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## **Analysis of Data from Two Phase 3 Studies Shows DIFICID® Offered Faster Diarrheal Symptom Improvement than Oral Vancomycin in Patients with Cancer Being Treated for Clostridium difficile-Associated Diarrhea (CDAD)**

**- Results will be presented at the 2012 American Society of Clinical Oncology (ASCO) Annual Meeting -**

SAN DIEGO, May 16, 2012 /PRNewswire/ -- Optimer Pharmaceuticals, Inc. (NASDAQ: OPTR) today announced the results of a retrospective subpopulation analysis of 183 patients with cancer from the company's two large Phase 3 trials, which showed the cancer patients with *Clostridium difficile*-associated diarrhea (CDAD) who were treated with DIFICID® (fidaxomicin) tablets experienced resolution of their diarrheal symptoms approximately two days faster than those treated with oral vancomycin. These results will be presented on June 2nd at the 2012 American Society of Clinical Oncology (ASCO) Annual Meeting in Chicago, IL.

(Logo: <http://photos.prnewswire.com/prnh/20090413/LA97352LOGO>)

"CDAD is a serious complication for cancer patients and potentially can disrupt the effects of cancer treatment and result in dehydration, impaired functioning, fatigue and, in severe cases, death," said Kathleen Mullane, D.O., Associate Professor of Medicine, Section of Infectious Diseases and Global Health, at the University of Chicago. "The results of this analysis indicate DIFICID may provide faster resolution of diarrhea when compared to vancomycin in a subset of cancer patients. While further studies are needed to confirm these findings, the results suggest the drug may serve as an important treatment option for this population and others at heightened risk for CDAD."

The analysis assessed treatment outcomes between patients with active cancer and patients without cancer who were treated with either DIFICID or oral vancomycin in two large, pivotal, Phase 3 studies. Results showed that overall, cancer patients with CDAD had slower time to resolution of diarrhea (TTROD) than non-cancer patients (100 hours vs. 55 hours, respectively; K-M log rank  $p < 0.001$ ). However, when treatment outcomes were compared between cancer patients receiving DIFICID or oral vancomycin, patients treated with DIFICID experienced resolution of diarrhea two days faster than those treated with vancomycin (74 hours vs. 123 hours, respectively; K-M log rank  $p=0.045$ ). The overall safety profile was similar between the DIFICID and vancomycin treatment groups.

"Fast resolution of diarrhea is critically important for patients undergoing cancer treatment to help resume focus on their primary cancer treatment as quickly as possible," said Sherwood L. Gorbach, M.D., Chief Scientific Officer and Senior Vice President of Optimer Pharmaceuticals, Inc. "These results further support the strong efficacy profile of DIFICID."

Additional results from the retrospective subpopulation analysis that demonstrate the effectiveness of DIFICID in treating CDAD in cancer patients recently were presented at the 22nd European Congress of Clinical Microbiology and Infectious Diseases (ECCMID). These results showed that DIFICID was five times more likely than vancomycin to produce a clinical response and three times more likely to lead to a sustained response, while patients treated with vancomycin had a 2.6 fold greater risk of experiencing recurrence. Specifically, DIFICID provided superior response compared to vancomycin across all clinical endpoints studied: clinical response (97.3% vs. 87.5%, 95% CI 1.07-23.98;  $p=0.041$ ), sustained response (83.6% vs. 61.3%, 95% CI 1.50-6.91;  $p=0.003$ ) and disease recurrence (14.1% vs. 30.0%, 95% CI 0.16-0.89;  $p=0.025$ ).

Infections caused by *C. difficile* and the resulting CDAD pose a significant threat to cancer patients, primarily those with compromised immune systems due to chemotherapy or stem cell transplants. Cancer patients also are at risk for CDAD due to prolonged hospitalization and exposure to antibiotics. In fact, cancer patients with solid tumor or hematologic malignancies account for 16% of hospital CDAD cases.

### **About the Study**

The results are based on two randomized, double-blind, non-inferiority Phase 3 trials conducted at sites in North America and Europe. Subjects with confirmed CDAD received either 200 mg DIFICID (fidaxomicin) dosed orally twice daily or 125 mg vancomycin dosed orally four times daily for 10 days. The primary objective of the trials was to compare the safety and efficacy of a 10-day course of DIFICID versus a 10-day course of vancomycin for the treatment of CDAD in adults. These studies served as the basis of approval for DIFICID by the U.S. Food and Drug Administration (FDA) in May 2011.

