Annual Report

2019

Can you imagine an independent life for our loved ones with PWS?

We can.

FOUNDATION FOR PRADER-WILLI RESEARCH

"There is finally real hope. We have a long way to go, but the possibility of an independent future is not just a dream."

Gillian Segal - age 18, with PWS

Several PWS clinical trials are currently ongoing and recruiting participants for possible treatments for hyperphagia. If any of these drugs are shown to be effective, we could have our first treatment for PWS since growth hormone.

We make strategic investments that accelerate research towards treatments, and a cure, for PWS.

In 2019, FPWR began our Venture Philanthropy program with the objective of helping biotech companies build the resources they need to move their clinical trials for PWS therapies forward. Two companies, Beryl Therapeutics and Inversago Pharma, were selected for early investments.

INVERSAGO PHARMA

BERYL THERAPEUTICS™

When new treatments for PWS have been found, it will be because of all of us.

11,215

donors in 2019

400+

fundraisers

IMPACT 2019

2014: FPWR funds the screening of 10,000 small molecules in search of drug candidates.

2019: Potential drug candidates are in pre-clinical testing for safety and efficacy.

IMPACT 2019

2014: FPWR co-funds the Phase 1 study of Diazoxide Choline Controlled Release (DCCR).

2019: Soleno begins its Phase 3 clinical trial, DESTINY-PWS, in the U.S. and U.K.

IMPACT 2019

2015: FPWR launches the Global PWS Registry to establish a comprehensive natural history of PWS.

2019:

- More than 2,000 patients are enrolled.
- PATH for PWS, a 4-year, $1M study into serious medical events, is launched within the Registry.
- The Registry is used to develop a new clinical trial endpoint measuring anxiety and distress.

IMPACT 2019

2015: FPWR establishes the PWS Clinical Trials Consortium to encourage collaboration and leverage the expertise of industry, academia and patient organizations.

2019: Treatment preferences for people with PWS are published to help guide the FDA for future drug approvals.

IMPACT 2019

2017 / 2019: FPWR individually awards Drs. Chamberlain and Carmichael pilot funding from for their work in PWS genetics research.

2019: Drs. Chamberlain and Carmichael receive a $3M collaborative grant from the NIH to further their work on the molecular underpinnings of PWS.

$3.9 Million Invested in Research

2019

18
Grants

19
Projects

7
Countries

2019 Research Grants

ROLE OF THE PWS GENE MAGEL2 IN THE DEVELOPING HYPOTHALAMUS.
Malcolm Low, PhD, University of Michigan

EVALUATING ENDOSOMAL RECYCLING PATHWAYS IN PRIMARY NEURONS FROM PWS INDIVIDUALS (YEAR 2). Ryan Potts, PhD, St. Jude

INVESTIGATION OF RAPAMYCIN AS A THERAPEUTIC OPTION IN A MOUSE MODEL OF SCHAAF-YANG SYNDROME (SYS).
Christian Schaaf, MD PhD, University Hospital Heidelberg

INVESTIGATING THE CAUSE OF MENTAL ILLNESS IN PWS USING MAGNETIC RESONANCE SPECTROSCOPY (MRS).
Tony Holland, MD, Cambridge University

ROLE OF MAGEL2 IN EXCITATORY SYNAPSE FUNCTION.
Deniz Atasoy, PhD, University of Iowa

ROLE OF CENTRAL AMYGDALA ANOREXIA NEURAL CIRCUITS IN PRADER-WILLI SYNDROME
Haijing Cai, PhD, University of Arizona

ALLELE-SPECIFIC DNA REPLICATION TIMING OF THE PRADER-WILLI LOCUS AND ITS INFLUENCE ON NEURONAL DEVELOPMENT.
Amnon Koren, PhD, Cornell University

NEURONAL MECHANISMS OF DEVELOPMENTAL COGNITIVE IMPAIRMENT IN THE SNORD116 DEL MOUSE MODEL FOR PWS.
Timothy Wells, PhD, Cardiff University

IMPROVING MUSCLE STRENGTH AND MUSCLE MASS IN PEOPLE WITH PWS.
Nora Shields, PhD LaTrobe University Australia

CRISPR-MEDIATED MOLECULAR DISSECTION OF PRADER-WILLI SYNDROME (YEAR 2).
Michael Talkowski, PhD, Massachusetts General Hospital

A MOUSE MODEL TO ASSESS GENETIC THERAPIES FOR PRADER-WILLI SYNDROME.
Jim Resnick, PhD, University of Florida

TARGETING SMCHD1 TO ADDRESS THE UNDERLYING CAUSE OF PWS AND SYS.
Marnie Blewitt, PhD, Walter + Eliza Hall Research Institute, Australia

INVESTIGATING A NEW POTENTIAL TARGET FOR TREATMENT IN PRADER-WILLI SYNDROME..
Lauren Rice, PhD, University of Sydney, Australia

THE FUNCTIONAL DEVELOPMENT OF HUNGER NEURONS IN PRADER-WILLI SYNDROME.
Marcelo Dietrich, PhD, Yale University

ENHANCING SATIATION SIGNALING TO REDUCE OVEREATING AND OBESITY IN PRADER-WILLI SYNDROME.
Edward Fox, PhD, Purdue University

INFLUENCES OF SOCIAL COGNITION AND REWARD ON ASD SYMPTOMS AND BEHAVIOUR IN PWS.
Louise Gallagher, PhD, Trinity College Dublin, Ireland

GENOMEWIDE IDENTIFICATION OF mRNA SITES OF 2'-O METHYLATION TARGETED BY SNORD116 snoRNAS.
Gordon Carmichael, PhD, University of Connecticut.

