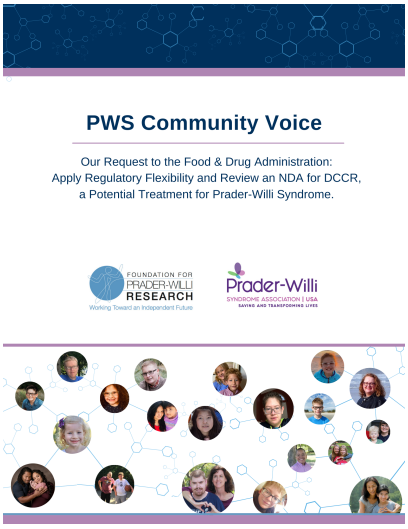
FOUNDATION FOR PRADER-WILLI RESEARCH
Working Toward an Independent Future

PWS affects approximately 350,000 people worldwide. Currently there are no treatments for the most debilitating challenges of the syndrome. We are working to change that.

Thank you for your continued support and generosity to FPWR and PWS research! Because of you, we are moving closer to treatments for PWS.

FPWR actively supports the advancement of clinical trials at all stages of development. In addition to funding early drug development research, we continue to document the unmet medical needs of our population, meet with the FDA, and evaluate data trends obtained from the Global PWS Registry, which now has more than 2,000 patients enrolled.

This year has been unique. With two completed Phase III Pivotal trials, **our advocacy work has come front and center as we advocate to the FDA for new treatments for our loved ones**. You can see our most recent FDA submission on our blog at: https://www.fpwr.org/blog




## DOUBLE YOUR IMPACT!

When you donate in May, our matching donor will MATCH YOUR GIFT, doubling your impact and accelerating treatments for PWS. Donations may be made at www.fpwr.org/doublemygift

## Advocating For New Treatments for PWS

Perhaps now, more than ever before, we are beginning to see the fruits of more than a decade of labor. With your support, FPWR has been 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. This spring, in response to the FDA's decision that an additional trial would be necessary for DCCR, a potential treatment for PWS, **FPWR and PWSA | USA submitted a 143 page community response letter requesting the FDA to apply regulatory flexibility when evaluating potential treatments for PWS.**

Thanks to your support and the participation of hundreds of PWS community members, **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.

Your donations have made this critical work possible. **Thank you!**

# FPWR is the largest funder of PWS research in the world.

We have invested more than $16,000,000 in PWS related research.

More than **$16M** invested since 2003

FPWR investments have attracted more than **$250M** in additional funding for PWS research

**$100,000** The amount that will be **matched** before May 31. **Donate today!**

**204** research grants

**7** Critical research tools support and de-risk research efforts

**196** Publications in medical journals

## Vagus Nerve Stimulation May Provide Treatment for Behavior

Temper outbursts and disruptive behaviors are among the most challenging aspects of PWS, both for the individual with PWS and their family. **These behaviors can severely limit the entire family's ability to do many of the things most of us take for granted,** like going to restaurants, or attending family events.

Vagus Nerve Stimulation (VNS) has been approved by the FDA to treat epilepsy and chronic recurrent depression. Now, **new evidence supports the use of VNS to reduce temper outbursts and improve disruptive behaviors in individuals with PWS.**

**Thanks in part to your research investment, FPWR was able to support an investigation of VNS** in a small number of PWS individuals in the UK. After 9 months of wearing a t-VNS device for four hours daily, four out of five participants had a statistically significant reduction in both the number and severity of temper outbursts.

**With your continued support, FPWR aims to advance a clinical trial of VNS** so we may collect the safety and efficacy data needed to gain FDA approval and make this technology available to our loved ones with PWS. Watch for more to come as we prepare for this big endeavor!

## Venture Philanthropy Advances Potential Therapeutics for PWS

**FPWR has a multi-prong approach for advancing treatments for PWS.** One strategy, venture philanthropy, allows FPWR to identify and invest in early-stage pharma companies with the aim of accelerating drug development.

**One of our earliest investments supported the Phase 1 study of DCCR**, which completed a Phase 3 Pivotal trial in 2020 and is now undergoing discussion with the FDA. **More recently, we made a small investment in Inversago Pharma** to support their development program of INV-101 for the treatment for PWS. INV-101 has since been granted a Rare Pediatric Disease designation by the FDA and **your support has helped advance this potential therapeutic from preclinical trials into a Phase I trial** in healthy individuals and we look forward to a trial in individuals with PWS.

We're thrilled to see the continued development of promising drugs for PWS and thank our supporters for making this possible.

## About FPWR

FPWR's vision is a world where those with Prader-Willi Syndrome lead full, healthy, independent lives. Currently, there are no treatments for the most debilitating symptoms our loved ones face, but 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 was established with one aim in mind: to **eliminate the challenges of Prader-Willi syndrome** through the advancement of research and therapeutic development. Our focus is funding research that will lead to new treatments and, one day, a cure for PWS. Through the management of our world-class research programs and PWS research tools, FPWR will advance PWS research in order to develop new treatments and improve the health and well-being of those with PWS.

**Contact Us**

340 S. Lemon Ave, Suite 3620, Walnut, CA 91789 phone 888-322-5487 email info@fpwr.org

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