PWSA | USA Bi-Weekly Pulse: September 6, 2024

Enhancing the Quality of Life and Empowering those Affected by PWS

PWSA | USA
PULSE

Vol. 106

September 6, 2024

www.pwsausa.org | 941.312.0400

Rare diseases, including PWS, make up 6% to 8% of the global population. Patients with rare diseases frequently face a challenging odyssey to find timely and accurate diagnosis. Increasing the options of newborn screening (NBS) programs is a key step for improving health outcomes for those living with rare diseases. Approximately four million babies are screened annually for at least 31 serious disorders that can be detected at birth.

Newborn blood spot screening involves taking a small sample of blood to find out if your baby has one of about 28 rare health conditions. While newborn screening for Prader-Willi syndrome may be feasible (according to this [2018 article from the National Library of Medicine]), and early diagnosis is critical to receiving valuable interventions and therapies, PWS is still not part of newborn screening. Some of the barriers to having PWS added to NBS may include budget, time, current genomic data and prevalence of the condition. Genetic sequencing has allowed for individuals to receive earlier diagnosis, however, there is still advocacy to be done.

The testing for PWS is initiated when the individual meets the criteria of physical attributes and symptoms. This process may take months to years, during which time individuals miss valuable therapies and treatment while their families lose access to resources and education to improve the quality of life for all.

Dini Rao shared her family's story of receiving a late diagnosis for their daughter, and how the reliance on physical attributes and symptoms to diagnose individuals negatively affects families of color. Read Dini's blog at the button below.

READ BLOG HERE

PWSA | USA'S SPOTLIGHT ON HOPE

"Ramsey has a smile that can light up the whole state of Kentucky. He has three older sisters who encouraged him to move and play, helping Ramsey to beat the odds and walk at 14 months old! He loves to dance, flirt with brunettes, and gives the best hugs. Ramsey never gives up and inspires everyone around him to keep going. We can't wait to see this little man's bright future ahead!"

SHARE YOUR SPOTLIGHT ON HOPE

EVENTS & FUNDRAISERS

We need more artwork for the International Rare Aware Art Share! Submit by September 15th!

United in HOPE
2025 PWS CONFERENCE
Prader-Willi Syndrome Association | USA | IPWSO International Prader-Willi Syndrome Organisation | Foundation for Prader-Willi Research

PWSA | USA 2024
Rare Aware Art Share

Our Rare Aware Art Share is an international opportunity to showcase the artistic voices of individuals living with PWS. As we are preparing to gather with our community at the 2025 International PWS Conference next June, we are seeking the voices of individuals around the world to share with us something that is unique about where they live. This is a wonderful opportunity to learn about individuals with PWS in a variety of cultures and places worldwide. **The deadline to submit artwork is September 15th**. Please share this opportunity with your PWS community and let's encourage individuals from all over the world to share their artistic voices! Learn more and submit artwork using the button below.

RARE AWARE ART SHARE

REGISTER FOR THE 2025 PWS CONFERENCE

Join Us in Creating a Tribute: Submit Your Photo for the 2025 PWS Conference Mural

With the 2025 International PWS Conference approaching, we're creating a special mural to showcase the diversity and beauty of our incredible PWS community. This mural will be on display during the 2025 conference at the Arizona Grand Resort & Spa. We want photos of your loved one to be a part of it! Submit a picture of your PWS warrior, even if you can't join us in person, at the button below. The pictures shared will help create a display of hope that everyone can take pride in. Let's come together to celebrate and inspire!

SUBMIT PHOTO HERE

2025 Conference Hotel Rooms are Filling Up Fast!

Planning to attend the 2025 International PWS Conference? Don't wait—book your stay at the Arizona Grand Resort & Spa today! With 55% of rooms already reserved, availability is limited. In addition, there are no double queen rooms left for June 21, 2025, and only king rooms remaining on June 22, 2025. You can reserve now and cancel up to a week before the event without any charges, and you won't be billed until check-in. Secure your room now and ensure your spot at this exciting event! Book your stay at the button below or call 877-800-4888 to make a reservation.