CELLULAR ROLE OF MAGEL2 IN PRADER-WILLI AND SCHAAF-YANG SYNDROMES (YEAR 2).
Rachel Wevrick, PhD, University of Alberta

We have invested $13 Million into PWS Research Grants since 2003.

FOUNDATION FOR PRADER-WILLI RESEARCH

Directed Research Projects

FPWR-Led Research

The Global PWS Registry

PATH for PWS

PWS Clinical Network

PWS Weight Project

PWS Genetic Therapy Workshop

Pre-Clinical Animal Model Network

Mental Heath Guidebook Development

PWS Clinical Trial Consortium

Resource Development

Generation of Non-Human Primate Model of PWS.
Juan Carlos Ispuzia-Belmote, PhD., Salk Institute

Development of SYS Patient Specific Isogenic IPS Cells. Michael Talkowski, PhD., Harvard

PWS Genome Analysis. Liz Worthey, PhD.
University of Alabama at Birmingham

PWS Mouse Model Development,
Medical Research Council, UK

Dental Pulp Stem Cell Resource. Larry Reiter, PhD.
University of Tennessee Health Sciences Center

PWS Brain Biobank - Autism BrainNet Partner

PWS Stem Cell Biobank, University of Connecticut

Venture Philanthropy

Cannabinoid-1 Receptor Blockade to Treat Hyperphagia, Obesity and Related Metabolic Disorders in PWS. Inversago

Preclinical evaluation of HU-671
Beryl Therapeutics

Clinical Care

CBDV vs Placebo in Children with PWS
Eric Hollander, PhD., Montefiore Medical Center

Study of Growth Hormone in Adults with PWS
Laura DeGraaf, PhD., Erasmus Medical Center

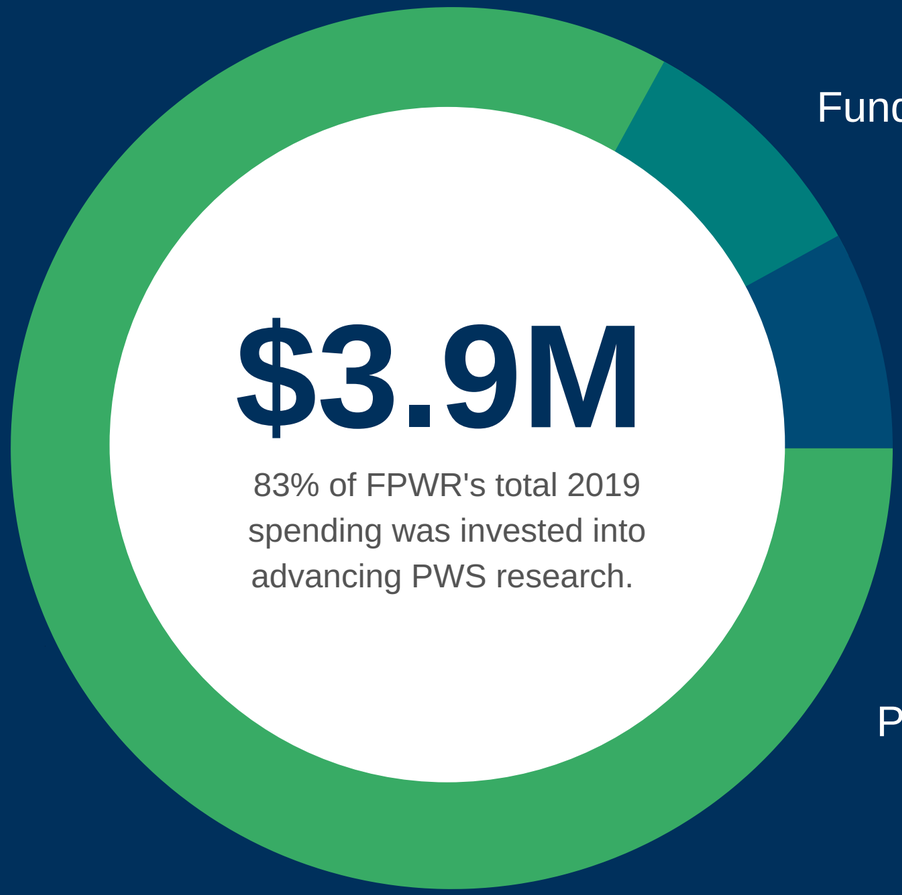
FOUNDATION FOR PRADER-WILLI RESEARCH

PWS is not incurable.
It just hasn't been cured yet.

In 2019, we brought together a 'dream team' of experts in PWS genetics to discuss the current landscape of genetic therapy and discuss how to most effectively establish the feasibility of genetic therapy for PWS. These scientists are exploring how the latest genetic therapy techniques can be applied to PWS and are developing a series of recommendations to help guide FPWR's next investments into this exciting area of research.

Jim Resnick, University of Florida
FPWR Scientific Advisory Board

2019 FINANCIALS



EXPENSES

Programmatic Expenses	$3,921,238
Mission Support	$414,543
Fundraising Expenses	$412,390
Total Expenses	**$4,748,172**

- Unaudited financials

FOUNDATION FOR PRADER-WILLI RESEARCH

FOUNDATION FOR
PRADER-WILLI
RESEARCH

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Join Us | 2020

Together, we will find treatments for PWS.

#WhateverItTakes
#FPWR

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