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Cempra's Fusidic Acid Achieves Primary Endpoint in Phase 3 Study of ABSSSI

—Fusidic acid met primary endpoint and secondary efficacy endpoints—

—Fusidic acid well tolerated in the study—

CHAPEL HILL, N.C., Feb. 24, 2017 (GLOBE NEWSWIRE) -- Cempra, Inc. (Nasdaq:CEMP), a clinical-stage pharmaceutical company focused on developing differentiated anti-infectives for the acute care and community settings to meet critical medical needs in the treatment of infectious diseases, today announced positive topline results from a phase 3 study of oral fusidic acid in 716 patients with acute bacterial skin and skin structure infections (ABSSSI). Fusidic acid was well tolerated in the study and achieved the primary endpoint, demonstrating non-inferiority (NI) (10% NI margin) of oral fusidic acid compared to oral linezolid for early clinical response (ECR) in the intent to treat (ITT) patient population.

Study Design and Demographics

The double-blind study was conducted at 62 sites in the United States. Patients randomized to treatment with oral fusidic acid received a loading dose of 1500 mg every 12 hours for two doses, followed by 600 mg every 12 hours thereafter, until the end of a 10 day course of therapy. Patients randomized to treatment with the active comparator, oral linezolid, received 600 mg every 12 hours for 10 days. Randomization was 1:1 and was stratified by type of infection (cellulitis, wound infection, major cutaneous abscess), by age and by prior use of an antibiotic within 36 hours prior to randomization.

Overall, 67.5 percent of study subjects had an infection associated with intravenous drug abuse. Less than five percent of study subjects received an antibiotic prior to randomization.

Balanced Baseline Demographic Characteristics

ITT Population	Fusidic Acid N=359	Linezolid N=357
Sex, n (%)		
Male	244 (68.0)	218 (61.1)
Female	115 (32.0)	139 (38.9)
Infection Type, n (%)		
Major Cutaneous Abscess	46 (12.8)	47 (13.2)
Cellulitis	92 (25.6)	92 (25.8)
Wound Infection	221 (61.6)	218 (61.1)
Prior Antibiotic Usage, n (%)		
Yes	17 (4.7)	18 (5.0)
No	342 (95.3)	339 (95.0)
Recent or Ongoing IV Drug Abuse, n (%)		
Yes	245 (68.2)	238 (66.7)
No	114 (31.8)	119 (33.3)

Consistent Efficacy at ECR, End-of-Treatment (EOT) and Post-therapy Evaluation (PTE)

The primary endpoint, ECR in the ITT population, was defined as the proportion of patients alive and achieving a ≥ 20 percent reduction from baseline in lesion size at 48-72 hours after the start of study drug, without receiving rescue antibiotics. In the study, 87.2 percent of ITT patients receiving fusidic acid demonstrated ECR, compared to 86.6 percent of ITT patients receiving linezolid (treatment difference 0.6%, 95% confidence interval (CI) -4.6, +5.9), demonstrating non-inferiority to linezolid.

Fusidic acid also showed comparable efficacy to linezolid in investigator-assessed clinical response in the ITT and clinically evaluable (CE) populations at EOT and PTE (7-14 days post-EOT) visits.

Clinical Response by Population

	Fusidic Acid	Linezolid	Treatment Difference (95% CI)
ITT Population			
ECR (Primary Endpoint)	**87.2 % (313/359)**	**86.6 % (309/357)**	**+0.6 (-4.6, +5.9)**
Clinical Success at EOT	91.9 % (330/359)	89.6 % (320/357)	+2.3 (-2.2, +6.8)
Clinical Success at PTE	88.6 % (320/359)	88.5 % (316/357)	+0.1 (-4.9, +5.0)
CE Populations			
Clinical Success at EOT (CE-EOT)	97.1 % (303/312)	97.3 % (288/296)	-0.2 (-3.1, +2.8)
Clinical Success at PTE (CE-PTE)	95.7 % (292/305)	96.9 % (283/292)	-1.2 (-4.5, +2.2)

Strong Activity Against Key Pathogens, Including MRSA

Microbiological response rates by pathogen were high in both treatment groups in both the microbiological ITT (mITT) and microbiologically-evaluable (ME) patient populations (patients with isolation of a baseline pathogen, who were also clinically evaluable). The most common pathogens identified were *Staphylococcus aureus*, *Streptococcus anginosus* group species, *Streptococcus pyogenes* and *Clostridium* species. Notably, the microbiological success rate among fusidic acid recipients in each ME population with methicillin-resistant *S. aureus* (MRSA) infection was 100 percent (99/99) at both the EOT and PTE visits.

Fusidic Acid Well Tolerated

Fusidic acid was well tolerated in the study. The rates of treatment-emergent adverse events (TEAEs) were comparable between treatment groups (37.9 percent fusidic acid, 36.1 percent linezolid). The most common TEAEs in both treatment groups were gastrointestinal events (22.8 percent fusidic acid, 18.2 percent linezolid). Serious adverse events (SAEs) occurred in six fusidic acid recipients and eight linezolid recipients, and were considered study-drug related in one fusidic acid recipient (vomiting) and in two linezolid recipients (one drug induced liver injury, one vomiting). Adverse events led to study drug discontinuation in 2.2 percent of fusidic acid recipients, and 2.0 percent of linezolid recipients. There was one death in the study, an event due to illicit drug overdose and aspiration which occurred in a patient receiving linezolid. Rates of treatment-emergent ALT elevation to > 3x ULN occurred in 1.0 percent of fusidic acid recipients and 0.7 percent of linezolid patients.