HOTEL RESERVATIONS

ARIZONA GRAND RESORT & SPA'S WEBSITE

The Inaugural Residential Providers Conference was a Success!

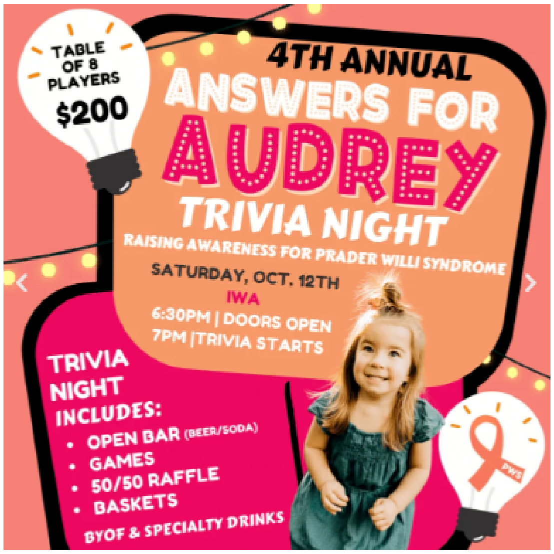
PWSA | USA's first-ever Residential Providers Conference was held August 18-20, 2024, both virtually via Zoom and in-person in Savannah, GA. The event offered a variety of lectures and workshops where more than 100 professionals from around the country met to share their knowledge and experiences. We are grateful and inspired by the people who choose to step in and care for our loved ones with PWS when they move into residential facilities. Your knowledge and dedication help our loved ones live independent, healthy, and happy lives. Thank you for taking the time to learn more about PWS and how to help our loved ones thrive!

"It is such an honor to be part of a group of committed and passionate people who have dedicated their careers to bettering the lives of people with PWS! I was thrilled to be a part of a conference that focused so much on the essential roles that we, as providers, play in the lives of people of PWS!" – Brittni Kliment, M. Ed from Latham Centers, Conference Attendee

"Rejuvenating! Being in a space with others who share the same hopes, dreams, goals, and passion for the PWS community is inspiring. Knowledge is power and we're all leaving with knowledge to fuel our superpowers! Until next time!" – Nicole Tingley, Director at DD Homes, Conference Attendee

READ MORE HERE

Upcoming Fundraisers



Hosted by Michelle Spring

Please join us at the 4th Annual (Trivia) Answers for Audrey event on Saturday, October 12th, 2024! Doors open at 6:30 pm and trivia starts at 7:00 pm. Our daughter, Audrey Rose Spring, was diagnosed with Prader-Willi syndrome on December 19, 2020. Proceeds raised will be divided between the Spring family's medical needs and PWSA | USA, a foundation that helped us in our darkest days and that is on the cutting edge of research, medical trials, and life-changing treatments. We cannot thank you enough for your interest in our fundraising event! Please contact Michelle or Michael with any interest or questions:
Email: AnswersForAudrey@gmail.com Phone : 636-634-5601

Please note, any donation made via the online platform is tax deductible. Donations made the night of the event will benefit the Spring family directly and therefore are not tax deductible. Due to the nature of this event, no refunds will be granted.

When: **Saturday, October 12, 2024, at 6:30 p.m.**
Where: **Incarnate Word Academy** (2788 Normandy Drive, Bel-Nor, MO, Missouri)

REGISTER TO ATTEND

PWSA | USA'S UPCOMING EVENTS

PWS hope united
BUILDING BRIGHT FUTURES FOR PWS FAMILIES
Prader-Willi Syndrome Association | USA

Interested in hosting a PWS Hope United event in your city? Contact hopeunited@pwsausa.org and click the button below to get started!

