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

BOSTON, Sept. 30, 2015 (GLOBE NEWSWIRE) -- Zafgen, Inc. (Nasdaq:ZFGN), a biopharmaceutical company dedicated to significantly improving the health and well-being of patients affected by obesity and complex metabolic disorders, today announced that the European Commission (EC) has granted orphan drug designation for beloranib for the treatment of craniopharyngioma, a rare form of benign brain tumor. Severe and intractable weight gain is a frequent manifestation of craniopharyngioma, which is the most common cause of hypothalamic injury-associated obesity, or HIAO.

"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. "We believe beloranib represents an important and promising new approach for the treatment of HIAO, and has the potential to meaningfully improve the lives of patients with this debilitating disorder. We remain dedicated to advancing beloranib for the treatment of severe and complicated forms of obesity and look forward to further exploring its potential as a safe and effective treatment option for HIAO as a component of our clinical development program."

Zafgen previously received orphan designation by the European Commission for beloranib for the treatment of Prader-Willi syndrome (PWS) in July 2014, and by the U.S Food and Drug Administration for PWS in January 2013.

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 Hypothalamic Injury-Associated Obesity (HIAO)

HIAO is most commonly caused by damage incurred during removal of a tumor called craniopharyngioma, but it can also result from less common types of hypothalamic injury such as strokes, brain trauma, or radiation therapy to the brain. Craniopharyngioma is a rare form of histologically benign brain tumor that occurs in both children and adults; its location is near the optic nerves, pituitary gland and the hypothalamus and invasion into these tissues results in significant morbidity. Treatment of these tumors commonly involves surgical removal of the tumor, and sometimes radiation treatment, both resulting in disruption or removal of neighboring structures including the hypothalamus. Post-treatment hypothalamic dysfunction results in significant obesity in up to 50% of these patients, resulting in a variety of co-morbid conditions and a deteriorated quality of life. Craniopharyngioma-associated obesity occurs in males and females equally and in all races, with the same incidence around the world. The incidence estimates of craniopharyngioma have ranged from 0.13 to 0.17 per 100,000 per year, or approximately 400 to 500 new cases per year in the United States and 650 to 850 new cases per year in the European Union.

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 methionine aminopeptidase 2, or 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 the development and commercialization of beloranib. Zafgen exclusively licensed beloranib from Chong Kun Dang (CKD) Pharmaceutical Corp. of South Korea.

About Zafgen

Zafgen (Nasdaq:ZFGN) is a biopharmaceutical company dedicated to significantly improving the health and well-being of patients affected by obesity and complex metabolic disorders. Zafgen is focused on developing novel therapeutics that treat the underlying biological mechanisms through the MetAP2 pathway. 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 two rare diseases, Prader-Willi syndrome and obesity caused by hypothalamic injury, including craniopharyngioma-

associated obesity; and severe obesity in the general population. Zafgen is also developing ZGN-839, a liver-targeted MetAP2 inhibitor, for the treatment of nonalcoholic steatohepatitis, or NASH, and abdominal obesity, as well as second-generation MetAP2 inhibitors. Zafgen aspires to improve the lives of patients through targeted treatments and has assembled a team accomplished in bringing therapies to patients with both rare and prevalent metabolic diseases.

Safe Harbor Statement

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, HIAO, including craniopharyngioma-associated obesity, and other forms of severe obesity, including severe obesity in patients with type 2 diabetes, its expectations with respect to the timing and success of its clinical trials of beloranib and other product candidates, the expected requirements and timing of additional clinical trials and pre-clinical studies, and its plans regarding commercialization of beloranib may constitute forward-looking statements for the purposes of the safe harbor provisions under The Private Securities Litigation Reform Act of 1995 and other federal securities laws. Forward-looking statements can be identified by terminology such as "anticipate," "believe," "could," "could increase the likelihood," "estimate," "expect," "intend," "is planned," "may," "should," "will," "will enable," "would be expected," "look forward," "may provide," "would" or similar terms, variations of such terms or the negative of those terms. 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 product candidates, the pre-clinical and clinical results for its product candidates, which may not support further development, actions of regulatory agencies, which may affect the initiation, timing and progress of clinical trials, obtaining, maintaining and protecting its 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?Zafgen's?most recent Quarterly Report on Form 10-Q filed with the?Securities and Exchange Commission, 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, whether as a result of new information, future events or otherwise.

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