"Considering complicated skin infections are one of the most rapidly growing reasons for hospitalizations and emergency department visits each year, the results with fusidic acid in this study are promising, especially for an outpatient population where there is a need for new oral drugs that are effective against MRSA," said William O'Riordan, M.D., chief medical officer of eStudySite, leaders in evaluating new therapeutic approaches for complicated skin infections.

Cempra plans to submit the full data from this study for presentation at an upcoming scientific forum.

"We are excited that the results of this phase 3 study with fusidic acid confirm the results of our phase 2 study and are consistent with the more than 40 years of experience that the product has accumulated outside the United States," said David Oldach, M.D., chief medical officer of Cempra.

"We look forward to meeting with the FDA to discuss the next steps required to bring fusidic acid to patients in the United States," Oldach added.

About Fusidic Acid

Cempra is developing fusidic acid exclusively in the U.S. for ABSSSI and is exploring its use for the long term oral treatment of refractory bone and joint infections. Fusidic acid is orally active against gram-positive bacteria, including *Staphylococcus aureus* strains such as healthcare-acquired methicillin-resistant *Staphylococcus aureus* (HA-MRSA) and community-acquired MRSA. Cempra completed a phase 2 clinical trial in patients with ABSSSI, which is frequently caused by MRSA, demonstrating a tolerability profile and efficacy comparable to linezolid, one of the few oral antibiotics with FDA approval for the treatment of MRSA. A phase 2 trial in patients with primarily staphylococcal infections of prosthetic hip and knee joints demonstrated that fusidic acid, in combination with rifampin, was generally comparable to intravenous standard of care antibiotics. Cempra has an ongoing exploratory study of fusidic acid for chronic oral treatment of refractory infections in bones and joints.

About Cempra, Inc.

Cempra, Inc. is a clinical-stage pharmaceutical company focused on developing differentiated anti-infectives for the acute care and community settings to meet critical medical needs in the treatment of infectious diseases. Cempra's two lead product candidates are currently in advanced clinical development. Solithromycin has been evaluated in two phase 3 clinical trials for community-acquired bacterial pneumonia (CABP). Cempra is currently seeking approval for both intravenous and

oral capsule formulations from the U.S. Food and Drug Administration and the European Medicines Agency. Solithromycin is licensed to strategic commercial partner Toyama Chemical Co., Ltd., a subsidiary of FUJIFILM Holdings Corporation, for certain exclusive rights in Japan. Solithromycin is also in a phase 3 clinical trial for uncomplicated urogenital urethritis caused by *Neisseria gonorrhoeae* or chlamydia. Cempra is contracted with BARDA for the development of solithromycin for pediatric use and has commenced enrollment in a global phase 2/3 trial to evaluate the safety and efficacy of solithromycin versus standard of care antibiotics in children and adolescents from two months to 17 years of age. Fusidic acid is Cempra's second product candidate, which has completed an initial phase 3 trial comparing fusidic acid to linezolid in patients with acute bacterial skin and skin structure infections (ABSSSI). Cempra also has an ongoing exploratory study of fusidic acid for chronic oral treatment of refractory infections in bones and joints. Both products seek to address the need for new treatments targeting drug-resistant bacterial infections in the hospital and in the community. Cempra is also studying solithromycin for ophthalmic conditions and has synthesized novel macrolides for non-antibiotic uses such as the treatment of chronic inflammatory diseases, endocrine diseases and gastric motility disorders. Cempra was founded in 2006 and is headquartered in Chapel Hill, N.C. For additional information about Cempra please visit www.cempra.com.

Please Note: *This press release contains forward-looking statements regarding future events. These statements are just predictions and are subject to risks and uncertainties that could cause the actual events or results to differ materially. These risks and uncertainties include, among others: our ability to address the issues identified by the FDA in the complete response letter relating to our new drug applications for solithromycin for community acquired bacterial pneumonia; our ability to obtain FDA and foreign regulatory approval of solithromycin as a treatment for community acquired bacterial pneumonia; the impact of the recently announced changes in senior management and our ability to retain and hire necessary employees and to staff our operations appropriately; our anticipated capital expenditures and our estimates regarding our capital requirements, including the costs of addressing the complete response letter; our dependence on the success of solithromycin and fusidic acid; our and our strategic commercial partners' ability to obtain FDA and foreign regulatory approval of our product candidates; the costs, sources of funds, enrollment, timing, regulatory review and results of our studies and clinical trials and those of our strategic commercial partners; results of our and our strategic commercial partners' pre-clinical studies and clinical trials are not predictive of results from subsequent clinical trials for any possible therapy; our need to obtain additional funding and our ability to obtain future funding on acceptable terms; the unpredictability of the size of the markets for, and market acceptance of, any of our products, including solithromycin and fusidic acid; our ability to commercialize and launch, whether on our own or with a strategic partner, any product candidate that receives regulatory approval; our ability to produce and sell any approved products and the price we are able to realize for those products; the possible impairment of, or inability to obtain, intellectual property rights and the costs of obtaining such rights from third parties; our ability to compete in our industry; innovation by our competitors; and our ability to stay abreast of and comply with new or modified laws and regulations that currently apply or become applicable to our business. The reader is referred to the documents that we file from time to time with the Securities and Exchange Commission.*

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