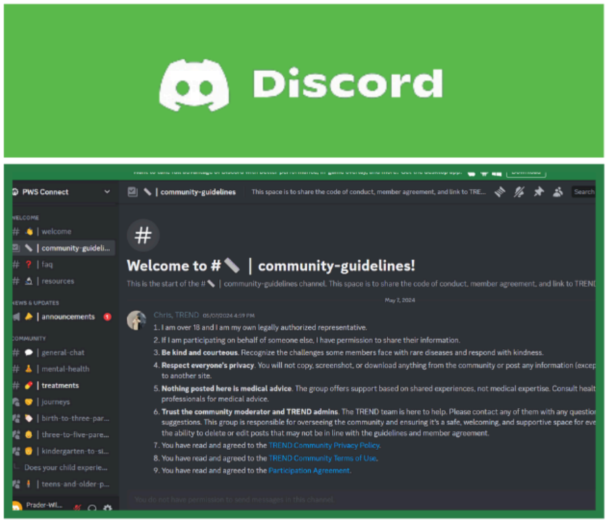
Become a Hope United Champion

PWS CONNECT PODCAST UPDATES

Exercise is not a dirty word! Kat Lucero, PWSA | USA Board Member and certified Integrative Health and Wellness Coach, and Stacy Ward, PWSA | USA's CEO, discuss the healthy and unhealthy ways we use exercise.

Kat shares the many benefits exercise has on our physical and mental health, the variety of ways caregivers can fit exercise and movement into their busy lives, how children benefit from their parents' understanding of health and wellness, group exercise, and more. Does exercise look different as we age? Listen to find out.

LISTEN ON PODBEAN



This space is to share the code of conduct, member agreement, and link to TRE...

Welcome to # | community-guidelines!

This is the start of the # | community-guidelines channel. This space is to share the code of conduct, member agreement, and link to TREND

May 7, 2024

Chris, TREND 05/07/2024 4:59 PM

1. I am over 18 and I am my own legally authorized representative.
2. If I am participating on behalf of someone else, I have permission to share their information.
3. **Be kind and courteous.** Recognize the challenges some members face with rare diseases and respond with kindness.
4. **Respect everyone's privacy.** You will not copy, screenshot, or download anything from the community or post any information (excep to another site.
5. **Nothing posted here is medical advice.** The group offers support based on shared experiences, not medical expertise. Consult healt professionals for medical advice.
6. **Trust the community moderator and TREND admins.** The TREND team is here to help. Please contact any of them with any questio suggestions. This group is responsible for overseeing the community and ensuring it's a safe, welcoming, and supportive space for eve the ability to delete or edit posts that may not be in line with the guidelines and member agreement.
7. You have read and agreed to the TREND Community Privacy Policy.
8. You have read and agreed to the TREND Community Terms of Use.
9. You have read and agreed to the Participation Agreement.

You do not have permission to send messages in this channel.

We invite you to continue the conversations and connect with other PWS families in our **PWS Connect Discord groups**. Sign up to join today at the button below!

JOIN TODAY!

ADVOCACY

PWSA | USA Receives #RAREis Grant

We're excited to announce that PWSA | USA has been selected as a 2024 #RAREis Global Advocate Grant recipient by the #RAREis program from Amgen biopharmaceutical! "The *#RAREis* Global Advocate Grant was established in 2022 to support the rare disease community by providing financial assistance to global advocacy groups working to advance, educate and address the needs of the community." We're motivated to continue making a positive impact for the rare disease community by expanding our efforts in Prader-Willi syndrome education and advocacy as we work to address the needs of all those impacted.

Learn more about the #RAREis Grant at the button below.

LEARN MORE

FDA Advisory Committee to Review DCCR

The FDA has announced its plan to conduct an Advisory Committee Meeting as part of its review of Soleno Therapeutics' DCCR New Drug Application. The FDA convenes Advisory Committees to provide independent expert advice that contributes to the agency's regulatory decision-making. As part of the Advisory Committee Meeting, interested community members are encouraged to share their views. **A date has not yet been announced for the meeting, but we expect it to be held toward the end of the review process, which has a target completion date of December 27, 2024.**

LEARN MORE

FAMILY SUPPORT

PWS in Adulthood Blog Series: Creating a Safe Home

What does it look like to create a home for your adult with PWS who lives independently from you? PWS parent Nancy Christoff shares how her family makes it work for her daughter, Nicole. From the role of administration manager (filled by Nancy herself) to the various personal care assistants, she breaks down the designated duties that each role takes on. She also discusses the two main daily issues, Nicole's trust and naivety and her ability to access food from external sources and how these are managed. For parents wondering about options for their loved one as they age, here is another example of what a living situation could look like. If your loved one with PWS is an adult and you would

like to share some of your wisdom and experience for our PWS in Adulthood blog series, please email africke@pwsausa.org to learn how.

