**PRESS RELEASE**

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# Saniona initiates Phase 2a study for Tesomet in Prader-Willi syndrome

**Saniona, a leading biotech company in the field of ion channels, today announces that it has initiated a Phase 2a clinical study in Czech Republic and Hungary for Tesomet in patients with Prader-Willi syndrome (PWS). The first three patients have been randomized to either receive Tesomet or placebo. It is an exploratory study in a limited number of patients and includes an interim safety review. The study is expected to take approximately a year from initiation.**

“We are proud to start testing Tesomet for this orphan indication. PWS is a severe hereditary disease for which there is no treatment today. Due to its mode of action, Tesomet could provide substantial benefits to the patients as well as their families,” says Jørgen Drejer, CEO of Saniona.

The objectives of this Phase 2a study are to examine the efficacy, tolerability, safety, and pharmacokinetics of Tesomet in patient with PWS.

This exploratory randomized, double-blind, placebo-controlled study may ultimately include up to 30 patients where patients will either receive Tesomet (tesofensine 0.5 mg + metoprolol 50 mg daily) or matching placebo (3:2 randomization) for a total of 12 weeks. The study is divided into two parts. The first part of the study will include 10-15 adult patients with PWS. After having established safety in the adult patients, the second part of the study may potentially include 10-15 adolescents with PWS. Saniona expects to report the results from the trial about one year from initiation.

The primary endpoint is change in body weight over 12 weeks of treatment compared to placebo. The secondary objectives are to examine eating behavior, food craving, body composition, lipids and other metabolic parameters. The study also includes comprehensive assessments of tolerability, safety and pharmacokinetic parameters in this patient population.

“PWS is an orphan indication and relative small clinical trials and fast track regulatory interactions could be expected. Therefore, we could potentially develop Tesomet ourselves all the way to the market in this indication. If the Phase 2 and subsequent pivotal Phase 3 clinical studies prove to be successful, market approval could potentially be achieved much faster than in other indications.”

### For more information, please contact

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## About Saniona

*Saniona is a research and development company focused on drugs for diseases of the central nervous system, autoimmune diseases, metabolic diseases and treatment of pain. The company has a significant portfolio of potential drug candidates at pre-clinical and clinical stage. The research is focused on ion channels, which makes up a unique protein class that enables and controls the passage of charged ions across cell membranes. Saniona has ongoing collaboration agreements with Boehringer Ingelheim GmbH, Upsher-Smith Laboratories, Inc., Productos Medix, S.A de S.V and Saniona's Boston based spinout Ataxion Inc., which is financed by Atlas Venture Inc. and Biogen Inc. Saniona is based in Copenhagen, Denmark, where it has a research center of high international standard. Saniona is listed at Nasdaq First North Premier and has about 4,500 shareholders. Pareto Securities is Certified Advisor for Saniona. The company's share is traded under the ticker SANION. Read more at [www.saniona.com](http://www.saniona.com).*

## About Prader-Willi Syndrome (PWS)

Prader-Willi Syndrome (PWS) is recognized as the most common genetic cause of life-threatening obesity. The disease results from a deletion or loss of function of a cluster of genes on chromosome 15, which leads to dysfunctional signaling in the brain's appetite/satiety center (hypothalamus). Patients suffer from a constant, extreme, ravenous insatiable appetite which persists no matter how much the patients eat. As a result, many of those affected with PWS become morbidly obese and suffer significant mortality. Compulsive eating and obsession with food usually begin before age 6. The urge to eat is physiological, overwhelming and difficult to control. Caregivers need to strictly limit the patients' access to food, usually by installing locks on refrigerators and on all closets and cabinets where food is stored. Currently, there is no cure for this disease. Patients with PWS have a shortened life expectancy. Common causes of mortality in PWS include respiratory disease, cardiac disease, infection, choking, gastric rupture, and pulmonary embolism. However, if obesity is avoided and complications are well managed, life expectancy for individuals with PWS is normal or near normal and most individuals can lead healthy lives[1]. PWS occurs in approximately one out of every 15,000 births[2]. Males and females are affected equally. The condition is named after Andrea Prader, Heinrich Willi, and Alexis Labhart who described it in detail in 1956. The common characteristics defined in the initial report included small hands and feet, abnormal growth and body composition (small stature, very low lean body mass, and early-onset childhood obesity), hypotonia (weak muscles) at birth, insatiable hunger, extreme obesity, and intellectual disability.

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[1] Butler MG, Lee PDK, Whitman, BY. Management of Prader-Willi Syndrome. 3rd ed. New York, NY: Springer Verlag Inc.; 2006. 0387253971

[2] https://www.fpwr.org/about-prader-willi-syndrome/ Foundation for Prader-Willi Research retrieved October 2016