July 10, 2014

Zafgen Granted Orphan Drug Designation in the European Union for Beloranib, a First-in-Class MetAP2 Inhibitor for the Treatment of Prader-Willi Syndrome

CAMBRIDGE, Mass., July 10, 2014 (GLOBE NEWSWIRE) -- Zafgen, Inc. (Nasdaq:ZFGN), a biopharmaceutical company dedicated to significantly improving the health and well-being of patients affected by obesity, today announced that the European Commission (EC) has granted orphan drug designation for beloranib for the treatment of Prader-Willi syndrome (PWS), a rare disease.

"We are very pleased that our application for orphan drug designation of beloranib has been positively reviewed and granted by the EC," said Thomas Hughes, Ph.D., Chief Executive Officer of Zafgen. "On the basis of our Phase 2 results reported earlier this year, we believe beloranib represents a promising new approach for the treatment of PWS, with the potential to meaningfully improve the lives of patients with this severe and life-threatening condition. We are dedicated to the advancement of beloranib for the treatment of PWS and other severe forms of obesity and we look forward to initiating our Phase 3 clinical program in PWS later this year."

In January 2013, the U.S. Food and Drug Administration granted Zafgen orphan designation to treat PWS with beloranib. Initial results from our Phase 2 study of beloranib in patients with PWS demonstrated improvements in hunger-related behaviors and body composition, including reductions in body fat content and preserved lean body mass.

Orphan Drug Designation by the European Commission provides regulatory and financial incentives for companies to develop and market therapies that treat a life-threatening or chronically debilitating condition affecting no more than five in 10,000 persons in the European Union (EU), and where no satisfactory treatment is available. In addition to a 10-year period of marketing exclusivity in the EU after product approval, Orphan Drug Designation provides incentives for companies seeking protocol assistance from the EMA during the product development phase, and direct access to centralized marketing authorization.

About Prader-Willi Syndrome

Prader-Willi syndrome (PWS), the most common known genetic cause of life-threatening obesity, results in constant and unrelenting hunger that drives patients with PWS to engage in problematic food-related behaviors and gain excessive weight. As a result, many of those affected become morbidly obese and suffer significant mortality. There is currently no cure for this disease. Although the cause is complex, it results from a deletion or loss of function of a cluster of genes on the 15th chromosome. PWS typically causes low muscle mass and function, short stature, incomplete sexual development, and a chronic feeling of hunger that, coupled with a metabolism that utilizes drastically fewer calories than normal, can lead to excessive eating and life-threatening obesity. PWS occurs in males and females equally and in all races, with the same incidence around the world. Prevalence estimates have ranged from 1:8,000 to 1:50,000. You can learn more through the Prader-Willi Syndrome Association website at www.pwsausa.org.

About Beloranib

Beloranib is a novel, first-in-class injectable small molecule therapy with a unique mechanism of action that reduces hunger while stimulating the use of stored fat as an energy source. Beloranib is a potent inhibitor of MetAP2, an enzyme that modulates the activity of key cellular processes that control metabolism. MetAP2 inhibitors work, at least in part, by directing MetAP2 binding to cellular stress mediators, and, thus, reducing the tone of signals that drive lipid synthesis by the liver and fat storage throughout the body. In this manner, MetAP2 inhibition increases metabolism of fat as an energy source. Zafgen holds exclusive worldwide rights (exclusive of South Korea) for development and commercialization of beloranib. Zafgen exclusively licensed beloranib from Chong Kun Dang (CKD) Pharmaceutical Corp. of South Korea.

About Zafgen, Inc.

Zafgen (Nasdaq:ZFGN) is a biopharmaceutical company dedicated to significantly improving the health and well-being of patients affected by obesity. Beloranib, Zafgen's lead product candidate, is a novel, first-in-class, twice-weekly subcutaneous injection being developed for the treatment of multiple indications, including severe obesity in Prader-Willi syndrome, craniopharyngioma-associated obesity, and severe obesity in the general population. Zafgen was founded in 2005 to explore novel approaches to obesity therapeutics, including agents known to inhibit MetAP2 that had been found to drive unprecedented weight loss and metabolic improvements in mice. The company is located in Cambridge, MA.

Zafgen Forward-Looking Statements

Various statements in this release concerning Zafgen's future expectations, plans and prospects, including without limitation, Zafgen's expectations regarding beloranib as a treatment for PWS and other forms of severe obesity, its expectations with respect to the timing and success of its clinical trials, the expected timing of additional clinical trials, and its plans regarding commercialization of beloranib, constitute forward-looking statements for the purposes of the safe harbor provisions under The Private Securities Litigation Reform Act of 1995. Actual results may differ materially from those indicated by these forward-looking statements as a result of various important factors, including, without limitation, Zafgen's ability to successfully demonstrate the efficacy and safety of its drug candidates, the pre-clinical and clinical results for its product candidates, which may not support further development of product candidates, actions of regulatory agencies, which may affect the initiation, timing and progress of clinical trials, obtaining, maintaining and protecting intellectual property, Zafgen's ability to enforce its patents against infringers and defend its patent portfolio against challenges from third parties, competition from others developing products for similar uses, Zafgen's ability to manage operating expenses, Zafgen's ability to obtain additional funding to support its business activities and establish and maintain strategic business alliances and new business initiatives, Zafgen's dependence on third parties for development, manufacture, marketing, sales and distribution of products, the outcome of litigation, and unexpected expenditures, as well as those risks more fully discussed in the section entitled "Risk Factors" in the final prospectus related to Zafgen's initial public offering filed with the Securities and Exchange Commission pursuant to Rule 424(b) of the Securities Act, as well as discussions of potential risks, uncertainties, and other important factors in Zafgen's subsequent filings with the Securities and Exchange Commission. In addition, any forward-looking statements represent Zafgen's views only as of today and should not be relied upon as representing its views as of any subsequent date. Zafgen explicitly disclaims any obligation to update any forward-looking statements.

CONTACT: Media Contact:

Shauna Elkin

(212) 850-5613

Shauna.Elkin@fticonsulting.com

Source: Zafgen, Inc.