READ HERE

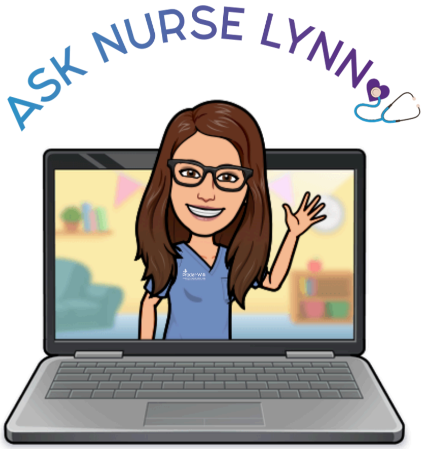
Ask Nurse Lynn

Question *(Male, 25 years-old, unknown subtype):*

"My son, age 25, was previously taking Propranol for blood pressure and anxiety. We were noticing increased anxiety and PCP just switched him to Lisinopril and Venlafax ER 75. Are these two medications found to be safe for patients with PWS?"

Visit our blog to read Nurse Lynn's response.

READ NURSE LYNN'S RESPONSE



Do you have a non-emergency medical question about Prader-Willi syndrome? Do you have a non-urgent medical question that keeps coming up, but you forget to ask every time you're at the doctor? Perhaps your question applies to numerous families who will benefit from hearing the answer.

Now you can **Ask Nurse Lynn,** PWSA | USA's very own PWS Nurse.

ASK YOUR QUESTION HERE

Family Support Webinars

Friendship and Beyond: Navigating Relationships and Sexuality in PWS with Patrice Carroll
When: Tuesday, September 17, 2024 | 5:00 p.m. PST / 8:00 p.m. EST

This session on friendships, relationships and sexuality will focus on the unique challenges that people with PWS face in forming and maintaining friendships as well as navigating their sexuality. Discussions will center around healthy relationships, addressing social deficits and creating an appropriate education in relation to sexuality. An emphasis will be on the importance of effective education, communication, and the dignity of risk.

REGISTER HERE

Watch the recording of our August Family Support Webinar, **Individualized Health Plans (IHP) for Students with Prader-Willi syndrome** with Dr. Destiny Pacha, at the button below.

CLICK HERE TO WATCH

DOWNLOAD THE PRESENTATION HERE

Celebrate National Grandparents Day!
September 8, 2024

We're honoring grandparents in the PWS community! September 8th is National Grandparents Day. We offered the community an opportunity to share a message to their grandparent. You all sent in beautiful messages and pictures to share. These will be rolling out all weekend on PWSA | USA's social media pages as we honor those grandparents. If you would like to read through the messages submitted, click the button below.

GRANDPARENTS DAY MESSAGES

RESEARCH

Calls for Abstracts for 2025 International PWS Conference

The abstract submission process is open for the 2025 International PWS Conference Clinical and Scientific Program and the Professional Providers and Caregivers Program! Read more information about how to apply using the button below. Our United in Hope: International PWS Conference will shape the future of PWS care and research. This 5-day event marks the first joint conference between IPWSO, PWSA | USA, and FPWR, and symbolizes a historic milestone! Register today!

Call for Abstracts

Professional Providers Program Submission
Deadline: November 5, 2024

Clinical & Scientific Program Submission
Deadline: January 31, 2025

APPLY HERE

REGISTER HERE

ECHO 4 PWS Health Care Series:
Three Upcoming ECHO Webinar Opportunities

PWS Health Care Providers!

Register for PWSA | USA's Upcoming ECHO 4 PWS Webinars

PWSA | USA is hosting **three** upcoming **ECHO 4 PWS** webinars.

Date: Wednesday, September 11, 2024 at 2:00 PM PST / 5:00 PM EST
Speakers: Roxann Diez Gross, PhD, CCC/SLP, and Ann Scheimann, MD, MBA
Topic: Feeding and Swallowing
Location: ZOOM

REGISTER HERE

This presentation will enhance the knowledge of PWS assessment and management, focusing on feeding and swallowing issues through a multidisciplinary approach.

