# C4 Therapeutics Announces FDA Orphan Drug Designation for CFT8634 for the Treatment of Soft Tissue Sarcoma

March 9, 2022

WATERTOWN, Mass., March 09, 2022 (GLOBE NEWSWIRE) -- C4 Therapeutics, Inc. (C4T) (Nasdaq: CCCC), a clinical-stage biopharmaceutical company dedicated to advancing targeted protein degradation science to develop a new generation of small-molecule medicines and transform how disease is treated, today announced that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation (ODD) to CFT8634 for the treatment of soft tissue sarcoma.

The FDA's Office of Orphan Products Development grants orphan designation status to drugs and biologics that are intended for the safe and effective treatment, diagnosis or prevention of rare diseases, or conditions that affect fewer than 200,000 people in the U.S. Orphan Drug Designation provides certain benefits, including financial incentives, to support clinical development and the potential for up to seven years of market exclusivity in the U.S. upon regulatory approval.

"Patients living with synovial sarcoma currently have limited treatment options. After first-line treatment with chemotherapy, the benefit of which is typically of limited durability, patients with metastatic synovial sarcoma tend to do poorly. The FDA's decision to grant orphan drug designation to CFT8634 is an important recognition of the potential of our targeted protein degrader to address this dire unmet medical need faced by patients and their families," said Adam Crystal, M.D., Ph.D., chief medical officer of C4 Therapeutics.

CFT8634 is a BiDAC™ degrader targeting BRD9 for the treatment of cancers that are dependent on BRD9, including synovial sarcoma and SMARCB1 deleted cancers. BRD9 has been considered an "undruggable" target because inhibitors of the bromodomain of BRD9 are not effective in treating these cancers. However, C4T's TORPEDO ® platform was leveraged to discover CFT8634, an orally bioavailable, selective degrader of BRD9. Unlike BRD9 inhibition, BRD9 degradation is efficacious in preclinical models of synovial sarcoma. In December 2021, the FDA cleared C4T's investigational new drug (IND) application for CFT8634 to proceed with the proposed Phase 1/2 trial in patients with synovial sarcoma and SMARCB1-null solid tumors. Site activation efforts have commenced and the trial remains on track to begin dosing patients in the first half of 2022.

**About C4 Therapeutics**
C4 Therapeutics (C4T) (Nasdaq: CCCC) is a clinical-stage biopharmaceutical company dedicated to delivering on the promise of targeted protein degradation science to create a new generation of medicines that transforms patients' lives. C4T is leveraging its TORPEDO ® platform to efficiently design and optimize small-molecule medicines that harness the body's natural protein recycling system to rapidly degrade disease-causing proteins, offering the potential to overcome drug resistance, drug undruggable targets and improve patient outcomes. C4T is advancing multiple targeted oncology programs to the clinic and expanding its research platform to deliver the next wave of medicines for difficult-to-treat diseases. For more information, please visit www.c4therapeutics.com.

**Forward-Looking Statements**
This press release contains "forward-looking statements" of C4 Therapeutics, Inc. within the meaning of the Private Securities Litigation Reform Act of 1995. These forward-looking statements may include, but may not be limited to, express or implied statements regarding our ability to develop potential therapies for patients; the design and potential efficacy of our therapeutic approaches; the predictive capability of our TORPEDO® platform in the development of novel, selective, orally bioavailable degraders; the potential timing, design and advancement of our pre-clinical studies and clinical trials, including the potential timing for regulatory authorization related to clinical trials; our ability and the potential to successfully manufacture and supply our product candidates for clinical trials; our ability to replicate results achieved in our pre-clinical studies or clinical trials in any future studies or trials; and regulatory developments in the United States and foreign countries. Any forward-looking statements in this press release are based on management's current expectations and beliefs of future events and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in or implied by such forward-looking statements. These risks and uncertainties include, but are not limited to: uncertainties related to the initiation, timing, advancement and conduct of pre-clinical and clinical studies and other development requirements for our product candidates; the risk that any one or more of our product candidates will cost more to develop or may not be successfully developed and commercialized; and the risk that the results of pre-clinical studies and/or clinical trials will or will not be predictive of results in connection with future studies or trials. For a discussion of these and other risks and uncertainties, and other important factors, any of which could cause our actual results to differ from those contained in the forward-looking statements, see the section entitled "Risk Factors" in C4 Therapeutics' most recent Annual Report on Form 10-K and/or Quarterly Report on Form 10-Q, as filed with the Securities and Exchange Commission. All information in this press release is as of the date of the release, and C4 Therapeutics undertakes no duty to update this information unless required by law.

**Investor Contact:**
Kendra Adams
SVP, Communications & Investor Relations
Kendra.Adams@c4therapeutics.com

**Media Contact:**
Loraine Spreen
Director, Corporate Communications & Patient Advocacy

LSpreen@c4therapeutics.com