Cidara Therapeutics and Mundipharma Announce Positive Topline Results from the Global Phase 3 Pivotal ReSTORE Trial of Rezafungin for the Treatment of Candidemia and Invasive Candidiasis

December 14, 2021

Trial met FDA and EMA-pre-specified primary endpoints versus daily standard of care

Data to support global regulatory filings in mid-2022

Cidara to host investor conference call today, December 14th, at 8:30 a.m. EST to provide further details on the data

SAN DIEGO and CAMBRIDGE, United Kingdom, Dec. 14, 2021 (GLOBE NEWSWIRE) -- Cidara Therapeutics, Inc. (NASDAQ: CDTX) and Mundipharma today announced positive topline data from the pivotal ReSTORE Phase 3 clinical trial evaluating the efficacy and safety of its once-weekly antifungal candidate rezafungin as a potential first-line treatment for candidemia and invasive candidiasis.

Rezafungin met the primary endpoint for the U.S. Food and Drug Administration (FDA) New Drug Application (NDA) submission of all-cause mortality at Day 30, and also met the primary endpoint for the European Medicines Agency (EMA) Marketing Authorization Application (MAA) submission of global cure at Day 14. Both results demonstrated statistical non-inferiority of rezafungin dosed once-weekly, versus caspofungin dosed once-daily, the current standard of care. Rezafungin was generally well tolerated and had a similar safety profile to caspofungin.

“The results of the ReSTORE trial reinforce our belief that rezafungin has the potential to have a significant impact on the care of patients battling difficult-to-treat and often deadly invasive *Candida* infections,” said Jeffrey Stein, Ph.D., president and chief executive officer of Cidara. “We are pleased by the overall efficacy and safety results including the data on early efficacy outcomes and ICU stay. We would like to thank all of the investigators and their site staff for their relentless hard work and dedication, as well as the patients who made it possible for us to generate these data. With the results of both the STRIVE and ReSTORE trials now in hand, which together form our registration package, we intend to file our NDA with the FDA and other regulators outside the U.S., in mid-2022.”

George Thompson, M.D., principal investigator in the ReSTORE trial and professor of clinical medicine at the University of California, Davis, School of Medicine, added, “Those of us treating deadly fungal infections have been anticipating these data and if rezafungin is approved it could be an important new option for the care of our patients. I’m excited that the data from the ReSTORE trial support a potential place for once-weekly rezafungin in the treatment of critically ill patients both inside and outside the hospital.”

Cidara has partnered with Mundipharma who has commercial rights to rezafungin outside the U.S. and Japan.

Brian Sheehan, Ph.D., chief scientific officer at Mundipharma, commented, “We are thrilled to announce these data that support the potential use of once-weekly rezafungin which, if approved, will be the first new treatment option for patients with candidemia and/or invasive candidiasis in over a decade.”

ReSTORE (NCT03667690) is a global, randomized, double-blind, controlled Phase 3 study. It completed enrollment with 187 patients diagnosed with candidemia and/or invasive candidiasis in 132 clinical sites across 18 countries. Study sites in China are recruiting patients for submission of rezafungin to the Center for Drug Evaluation. ReSTORE evaluated one 400 milligram (mg) dose of rezafungin for the first week followed by 200 mg of rezafungin dosed once-weekly for up to four weeks in total. The treatment arm was compared to approved daily dosing of caspofungin in a 1:1 randomization.

Summary of topline efficacy results:

	Rezafungin once-weekly 400mg Wk1/200mg N=93 (mITT) n (%)	**Caspofungin once-daily 70mg D1/50mg N=94 (mITT) n (%)**	**95% CI**
Primary Endpoints			
Day 30 All-Cause Mortality (FDA)	22 (23.7)	20 (21.3)	2.4 (-9.7, 14.4)
Day 14 Global Cure (EMA)	55 (59.1)	57 (60.6)	-1.1[1] (-14.9, 12.7)
Secondary Endpoints			
Day 5 Mycologic Eradication[2]	50/64 (78.1)	46/67 (68.7)	
Day 5 Global Cure	52 (55.9)	49 (52.1)	
Day 14 Mycologic Eradication[2]	46/64 (71.9)	47/67 (70.1)	
Exploratory Endpoints			
Day 1 Negative Blood Culture[3]	36/67 (53.7)	30/65 (46.2)	
Day 2 Negative Blood Culture[3]	49/66 (74.2)	41/64 (64.1)	
Median ICU Length of Stay[3,4]	5.0 days (n=17)	14.5 days (n=28)	

[1] Point estimate and confidence interval for the difference is adjusted for the randomization factors. [2] Patients with candidemia only. [3] Not powered for statistical comparison. [4] All patients in the ICU on day 1 or admitted to the ICU during the study included except for those who died prior to ICU discharge.

Summary of topline safety results:

Overall rates of adverse events and serious adverse events were comparable in patients receiving rezafungin and caspofungin. Rates of adverse events leading to study drug discontinuation were also similar for rezafungin and caspofungin.

Additional details from the ReSTORE dataset will be presented at upcoming medical meetings.

Investor conference call and webcast:
Cidara management will host an investor conference call and webcast at 8:30 a.m. EST today, December 14 to review the topline data.