Date: Thursday, October 24, 2024 at 10:00 AM PST / 1:00 PM EST
Speaker: Deepan Singh, MD
Topic: Psychiatry and PWS
Location: ZOOM

REGISTER HERE

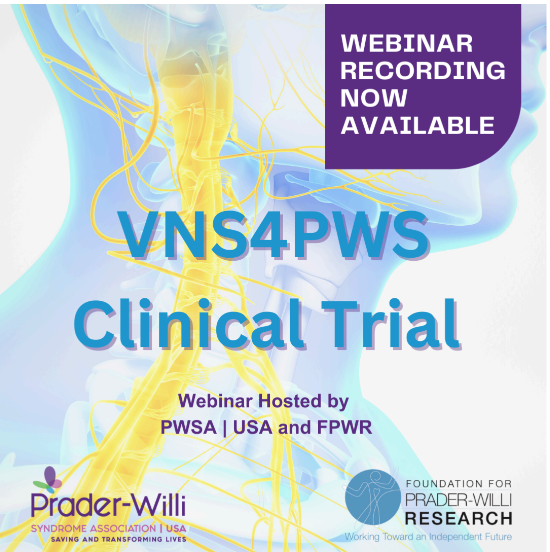
Date: Monday, November 11, 2024 at 2:00 PM PST / 5:00 PM EST
Speaker: Daniela Rubin, Ph.D.
Topic: Exercise Physiology
Location: ZOOM

REGISTER HERE

These webinars are for health care professionals to attend only. We will have recordings of these webinars available on PWSA | USA's YouTube channel following the presentations at this link. Find more information about ECHO 4 PWS at the button below.

PROJECT ECHO

WATCH: VNS4PWS Clinical Trial Webinar



PWSA | USA and FPWR co-hosted a webinar for families to learn more about the VNS4PWS clinical trial. VNS4PWS is a phase 3 clinical trial evaluating the effectiveness of a Vagus Nerve Stimulation (VNS) device for improving disruptive behaviors and temper outbursts for people with PWS. If your loved one is between the ages of 10 and 40, you may be eligible to participate! (fpwr.org)

Here is a brief summary of key takeaways and resources discussed during the webinar:

- This trial is assessing whether a wearable device decreases temper outbursts and challenging behaviors.
- The device should be worn for 4 hours per day. These 4 hours do not need to be consecutive.
- This is a phase 3 trial is currently enrolling individuals with PWS ages 10 – 40.
- In order to qualify for this study, you cannot be participating in any other study.

If you have any questions or need further information, feel free to reply to this email or reach out to us at info@pwsausa.org or visit https://www.fpwr.org/vns4pws.

WATCH HERE

Harmony Biosciences Opens New Clinical Trial Site in Colorado

Harmony Biosciences has announced an additional clinical trial site for its TEMPO PWS study:

- **Aurora, CO** | Colorado Children's Hospital

Harmony Biosciences' TEMPO study is a global Phase 3 trial investigating pitolisant as a potential treatment for excessive daytime sleepiness (EDS) in individuals aged six years and older with Prader-Willi syndrome. Pitolisant is a medication that could help manage sleepiness and behavioral issues in people with PWS. Find all 12 TEMPO trial site locations and learn more at the button below.

LEARN MORE ABOUT TEMPO

PWSA | USA ANNOUNCEMENTS

PWSA | USA Resource Spotlight

PWSA | USA is proud to offer a dedicated **PWS Clinics** webpage on our website, providing a valuable resource for families seeking specialized care for loved ones with Prader-Willi syndrome. This comprehensive directory includes clinics across the country that are equipped to offer expert guidance and support for individuals with PWS. Whether you're looking for medical care, therapies, or nutritional advice, our PWS Clinics page connects you to trusted professionals who understand the unique needs of the PWS community. Visit our website at the button below to explore this essential tool for families.