The subpopulation analysis evaluated the treatment of 183 CDAD patients with active cancer compared to 922 CDAD patients without cancer who were among the modified intent to treat population of the Phase 3 studies (N=1105). To be included in the analysis, patients must have had a current diagnosis of cancer at the time of CDAD diagnosis and were receiving various forms of treatment. Patients with active cancer (solid tumor or hematologic malignancy) were identified from medical history, concomitant medication indications, and adverse event entries in the case report forms. The analysis assessed the number of unformed bowel movements (UBM) per 24 hours to determine time to resolution of diarrhea (TTROD) — defined as the duration of passing UBMs following initial treatment, with an active response considered as  $\leq 3$  UBM/24 hours. TTROD was defined as hours from first dose of treatment to last UBM on the day preceding two days of  $\leq 3$  UBM/24 hours.

### **About DIFICID® (fidaxomicin) Tablets**

DIFICID is the first antibacterial drug indicated for *Clostridium difficile*-associated diarrhea (CDAD) to be approved in more than 25 years. It is indicated for the treatment of CDAD in adults 18 years of age or older. DIFICID is administered in 200 mg tablets given orally twice daily.

### **Important Safety Information for DIFICID**

DIFICID should not be used for systemic infections. Only use DIFICID for infection proven or strongly suspected to be caused by *C. difficile*. Prescribing DIFICID in the absence of a proven or strongly suspected *C. difficile* infection is unlikely to provide benefit to the patient and increases the risk of the development of drug resistant bacteria. The most common adverse reactions are nausea (11%), vomiting (7%), abdominal pain (6%), gastrointestinal hemorrhage (4%), anemia (2%), and neutropenia (2%).

Please visit [www.DIFICID.com](http://www.DIFICID.com) or call 855-DIFICID (343-4243) for full prescribing information for DIFICID.

### **About CDAD**

*Clostridium difficile*-associated diarrhea (CDAD) has become a significant medical problem in hospitals, long-term care facilities and in the community. CDAD is a serious illness resulting from infection of the inner lining of the colon by *C. difficile* bacteria, which produce toxins that cause inflammation of the colon, severe diarrhea and, in the most serious cases, death. Patients typically develop CDAD from the use of broad-spectrum antibiotics that disrupt normal gastrointestinal (gut) flora, possibly allowing *C. difficile* bacteria to flourish. Older patients in particular are at risk for CDAD, potentially because of a weakened immune system or the presence of underlying disease. Approximately two-thirds of CDAD patients are 65 years of age or older. Historically, approximately 20% to 30% of CDAD patients who initially respond to treatment experience a clinical recurrence.

### **About Optimer Pharmaceuticals**

Optimer Pharmaceuticals, Inc. is a global biopharmaceutical company focused on developing and commercializing innovative hospital specialty products that have a positive impact on society. Optimer developed and commercialized DIFICID® (fidaxomicin) tablets, an FDA-approved antibacterial drug for the treatment of adult patients with *Clostridium difficile*-associated diarrhea (CDAD). Optimer has also received marketing authorization for fidaxomicin tablets in the European Union under the trade name DIFICLIR™. The company is seeking marketing authorization for fidaxomicin in Canada and is exploring marketing authorization in other parts of the world where *C. difficile* has emerged as a serious health problem, including Asia. Additional information can be found at <http://www.optimerpharma.com>.

### **Forward-Looking Statements**

Statements included in this press release that are not a description of historical facts are forward-looking statements, including without limitation statements related to, the potential benefits of DIFICID in treating certain patients and the risk, impact and burden of CDAD. Words such as "believes," "would," "anticipates," "plans," "expects," "may," "intend," "will" and similar expressions are intended to identify forward-looking statements. The inclusion of forward-looking statements should not be regarded as a representation by Optimer that any of its plans will be achieved. These forward-looking statements are based on management's expectations on the date of this release. Actual results may differ materially from those set forth in this release due to the risks and uncertainties inherent in Optimer's business including, without limitation, risks relating to: the possibility of alternative means of preventing or treating CDAD, whether Optimer will conduct additional clinical trials with respect to DIFICID or whether any such trials will be successful, and other risks detailed in Optimer's filings with the Securities and Exchange Commission. Forward-looking statements speak only as of the date of this release, and Optimer undertakes no obligation to update or revise these statements, except as may be required by law.

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