



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



FOUNDATION FOR
PRADER-WILLI
RESEARCH



We're not just waiting and hoping for new treatments, and a cure, for Prader-Willi syndrome.
We're aggressively doing something about it.

The Foundation for Prader-Willi Research

From Humble Beginnings...

Nearly 20 years ago four moms set out to improve the lives of their loved ones with PWS. They began small, funding several academic research grants. Since then...

- FPWR has invested **\$16mm** into more than **200 research projects**.
- **200+ publications** have resulted from FPWR funding, building a robust body of knowledge on PWS.
- An additional **\$250mm+** has been invested by Industry.
- **20 companies** are in various stages of drug development for PWS.
- We have developed a **robust library of resources** to support ongoing discussions with the FDA in support of new treatments for PWS.



And yet there's much work to be done!

It Takes A Village....

When you support FPWR, you are joining a team of parents who want to change the future for their loved ones with PWS. Your fundraising efforts:

- ✓ Identify new potential treatments
- ✓ Expand the drug development pipeline
- ✓ Advance genetic therapies
- ✓ Improve clinical care
- ✓ Establish and maintain research tools
- ✓ Develop advocacy resources for new treatments



Supporting Research to Identify New Ways to Treat PWS

FPWR is dedicated to supporting research to advance the understanding and treatment of Prader-Willi syndrome (PWS). We are particularly interested in **supporting research that will lead to new interventions** to alleviate the symptoms associated with PWS.

Our grant program supports innovative projects across a broad range of disciplines, including basic science, resource and therapeutic development, and preclinical and clinical studies.

More than 100 publications in the medical literature have resulted from FPWR funding.

In 2020, FPWR invested **\$1.6M** to advance **18 grants** and **15 directed projects**.

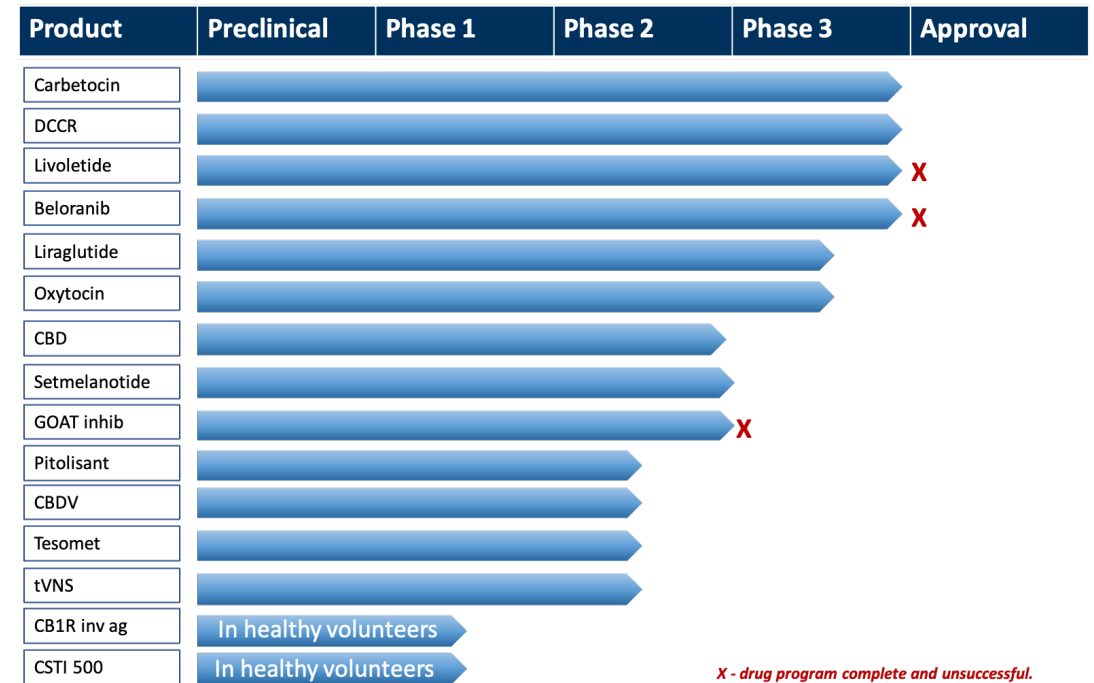


Expanding the Drug Development Pipeline for PWS

We spend hundreds of hours each year on the phone and in person, talking with companies helping them understand PWS and the unmet needs of our community, facilitating clinical trial design and working to develop trials that have the best chances for success.

We work with each company to de-risk the drug development process and speed up the timeline to taking a drug to the FDA for approval. We are committed to working with any company that has the potential to develop a medical product that might benefit individuals with PWS.