PWS Clinics

Home » PWS Clinics

Name	Location	Contact/Phone/ Web	Providers/ Clinicians	Frequency	Funding/Billing
Rady Children's Hospital	7920 Frost Street, Suite 200 San Diego, CA	Dr. Lynne Bird (858) 966-5840 Website	Clinical Geneticist, who is a pediatrician Acts as a Case Manager Endocrinologist, Nutritionist & Social Worker	--	Grant from Prader-Willi California Foundation provides salary support for the Social Worker and Nutritionist Physicians each submit a bill
CHOC – Children's Hospital of Orange County Division of Endocrinology Prader- Willi Clinic @ Thompson Autism Center	170 S. Main St. Orange, CA 92868	Phone Numbers: 714-288-7651 714-462-1837 Fax: 714-288-7379 Website	Endocrinologist, Endocrine NP, Social Worker, Nutritionist, Behavioral Pediatrician & Pulmonologist	Twice a month	Funding/Billing: Private insurance, MediCal Grants from Prader-Willi California Foundation
			Clinical Social Worker, Endocrine Nurse		

PWS CLINICS

Your gift to PWSA | USA ensures individuals and families living with Prader-Willi syndrome have the connection, guidance, and hope they so desperately need. Thank you!

Donate

Privacy Policy | Unsubscribe

Prader-Willi Syndrome Association | USA

1032 E Brandon Blvd #4744 Brandon, FL 33511

PWSA | USA Bi-Weekly Pulse: September 20, 2024

Enhancing the Quality of Life and Empowering those Affected by PWS

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PULSE

Vol. 107

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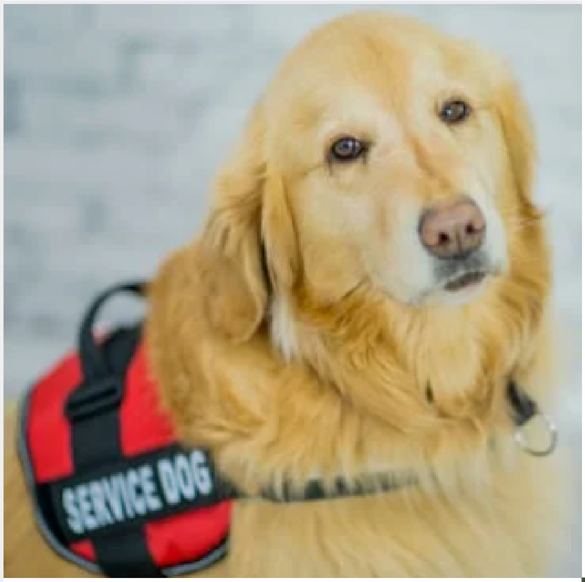
National Service Dog Month is celebrated every September to honor and recognize the outstanding ways specially trained dogs assist people around the world. They are the "eyes" for people who are blind, perform tasks for those with mobility issues, alert people to impending seizures, heart rate spikes, or blood sugar irregularities, soothe veterans who suffer from PTSD, help children with social and emotional challenges, and so much more. Could service dogs help our loved ones with PWS? According to the following blogs, they already are.

From the ability to calm a person on the verge of an emotional meltdown, distract from skin-picking or potential food seeking, and alerting for elopement, service dogs can be a wonderful asset when facing the challenges of PWS. Getting a service dog may not be an easy road, but a worthy one. Read the blogs below to learn how some families with PWS have navigated this path, what it looks like with a trained dog present in the life of someone with PWS, and how you can begin your search for an adorable and helpful new family member.



Getting a Service Dog; How, Why, and Who Pays for It?

