Zafgen and the Foundation for Prader-Willi Research Announce PATH for PWS Natural History Study

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Information and data from study will advance understanding of medical history and medical events in people with PWS

People living with PWS ages 5 and older and their caregivers are encouraged to participate

BOSTON, July 09, 2018 (GLOBE NEWSWIRE) -- Zafgen, Inc., (Nasdaq:ZFGN), a clinical-stage biopharmaceutical company using its proprietary knowledge of MetAP2 systems biology to help patients affected by a range of metabolic diseases, and the Foundation for Prader-Willi Research (FPWR), a nonprofit organization founded to eliminate the challenges of Prader-Willi syndrome (PWS) through the advancement of research and therapeutic development, announced today the launch of a co-sponsored natural history study to advance understanding of the medical history of and medical events in people with PWS. PATH for PWS (**P**aving the way for **A**dvances in **T**reatments & **H**ealth for PWS) is a non-interventional, observational study of serious medical events in PWS, intended to inform development and clinical trial design for potential new treatments for PWS, including ZGN-1258, Zafgen's new, second-generation program for PWS designed to decrease hyperphagia, change the way the body metabolizes fat, and reduce fat mass.

Enrollment for the four-year study is anticipated to begin in September 2018 through the Global Prader-Willi Syndrome Patient Registry, which is powered by the National Organization for Rare Disorders' (NORD) IAMRARE™ Registry Program.

"While we understand more about Prader-Willi syndrome than ever before, it is critical that we continue to build on the body of knowledge to improve care and explore new treatment options," said Theresa Strong, Ph.D., Director of Research Programs, FPWR, and the lead study investigator. "The PATH for PWS study will allow us to collect and analyze important health information about those with PWS to better understand how the condition presents and how that could inform clinical trial design, new and better treatment options, and even additional resources for families and physicians. We hope caregivers of adults and children living with PWS will consider participating in the PATH for PWS to contribute to the actionable information available to our community."

Every six months, caregivers of people with PWS enrolled in the study will be asked to provide an update on any medical issues experienced and

related medical procedures or prescriptions, and information about conditions often associated with PWS such as hyperphagia. Clinic visits will not be required, as the information will be provided through internet-based surveys and questionnaires. The data will be analyzed to evaluate the incidence of serious medical events, prescription medication use associated with medical events of interest, patterns of hyperphagia-related behaviors, as well as additional areas of importance to the PWS community such as changes in body weight, management strategies for hyperphagia, and characterization of traits and actions common to individuals with PWS. The analyses will also help Zafgen, FPWR and others in the Prader-Willi research community identify potential areas for future study.

"PATH for PWS is a testament to the community-wide commitment to PWS. This isn't about a single therapy, but about the power of a partnership between PWS research and advocacy organizations, industry and the broader community to transform outcomes for patients and their families through greater knowledge and understanding," said Jeff Hatfield, Chief Executive Officer, Zafgen.

"Natural history studies are a best practice in rare disease drug development, and we are looking forward to important insights that the PATH for PWS study will provide into medical events experienced by people with PWS," said Tom Hughes, Ph.D., President and Chief Scientific Officer, Zafgen. "As we continue work on our ZGN-1258 program, the PATH for PWS study will be an invaluable resource to inform its development and potentially other therapeutic development work for those with PWS."

To be eligible for the study, patients must have a confirmed diagnosis of PWS, be at least 5 years of age, live in the United States, Canada or Australia, and be enrolled or willing to enroll in the Global PWS Registry. The primary caregiver of the enrolled person with PWS must have access to the internet to enter study data and consent to being contacted by registry staff and allowing registry staff to enter specific data on their behalf. Participation in clinical trials and other non-interventional studies will be permitted while enrolled in PATH for PWS.

For more information about PATH for PWS, please visit www.fpwr.org or www.zafgen.com. Ahead of PATH for PWS study enrollment opening in September 2018, people with PWS and their caregivers can enroll in the broader Global PWS Patient Registry at www.pwsregistry.org.

About Prader-Willi Syndrome (PWS)
Prader-Willi syndrome (PWS) is a rare, genetic form of life-threatening obesity characterized by unrelenting pathologic hunger (hyperphagia) leading to dangerous food-seeking behavior, and there is currently no approved treatment for hyperphagia. People with PWS are also impacted by slowed metabolism, psychiatric conditions and higher risk for cardiopulmonary and metabolic co-morbidities. Our best estimate of prevalence is 1:40,000 people. People with PWS have a shortened life expectancy of approximately 32 years.

About Zafgen
Zafgen (Nasdaq:ZFGN) is a clinical-stage biopharmaceutical company leveraging its proprietary MetAP2 biology platform to develop novel therapies for patients affected by complex metabolic diseases. Zafgen has pioneered the study of MetAP2 inhibitors in both common and rare metabolic disorders and is currently advancing programs for type 2 diabetes, Prader-Willi syndrome and liver diseases. The Company's lead product candidate, ZGN-1061, a MetAP2 inhibitor for difficult-to-control type 2 diabetes, has successfully completed the initial part of a Phase 2 clinical trial. Learn more at www.zafgen.com.

About Foundation for Prader-Willi Research (FPWR)
FPWR is composed of thousands of parents, family members, researchers, and others who are interested in addressing the many issues related to PWS, including childhood obesity, developmental delays, psychiatric disorders, and autism spectrum disorders. The mission of FPWR is to eliminate the challenges of Prader-Willi syndrome through the advancement of research and therapeutic development. FPWR supports cutting edge research studies around the world to advance the understanding of PWS, and collaborates with research institutions, pharmaceutical companies and patient advocacy groups to advance new treatments that will help those with PWS. To date, FPWR has funded over $10 million in PWS research. For more information please visit www.fpwr.org.

Safe Harbor Statement
Various statements in this release concerning Zafgen's future expectations, plans and prospects, including without limitation, Zafgen's expectations regarding the collection of medical history and medical events from PATH for PWS participants to inform development and clinical trial design for potential new treatments for PWS, including ZGN-1258, and the use of ZGN-1258, ZGN-1061 and other second-generation MetAP2 inhibitors as treatments for metabolic diseases including Prader-Willi syndrome, type 2 diabetes, liver diseases and obesity and Zafgen's expectations with respect to the timing and success of its ability to collect and analyze PATH for PWS data for development and clinical trial design and with respect to its nonclinical studies and clinical trials of ZGN-1258, ZGN-1061 and its other product candidates, 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 collect and analyze medical history and medical events from PATH for PWS participants, the capacity for such data to inform clinical trial design and potential areas for future study, Zafgen's ability to successfully demonstrate the efficacy and safety of ZGN-1258, ZGN-1061 and its other product candidates and to differentiate ZGN-1258, ZGN-1061 and its other product candidates from first generation MetAP2 inhibitors, such as beloranib, the nonclinical and clinical results for ZGN-1258, ZGN-1061 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 nonclinical studies and clinical trials of its product candidates, 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 when needed, Zafgen's dependence on third parties for development, manufacture, marketing, sales and distribution of product candidates, 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, including without limitation Zafgen's Quarterly Reports on Form 10-Q, 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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