Orphan Drug Designation granted to rezafungin in EU for the treatment of invasive candidiasis

- Invasive candidiasis (IC) is characterised as a severe, life-threatening *Candida* infection of the bloodstream and/or deep/visceral tissues[1,2]
- Despite advances in antifungal therapy, IC is still an area of significant unmet patient need associated with high mortality, particularly in immunocompromised and critically ill patients[3]
- Rezafungin is a novel, once-weekly echinocandin, currently in Phase 3 clinical trials for both the treatment of invasive candidiasis and prophylaxis against severe invasive fungal disease in patients undergoing allogeneic blood and marrow transplantation
- Mundipharma has the exclusive rights to develop and commercialise rezafungin in all markets outside of the United States and Japan, where rights are retained by Cidara

Cambridge (UK), San Diego (U.S.), January 26, 2021 – Mundipharma and Cidara Therapeutics today announced that the European Commission (EC) has now adopted the European Medicines Agency's (EMA) Committee for Orphan Medicinal Products (COMP) recommendation to grant Orphan Drug Designation (ODD) to rezafungin, a novel, once-weekly echinocandin, for the treatment of invasive candidiasis (IC).[4]

The EMA considers ODD status for medicines intended for the treatment, diagnosis or prevention of rare diseases or disorders that affect fewer than five per 10,000 people in the EU.[5] It is estimated that IC occurs in around 0.84 per 10,000 people in the EU,[6] and mostly affects hospitalised, immunocompromised or critically ill patients.[2]

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%.[3] IC is characterised as a severe, life-threatening systemic *Candida* infection of the bloodstream and/or deep/visceral tissues, known as candidaemia and deep-seated tissue candidiasis.[1]

Brian Sheehan, Ph.D., Chief Scientific Officer, Mundipharma, commented: *"Orphan drug designation is an important milestone in the development of rezafungin, which is currently in phase 3 clinical trials. Fungal infections still pose a major threat to the lives of hospitalised or immunocompromised patients. We are proud that patients affected with invasive candidiasis may have an additional treatment option to treat this potentially life-threatening condition."*

The EC decision adopting the COMP opinion follows that of the U.S. Food and Drug Administration (FDA), which has already designated rezafungin as a Qualified Infectious Disease Product (QIDP) with Fast Track status and ODD for its use in the treatment of IC, including candidaemia.

Jeffrey Stein, Ph.D., President and Chief Executive Officer, Cidara, added: "*We are pleased by the decision of the EMA to grant orphan drug designation to rezafungin, further supporting its potential as the first new antifungal for the treatment of serious invasive Candida infections in nearly 15 years. We look forward to continuing to work closely with our colleagues at Mundipharma to advance rezafungin through late-stage clinical development.*"

To find out more about the rezafungin clinical trials programme, please visit: https://clinicaltrials.gov/ct2/results?cond=&term=rezafungin&cntry=&state=&city=&dist=&Search=Search

-Ends-

About Rezafungin

Rezafungin is a novel once-weekly echinocandin being developed for both the treatment and prevention of severe fungal infections. Echinocandins are a class of antifungal drugs that act by inhibiting β (1, 3)-D- glucan synthase, a key enzyme necessary for integrity of the fungal cell wall.[7]

In studies to date, rezafungin has demonstrated a unique pharmacokinetic profile with a prolonged half-life and is also dosed for front-loaded plasma exposure which, in contrast to all other echinocandins, is intended to allow for once-weekly IV therapy for inpatient and outpatient use.[8] *In Vitro* data demonstrate that rezafungin has potent antifungal activity against representative strains of *Candida* spp, *Aspergillus* spp, and *Pneumocystis* spp.[9,10] Data from the Phase 2 STRIVE study showed that rezafungin met all of its objectives for safety, tolerability and efficacy in the treatment of patients with candidaemia and/or IC.[11]

Rezafungin is currently in Phase 3 development. The ReSTORE trial (NCT03667690) is an ongoing, global, randomised, double-blind, controlled, pivotal Phase 3 study to evaluate the safety, tolerability and efficacy of rezafungin compared to caspofungin for the treatment of candidemia and IC in approximately 184 qualifying patients. The primary efficacy outcome measure for the EMA is Global Cure at Day 14 while the primary efficacy outcome measure for the FDA is All-Cause Mortality at Day 30. The ReSPECT trial (NCT04368559) is an ongoing global, randomised, double-blind, controlled, pivotal Phase 3 trial of rezafungin versus the standard antimicrobial regimen to prevent invasive fungal disease due to *Candida*, *Aspergillus* and *Pneumocystis* in subjects undergoing allogeneic blood and marrow

transplantation. The trial is expected to enroll approximately 462 adults with underlying conditions, and receive either rezafungin or the standard antimicrobial regimen for 90 days, at which time fungal-free survival will be measured as the primary efficacy outcome.

About Mundipharma

Mundipharma is a global (ex-US) network of independent associated companies with a presence across Africa, Asia Pacific, Canada, Europe, Latin America and the Middle East.

As a dynamic, forward-looking organisation we are dedicated to bringing innovative treatments to patients in the areas of Pain & Supportive Care and Consumer Healthcare as well as other severe and debilitating disease areas.

Our guiding principles, centred around Integrity and Patient-Centricity, are at the heart of everything we do. For more information visit www.mundipharma.com

About Cidara Therapeutics

Cidara is developing long-acting therapeutics designed to transform the standard of care for patients facing serious fungal or viral infections. The Company's portfolio is comprised of its lead antifungal candidate, rezafungin, in addition to antiviral conjugates (AVCs) for the prevention and treatment of influenza and other viral diseases from Cidara's proprietary Cloudbreak® antiviral platform. Cidara is headquartered in San Diego, California. For more information, please visit www.cidara.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 rezafungin's efficacy and potential as a once-weekly treatment and its ability to prevent severe fungal infections and disease. 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. 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.

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