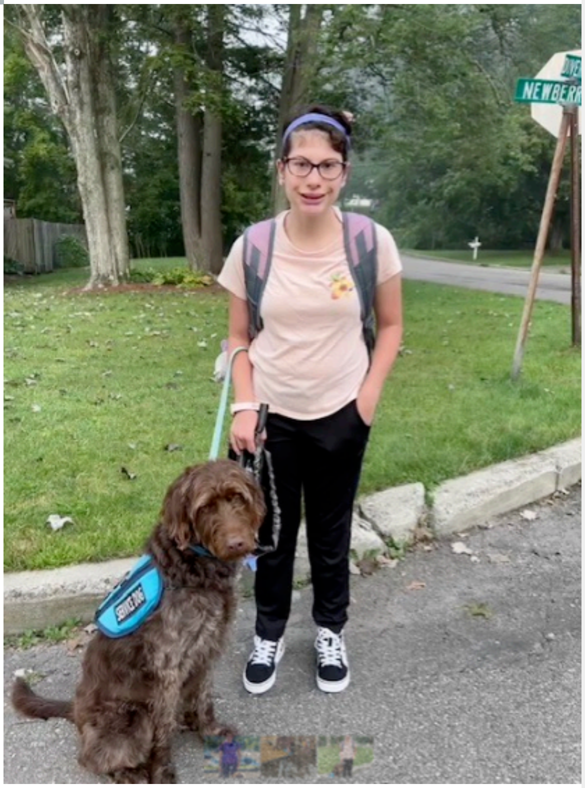
In a few months, Pia Dorson and her family will be welcoming a service dog into their home. Read about how they started the process, why they are seeking a service dog, and who is paying. *"As a whole, it is an emotional support dog for our entire family."*

READ ARTICLE

Obtaining and Training a Service Dog, contributed by Rachel Johnson

"Having a service dog requires dedication to the process and a willingness to overcome some obstacles along the way. If you are willing to put in the effort required to train and maintain a service dog, the payoff is immeasurable. We absolutely cannot imagine our lives without Stanley. He has been a huge support to Ellie, in fact our whole family, in this PWS journey."

READ BLOG



Service Dog at Home and at School, contributed by Winnie White

"Sandy Kay's dog's name is Fitz. He will be a facility dog during school hours and when school is out, he will become Sandy Kay's personal service dog. He is being dually trained and certified as a service dog and a facility dog."

READ BLOG

PWSA | USA'S SPOTLIGHT ON HOPE

"I am so grateful that Freya goes to a school that challenges and encourages her, where the teachers seem to know the right amount of softness and grit to help Freya participate in every activity her school mates do. I am grateful that for the second time I have been there to watch Freya climb a literal mountain, knowing all of the metaphorical ones she continues to climb."

Freya, a 12-year-old with PWS, went on her first backpacking trip with her class where she not only carried her pack three miles into camp, but the next day made a five-mile round trip hike to the top of Mount Eddy (9,025 feet).

READ MORE HERE

We want to celebrate your loved one!

Our **Spotlight on Hope** series strives to share and celebrate the successes of our loved ones. No success is too small! Whether they took their first steps, had a great time at the school dance, made a shot in a basketball game, or are working towards a goal, we want to celebrate it all.

If you'd like to honor your loved one and share their success story with the PWS community, please fill out our Spotlight on Hope form below. We're looking forward to celebrating with you!

SHARE YOUR SPOTLIGHT ON HOPE

EVENTS & FUNDRAISERS

2025 Conference Hotel Rooms Filling Up Fast!

June 24, 2025	June 25-26, 2025	June 26, 2025	June 27-28, 2025
PWS Clinical Workshop	Clinical & Scientific Program	Adults with PWS Program	Family Program PWS Camps
Invite Only	Professional Providers Program	*1/2 Day*	

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SUBMIT PHOTO HERE

Upcoming Fundraisers

Giving Pump supports PWSA | USA! For the months of September and October, the Mt. Ivy Country Corner Shell Gas Station in Rockland County, NY, is teaming up with Shell's Giving Pump Initiative to raise money for PWSA | USA, thanks to PWS mom Dominique Valenti! Simply pull up to any of the purple-wrapped pumps at the Shell gas station located at **361 Route 202, Pomona, NY 10970**, fuel up as usual, and a portion of the proceeds will be donated directly to support families affected by Prader-Willi syndrome. There's no extra cost to you – the more gas pumped, the more that can be donated! A big thank you to Dominique, Shell USA, and those who fill up their tanks through the Giving Pump!

