February 28, 2014

Zafgen Announces Support of NORD in Observing Rare Disease Day

CAMBRIDGE, Mass – February 28, 2014 -- Zafgen, Inc., a leading biopharmaceutical company dedicated to addressing the unmet needs of severely obese patients, today announced it will join the National Organization for Rare Disorders (NORD) and others around the world in observing Rare Disease Day on February 28, 2014. On this day, millions of patients and their families will share their stories to focus a spotlight on rare diseases as a global public health concern.

A rare disease is one that affects fewer than 200,000 Americans. There are nearly 7,000 such diseases affecting nearly 30 million patients in America, according to the National Institutes of Health (NIH). Rare diseases also tend to be serious and lifelong, and most have no approved treatment.

“We are excited and proud to join NORD and its members globally in their commitment to raising awareness and providing better care for those living with rare diseases,” said Thomas Hughes, Ph.D., President and Chief Executive Officer of Zafgen. “We hope that our support of Rare Disease Day encourages others to become more involved with patient populations for whom limited treatment options are available, such as those suffering from Prader-Willi Syndrome.”

“Prader-Willi Syndrome is a devastating condition that represents one of the most severe forms of genetic obesity,” said Janalee Heinemann, Director of Research & Medical Affairs for the Prader-Willi Syndrome Association (USA) (PWSA). “We are very encouraged by and welcome Zafgen’s continued efforts to help address the needs of this greatly underserved patient population.”

For more information about Rare Disease Day activities in the U.S., go to www.rarediseaseday.us. For information about global activities, go to www.rarediseaseday.org.

About Prader-Willi Syndrome

Prader-Willi syndrome, or PWS, is the most common known genetic cause of life-threatening obesity. PWS is a rare and complex non-inherited genetic disorder, which results from abnormalities of the fifteenth chromosome. Symptoms associated with PWS are believed to result, in part, from a defect in the hypothalamus, an important supervisory center in the brain that controls many important bodily functions, such as hunger and metabolism of fats and carbohydrates. The vast majority of PWS patients suffer from hyperphagia, or insatiable life-threatening hunger and hunger-related behaviors, and severe obesity. PWS patients are constantly preoccupied with food and an unrelenting and overriding physiological drive to eat. Published population studies estimate that the prevalence of PWS in the United States and in the European Union ranges from 1 in 8,000 to 1 in 50,000. We believe there are currently no effective pharmacological treatments for obesity and hyperphagia in PWS. You can learn more through the Prader-Willi Syndrome Association (USA) 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 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 PWS, 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.