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ChemoCentryx Receives FDA Orphan Drug Designation for Avacopan in the Treatment of Debilitating Kidney Disease C3 Glomerulopathy (C3G)

MOUNTAIN VIEW, Calif., March 22, 2017 (GLOBE NEWSWIRE) -- ChemoCentryx, Inc., (Nasdaq:CCXI), a biopharmaceutical company developing new medications targeted at inflammatory and autoimmune diseases and cancer, announced today that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation for avacopan in the treatment of patients with C3 glomerulopathy (C3G).

C3G is a severe kidney disease characterized by deposits of proteins from the body's complement system into and around the kidney's filtration units, the glomeruli. The deposition of complement protein disrupts kidney function and also triggers a profoundly destructive inflammation of the kidney. The disease process ultimately leads to renal failure, with the need for dialysis and kidney transplant.

There is currently no approved treatment for C3G, and relapse is common even after kidney transplant.

Avacopan targets the complement C5a receptor (C5aR) which drives the destructive inflammatory cells in C3G and other kidney diseases. In this way, avacopan is designed to arrest the ongoing process of kidney damage in C3G, allowing kidney function to stabilize.

"Scientific and clinical evidence supports avacopan's potential in the treatment of C3G, a debilitating kidney disorder that strikes young people," said Thomas J. Schall, Ph.D., President and Chief Executive Officer of ChemoCentryx. "Receipt of FDA orphan status will allow us to expedite development of this promising treatment option for C3G, which has already had a life-changing effect on a C3G patient treated continuously with avacopan for well over a year under the Special Needs program (the equivalent of a compassionate use protocol) in the United Kingdom."

ChemoCentryx plans to initiate a multi-center clinical endpoint study of avacopan for the treatment of C3G in the first half of 2017.

About Avacopan (CCX168)

Avacopan (CCX168) is an orally-administered small molecule that is a selective inhibitor of the complement C5a receptor, or C5aR. Avacopan is in Phase III development for the treatment of anti-neutrophil cytoplasmic auto-antibody-associated vasculitis (AAV). In clinical studies to date, avacopan was shown to be safe, well tolerated and provided effective control of the disease while successfully allowing elimination of high-dose steroids, part of the standard of care for AAV patients. Avacopan is also being developed in patients with C3 glomerulopathy (C3G) and atypical hemolytic uremic syndrome (aHUS).

The U.S. Food and Drug Administration has now granted avacopan orphan-drug designation for all three of these diseases: AAV, C3G and aHUS. The European Commission has granted orphan medicinal product designation for avacopan for the treatment of two forms of AAV: microscopic polyangiitis and granulomatosis with polyangiitis (formerly known as Wegener's granulomatosis).

Avacopan was also granted access to the European Medicines Agency's (EMA) PRIority MEdicines (PRIME) initiative, which supports accelerated assessment of investigational therapies addressing unmet medical need.

About Complement 3 Glomerulopathy (C3G)

C3 glomerulopathy is characterized by evidence of alternative complement activation based on C3 deposition in the glomeruli. Patients with C3G often have high levels of protein in the urine (proteinuria) and experience progressive deterioration in renal function. Without treatment, C3G leads to kidney failure, with kidney transplant often the only option. Even after transplantation, the new kidney will frequently manifest the disease.

There is no approved treatment for C3G; non-specific immunosuppressants are frequently employed. The estimated prevalence of C3G is two-to-three per million people.

About ChemoCentryx

ChemoCentryx is a biopharmaceutical company developing new medications targeted at inflammatory and autoimmune diseases, and cancer. ChemoCentryx targets the chemokine and chemoattractant systems to discover, develop and commercialize orally-administered therapies. ChemoCentryx is currently focusing on its late stage drug candidates for patients with rare kidney diseases, avacopan (CCX168) and CCX140.

Avacopan is an orally-administered small molecule that is a selective inhibitor of the complement C5a receptor, or C5aR. Avacopan is in Phase III development for the treatment of anti-neutrophil cytoplasmic auto-antibody-associated vasculitis (AAV). In clinical studies to date, avacopan was shown to be safe, well tolerated and provided effective control of the disease while allowing elimination of high-dose steroids, part of the current standard of care. Avacopan is also being developed in patients with C3 glomerulopathy (C3G) and atypical hemolytic uremic syndrome (aHUS). The U.S. Food and Drug Administration has now granted avacopan orphan-drug designation for three indications, AAV, C3G and aHUS. The European Commission has granted orphan medicinal product designation for avacopan for the treatment of two forms of AAV: microscopic polyangiitis and granulomatosis with polyangiitis (formerly known as Wegener's granulomatosis). Avacopan was also granted access to the European Medicines Agency's (EMA) PRIority MEdicines (PRIME) initiative, which supports accelerated assessment of investigational therapies addressing unmet medical need.

The Company's other late stage drug candidate is CCX140, an inhibitor of the chemokine receptor known as CCR2, which is currently being developed for patients with focal segmental glomerulosclerosis (FSGS), a debilitating kidney disease.

ChemoCentryx's Kidney Health Alliance with Vifor Pharma provides Vifor Pharma with exclusive rights to commercialize avacopan and CCX140 in markets outside of the U.S. and China.

ChemoCentryx also has early stage drug candidates that target chemoattractant receptors in other Inflammatory and autoimmune diseases and in cancer.

Forward-Looking Statements
ChemoCentryx cautions that statements included in this press release that are not a description of historical facts are forward-looking statements. Words such as "may," "could," "will," "would," "should," "expect," "plan," "anticipate," "believe," "estimate," "intend," "predict," "seek," "contemplate," "potential," "continue" or "project" or the negative of these terms or other comparable terminology are intended to identify forward-looking statements. These statements include the Company's statements whether avacopan (CCX168) will be shown to be safe and effective in the treatment of C3 glomerulopathy and other rare diseases and the Company's statement regarding the timing of initiating additional clinical trials to further investigate avacopan in the treatment of C3G. The inclusion of forward-looking statements should not be regarded as a representation by ChemoCentryx that any of its plans will be achieved. Actual results may differ from those set forth in this release due to the risks and uncertainties inherent in the ChemoCentryx business and other risks described in the Company's filings with the Securities and Exchange Commission ("SEC"). Investors are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof, and ChemoCentryx undertakes no obligation to revise or update this news release to reflect events or circumstances after the date hereof. Further information regarding these and other risks is included under the heading "Risk Factors" in ChemoCentryx's periodic reports filed with the SEC, including ChemoCentryx's Annual Report on Form 10-K filed with the SEC on March 14, 2017 and its other reports which are available from the SEC's website (www.sec.gov) and on ChemoCentryx's website (www.chemocentryx.com) under the heading "Investors." All forward-looking statements are qualified in their entirety by this cautionary statement. This caution is made under the safe harbor provisions of Section 21E of the Private Securities Litigation Reform Act of 1995.

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