, filed with the U.S. Securities and Exchange Commission (SEC) on March 14, 2023, Clearside's Quarterly Report on Form 10-Q for the quarter ended June 30, 2023 filed with the SEC on August 14, 2023 and Clearside's other Periodic Reports filed with the SEC. Any forward-looking statements speak only as of the date of this press release and are based on information available to Clearside as of the date of this release, and Clearside assumes no obligation to, and does not intend to, update any forward-looking statements, whether as a result of new information, future events or otherwise.

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