Toll Free:	+1-877-407-0789
International:	+1-201-689-8562
Conference ID:	13725670
Webcast:	https://viavid.webcasts.com/starthere.jsp?ei=1519692&tp_key=509fc6e13f

A replay will be archived on the Company's website at www.cidara.com after the webcast.

Additional Phase 3 Trial Focused on Prophylaxis (Prevention)
The ongoing global Phase 3 ReSPECT trial (NCT04368559) evaluating rezafungin versus the standard antimicrobial regimen to prevent invasive fungal disease due to *Candida, Aspergillus* and *Pneumocystis* in subjects undergoing allogeneic bone marrow transplants continues to enroll patients. In ReSPECT, rezafungin is dosed once-weekly and compared to a daily regimen containing multiple drugs including fluconazole, posaconazole and trimethoprim-sulfamethoxazole, for 90 days, at which time fungal-free survival will be measured (the primary outcome measure). The trial is enrolling adults with underlying conditions including acute myeloid leukemia, acute lymphoblastic leukemia, chronic myelogenous leukemia, myelodysplastic syndrome(s), lymphoma and aplastic anemia.

About Invasive Candidiasis
Invasive candidiasis (IC) continues to be an area of significant unmet need, especially for critically ill patients in hospitals and patients with compromised immune systems. Despite a number of available treatments, mortality rates are as high as 40%.[1] IC is characterized as a severe, life-threatening systemic *Candida* infection of the bloodstream and/or deep/visceral tissues, known as candidemia and deep-seated tissue candidiasis.[2]

About Rezafungin
Rezafungin is a novel once-weekly echinocandin being developed for both the treatment and prevention of serious fungal infections, such as candidemia and invasive candidiasis. The structure and properties of rezafungin are specifically designed to improve upon a clinically validated mechanism intended to enhance its efficacy and safety potential for patients. Cidara has completed a Phase 3 clinical trial with rezafungin for the first-line treatment of candidemia and/or invasive candidiasis (ReSTORE trial) and is currently conducting a second Phase 3 clinical trial of rezafungin for the prevention of invasive fungal disease in patients undergoing allogeneic blood and marrow transplantation (ReSPECT trial). Rezafungin has been designated a Qualified Infectious Disease Product (QIDP) with Fast Track status by the FDA, and has been granted Orphan Drug Designation for its use in the treatment of invasive candidiasis in both the U.S. and EU.[3,4]

About Cidara Therapeutics
Cidara is developing long-acting therapeutics designed to improve the standard of care for patients facing serious diseases. The Company's portfolio is comprised of new approaches aimed at transforming existing treatment and prevention paradigms, first with its lead Phase 3 antifungal candidate, rezafungin, in addition to drug-Fc conjugates (DFCs) targeting viral and oncology diseases from Cidara's proprietary Cloudbreak ® platform. Cidara is headquartered in San Diego, California. For more information, please visit www.cidara.com.

About Mundipharma
Mundipharma is a global healthcare company with a presence across Africa, Asia Pacific, Canada, Europe, Latin America, and the Middle East.

Mundipharma is dedicated to bringing innovative treatments to patients in the areas of Pain Management, Infectious Disease and Consumer Healthcare as well as other severe and debilitating disease areas. Our guiding principles, centered around Integrity and Patient-Centricity, are at the heart of everything we do. For more information visit www.mundipharma.com.

Forward-Looking Statements
This release contains "forward-looking statements" within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended, and such forward-looking statements are made pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995. "Forward-looking statements" describe future expectations, plans, results, or strategies and are generally preceded by words such as "anticipates," "expect," "may," "plan" or "will". Forward-looking statements in this release include, but are not limited to, statements related to our expectations that the ReSTORE trial data will support an NDA submission in the U.S. and similar marketing authorization submissions in other countries; the potential timing of such submissions; and the likelihood that rezafungin, if approved, will be prescribed by physicians or included in formularies or treatment guidelines. Such statements are subject to a multitude of risks and uncertainties that could cause future circumstances, events, or results to differ materially from those projected in the forward-looking statements, such as unanticipated delays in or negative results from Cidara's pre-clinical or clinical trials, delays in action by regulatory authorities due to limitations on inspections and other COVID-19-related effects, and impacts of the COVID-19 pandemic or other obstacles on the enrollment of patients or other aspects of rezafungin development. These and other risks are identified under the caption "Risk Factors" in Cidara's most recent Quarterly Report on Form 10-Q and other filings subsequently made with the Securities and Exchange Commission. All forward-looking statements contained in this press release speak only as of the date on which they were made and are based on management's assumptions and estimates as of such date. Cidara does not undertake any obligation to publicly update any forward-looking statements, whether as a result of the receipt of new information, the occurrence of future events or otherwise.

INVESTOR CONTACT:
Brian Ritchie

LifeSci Advisors
(212) 915-2578
britchie@lifesciadvisors.com

MEDIA CONTACT:
Patrick Bursey
LifeSci Communications
(203) 430-9545
pbursey@lifescicomms.com

MEDIA CONTACT (Ex-US and Japan)
Jan Milton-Edwards
Mundipharma
+44 7341 739 984
jan.milton-edwards@mundipharma.com

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