Our goal is to provide the patient perspective to facilitate the development of treatments that are safe and effective for PWS, helping to advance potential treatments for PWS into clinical trials and beyond.



Currently there are 12 compounds in Phase 1 – 3 clinical trials for PWS.

Advancing Genetic Therapies for PWS

Genetic therapies have the potential to provide transformative treatments in PWS but much still needs to be learned, and tested, to determine what benefits this approach will have for individuals with PWS. The following approaches are currently being investigated, focused on activating the PWS genes on maternal chromosome 15:

- Designer epigenome modifiers
- CRISPR-based epigenome modification
- Small molecule modulators of the epigenome
- Targeting the epigenetic modifier, SMCHD1 in stem cells
- Targeting epigenetic regulators of the PWS region on maternal chromosome 15

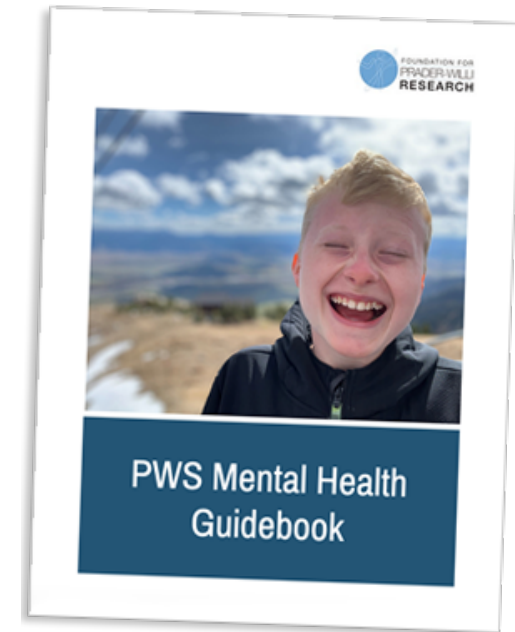


Learn more at <https://www.fpwr.org/genetic-therapy-for-prader-willi-syndrome>

Improving Clinical Care for People with PWS

On average it takes 12 years to bring a new drug to market, but **people with PWS and their families need treatments NOW!** Clinical care research aims to find near-term solutions that will alleviate the symptoms of PWS (such as behavioral challenges, scoliosis, seizures, GI motility) to improve quality of life. A few of our highlighted projects include:

- Mindfulness Behavioral intervention
- Guanfacine clinical trial for the treatment of aggression
- The PWS Mental Health Guidebook
- Investigations into diet and nutrition interventions
- Acceptance & Commitment Training for dads
- Improving Social skills across the ages
- Improving orthopedic care: hip dysplasia and scoliosis treatments



Establishing Resources for the PWS Research Community

Research tools, such as PWS cell lines and animal models, are critical to support PWS research and accelerate therapeutic development. **FPWR has established and maintains numerous resources** that are available to our research community and support research at all stages of therapeutic development.

One tool, **the Global PWS Registry**, launched in 2015, allows patients around the world to share information about PWS on developmental history, medical complications, and quality of life. **Data from the registry is shared back to the PWS community and used by researchers and scientists to advance PWS research.** It has also been critical in documenting the natural history of PWS which is needed to develop clinical trials and advocate with the FDA.



The Global PWS Registry provides a comprehensive database of individuals with PWS.

[Learn more at: https://www.fpwr.org/the-global-pws-registry-empowering-families-advancing-research](https://www.fpwr.org/the-global-pws-registry-empowering-families-advancing-research)

Advocating for New Treatments for PWS

FPWR is working diligently to build resources that address the FDA's call for patient experience data, demonstrate the high disease burden of PWS and establish the need for effective therapies. **We have established a library of more than one dozen resources that have been shared with the FDA** to support the review and approval of treatments for PWS. These resources:

- Demonstrate through family stories, surveys, and a best-worst scaling study that **hyperphagia is “the” aspect of PWS that families want addressed** through new therapies, followed by other critical behavioral issues such as anxiety and aggression.
- Demonstrate that **caregivers are willing to accept considerable risk** in exchange for modest improvements in hyperphagia. In the absence of a ‘cure’, families would welcome treatments that alleviate PWS symptoms.
- Demonstrate the **tremendous unmet medical needs of individuals with PWS** and the considerable burden of disease, showing that caregiver burden in PWS exceeds that of caregivers for stroke and Alzheimer disease.
- **Share patient input** from individuals with PWS, speaking on their own behalf. This patient experience data is complemented by natural history studies that provide a critical information to support clinical trials.

Questions? We Would Love to Chat With You!

Contact us today to learn how you can get involved with the mission of FPWR.



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