Prader-Willi Research Priorities

The Foundation for Prader-Willi Research seeks to stimulate research that will improve the lives of individuals with PWS in the near term; thus, we are particularly supportive of research to develop and evaluate new therapeutic approaches and interventions for PWS. We also are interested in supporting innovative research that will lead to significant advances in the understanding of this disorder.

All scientifically meritorious research related to PWS will be considered, but areas of particular interest include:

Genetics: Genotype to Phenotype

- Use of advanced technologies to define and characterize the contribution of PWS-region genes, including noncoding RNAs and protein coding genes, to the PWS phenotype
- Studies to characterize alterations in gene regulation, RNA/protein expression and function in PWS model systems
- Use of cells or tissues derived from animal and cellular models of PWS to explore molecular and cellular changes, and to understand abnormalities in neurobiology, metabolism, behavior and development in PWS
- Phenotypic characterization of PWS cellular models, in particular PWS iPSCs available at: https://www.fpwr.org/ipsc-biobank

Neurobiology of hunger / feeding behavior in PWS

- Application of advanced neurobiology techniques, including brain imaging technology, to understand the basis of hyperphagia and lack of satiety in PWS, with a focus on defining therapeutic targets
- Development of objective biomarkers of hyperphagia
- Studies to understand how failure to thrive progresses to hyperphagia during development
- Studies to define and normalize alterations in hunger/satiety hormones and energy balance in PWS
- Manipulation of existing rodent models to mimic extreme appetite and obesity, to develop and evaluate therapeutic interventions for PWS
    - **Note: FPWR has a novel MAGEL2 deficient rat model available for study*

Neurobiology of cognition deficits, maladaptive behavior and mental illness in PWS

- Use of cellular models of PWS, animal models, and patients, including imaging studies – to understand developmental delay, intellectual disability, maladaptive behaviors, autistic behavior, anxiety, depression and psychosis in PWS
- Studies to understand the genetic underpinnings of psychiatric illness in PWS
- Studies to define early markers / triggers of mental illness
- Development and evaluation of novel therapeutic interventions to mitigate mental health problems
- Approaches to reduce family stress

Clinical Care Research

- Characterization of significant clinical issues in PWS [eg, sleep disturbances, hyperphagia, gastroparesis, seizures, scoliosis, osteoporosis, hypotonia, endocrine dysfunction, anxiety and other clinical problems that impact quality of life]
- Evaluation of therapeutic interventions to address these clinical problems – existing pharmaceutical interventions, behavioral approaches, diet, supplements, devices, etc.
- Investigation of aging-related issues in PWS
- Development and evaluation of methods to overcome learning disabilities common in PWS
- Development of new standards of care

Therapeutics Development – Genetic Therapies

- Proof of concept and feasibility studies to evaluate pharmacological and/or genetic approaches to selectively activate gene expression in the PWS region
- Studies to develop therapeutics to replace PWS gene function(s), including gene replacement

Therapeutics Development – Pharmacologic Therapy (novel and repurposed drugs)

- Development and clinical evaluation of novel or repurposed therapeutic interventions for hyperphagia
- Development and clinical evaluation of novel or repurposed therapeutic interventions for other clinically significant issues in PWS

Resource Development

FPWR will also consider supporting the development of unique in vitro and in vivo resources and models, or bioinformatic capabilities to be shared with the PWS research community

Funded Projects

view all

shRNA/AAV9 Gene Therapy for the Treatment of Prader-Willi Syndrome

Funding Summary This research team is exploring a novel approach to activate maternal gene...

Investigation of cerebellar control of striatal dopamine activity and food intake in PWS

Funding Summary These researchers have demonstrated that activation of a distinct class of...

PWS Smart-Start – A Randomized Clinical Trial

Funding Summary This project aims to evaluate a parent training program (PWS Smart Start) for...

In their own voices: Developing a self-report measure of Hyperphagia for Individuals with PWS

Funding Summary Currently, hyperphagia is often assessed by proxy informants on the Hyperphagia...

About FPWR

The Foundation for Prader-Willi Research (federal tax id 31-1763110) is a nonprofit corporation with federal tax exempt status as a public charity under section 501(c)(3).

Main Menu

⟩ What is PWS

⟩ Our Foundation

⟩ Research

⟩ Advocacy

⟩ Clinical Trials

⟩ Registry

⟩ Events

⟩ Blog

⟩ For Researchers

⟩ Get Involved

Our Mission

The mission of FPWR is to eliminate the challenges of Prader-Willi syndrome through the advancement of research and therapeutic development.

Contact Us

Foundation for Prader-Willi Research
Phone: 888-322-5487
Email: info@fpwr.org

FPWR
340 S. Lemon Ave, #3620
Walnut, CA 91789

Copyright © 2020. All Rights Reserved. Terms of Use. Privacy Policy. Copyright Infringement Policy. Disclosure Statement.