

# Zafgen to Present Data from bestPWS Study of Beloranib in Prader-Willi Syndrome at ENDO 2016

BOSTON, March 28, 2016 (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 new scientific data from the bestPWS ZAF-311 study, a pivotal, double-blind, placebo-controlled Phase 3 trial evaluating the safety and efficacy of beloranib, a MetAP2 inhibitor, in patients with Prader-Willi syndrome (PWS) during a six-month randomized treatment period, will be presented at ENDO 2016, the Endocrine Society's 98th Annual Meeting & Expo taking place in Boston from April 1-4.

Data from the bestPWS ZAF-311 trial will be presented during ENDO 2016, including:

### ORAL PRESENTATION:

Weight loss and Improvement in Hyperphagia-Related Behavior: Results from bestPWS, a Phase 3, Randomized, Placebo-Controlled, Clinical Trial of Beloranib in Patients with Prader-Willi Syndrome

- Abstract # 28090
- Sunday, April 3, 11:30 a.m. 1:15 pm EST (Session: LB-0402-7)
  - Presenter: Merlin G. Butler, M.D., Ph.D., FFACMG, Professor of Psychiatry, Behavioral Sciences and Pediatrics, Director, Division of Research and Genetics, Department of Psychiatry, Behavioral Sciences and Pediatrics at the Kansas University Medical Center

### POSTER PRESENTATIONS:

Improvement in Lipids and Markers of Cardiometabolic Risk: Results from bestPWS, a Phase 3, Randomized, Placebo-Controlled, Clinical Trial of Beloranib in Patients with Prader-Willi Syndrome

- Abstract # 28186
- Sunday, April 3, 1:15 3:15 p.m. (Session: LBSUN-40)

Weight loss and Improvement in Body Composition: Results from bestPWS, a Phase 3, Randomized, Placebo-Controlled, Clinical Trial of Beloranib in Patients with Prader-Willi Syndrome

- Abstract # 28188
- Sunday, April 3, 1:15 3:15 p.m. EST (Session: LBSUN-41)

# About Beloranib

Beloranib is a novel, first-in-class injectable small molecule therapy that works by inhibiting MetAP2, an enzyme that modulates the activity of key cellular processes that control metabolism. Once a person becomes obese, the body undergoes certain metabolic changes and becomes "programmed" to create and store more fat, making it much more difficult to reduce body weight. Beloranib is believed to help reduce hunger and restore balance to fat metabolism, enabling calories to once again be used as a productive energy source. Because beloranib works beyond just regulating hunger through the hypothalamus, it has the potential to be used in a variety of complex metabolic disorders such as Prader-Willi syndrome and hypothalamic injury associated obesity. Zafgen holds exclusive worldwide rights (exclusive of South Korea) for the development and commercialization of beloranib. Zafgen exclusively licensed beloranib from Chong Kun Dang Pharmaceutical Corporation (CKD Pharma) of South Korea.

# About Prader-Willi Syndrome (PWS)

Prader-Willi syndrome (PWS), is the most common known genetic cause of life-threatening obesity. A dysfunctional signaling to the hypothalamus results in constant and unrelenting perception of starvation, driving patients with PWS to engage in problematic hunger-related behaviors, known as hyperphagia, and to gain excessive weight. As a result, many of those affected with PWS become morbidly obese and suffer significant mortality. Currently, there is no cure for this disease. Although the cause of PWS 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, when 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. Patients with PWS have a shortened life expectancy of approximately 32 years, as a result of an estimated three percent annual death rate for the PWS population. Common causes of mortality in PWS include respiratory disease, cardiac disease, infection, choking, gastric rupture, and pulmonary embolism.

# 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, which may be developed for the treatment of severe obesity in the general population. 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 and obesity caused by hypothalamic injury, including craniopharyngioma-associated obesity, Zafgen's expectations regarding the use of other MetAP2 inhibitors as treatments for other forms of severe obesity, including severe obesity in the general population, Zafgen's expectations with respect to the timing and success of its non-clinical studies and clinical trials of beloranib and its other product candidates, the expected requirements and timing of additional requirements for planned clinical trials, and the need for additional clinical trials and pre-clinical studies, and Zafgen's 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 obtain a release of the full clinical hold that the FDA placed on the investigational new drug application for beloranib, Zafgen's ability to successfully demonstrate the efficacy and safety of beloranib and its other product candidates, the pre-clinical and clinical results for beloranib and its other product candidates, which may not support further development and marketing approval, actions of regulatory agencies, which may affect the initiation, timing and progress of pre-clinical studies and clinical trials, Zafgen's ability to obtain, maintain and protect 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 Annual Report on Form 10-K 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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