Tee off for a great cause at RMC's inaugural golf tournament, benefiting innovative research and community support initiatives. Enjoy a round of golf while making a positive impact on those living with Prader-Willi syndrome. The RMC Foundation works to provide funding to PWSA | USA's Advocacy Programs. These Programs help enrich the lives of people living with Prader-Willi Syndrome.

When: **Monday, September 30, 2024,** *8:30 a.m. shotgun start*
Where: **Ritz-Carlton Members Golf Club,** *Bradenton, FL*

REGISTER TO ATTEND

PWSA | USA'S UPCOMING EVENTS

Thank you, Gill family!

The **Gavin Gill Classic Golf Tournament** was an amazing day! Hosted by Joe Gill and his family in honor of Gavin, who's 17 and living with PWS, the event took place on Saturday, September 7th at the North Hill Country Club in Duxbury, MA—and it completely sold out! We're so grateful to everyone who came out to golf, sponsor, and support. A huge thank you to the Gill family for putting together such a special event, and to everyone who helped make it a success. Your support means the world to PWSA | USA!

PWS hope united
BUILDING **BRIGHT** FUTURES FOR PWS FAMILIES
Prader-Willi Syndrome Association | USA

Interested in hosting a PWS Hope United event in your city? Contact hopeunited@pwsausa.org and click the button below to get started!

[Become a Hope United Champion]

PWS UNITED PODCAST UPDATES

PWSA | USA's Podcast Has a New Name

As part of our ongoing efforts to best serve the Prader-Willi syndrome community, we are introducing a new name for our podcast. Moving forward, PWSA | USA's podcast will be known as **PWS United**! To learn more about why we decided to make the change, please read our blog at the button below.

If you have already followed our podcast on your podcast app, you don't need to do anything. New episodes will still automatically download to your app. If you have not already followed the podcast, please do! Learn how to listen and follow HERE.

PWS United

Prader-Willi Syndrome Association | USA

NAME CHANGE UPDATE

Latest PWS United Episode

In this PWS United episode, we're reflecting on **Direct Support Professionals Week**, which took place between September 8th and 14th. This special week is dedicated to honoring the incredible individuals who provide essential care and support to those living with developmental disabilities, including PWS. PWSA | USA CEO, Stacy Ward, leads an important discussion on the history and significance of this week, and how direct support professionals continue to make a difference in the PWS community. Joining Stacy are four remarkable experts: Patrice Carroll from Latham Centers, Nicole Tingley from DD Homes, Lynn Garrick from AME Community Services, and Marguerite Rupnow from Prader-Willi Homes. Together, they share how their organizations celebrated the week and continue to highlight the vital role direct support professionals play in enhancing lives every day.

LISTEN ON PODBEAN

ADVOCACY

PWSA | USA'S

SPOTLIGHT ON ADVOCACY

We are excited to share a powerful example of group advocacy that recently took place in Minnesota! Jen Andrews and her daughter Jojo, Denise Servais and her daughter Maya, and Lynn Garrick and her son Alex, met with Rep. Angie Craig (D-MN) to discuss the challenges faced by those living with PWS. During the meeting, everyone had an opportunity to share personal stories and the ongoing needs for our community, including better healthcare access, education, and support. Rep. Craig, who was one of the elected officials to sign our congressional letter to the FDA in support of PWS treatments, showed genuine interest in the community's concerns. This meeting marked a meaningful step forward in advocating for the needs of PWS families! You can read the full recap of the meeting and testimonials from those who attended by clicking the button below.

READ HERE

FAMILY SUPPORT

World Narcolepsy Day, September 22
A Personal Experience with Narcolepsy and PWS

"Ever since I can remember, I have been tired. Not the kind of tired you feel when you stay up too late or how you feel at the end of a busy day. The tired I feel is more like how you would feel if you were forced to stay awake for days. This feeling would keep me from doing most things and if I tried to go do anything fun, I would not be able to stay awake to enjoy them."

World Narcolepsy Day is September 22nd. Justice Rickenbach, an adult living with PWS, wrote about her experiences of narcolepsy. Read her blog at the button below.

READ HERE

PWS Blog: PWS Mom's Hiking Weekend

What started as a simple question turned into an incredible weekend of laughter, friendship, blisters, love, organizational planning, and reminders of the fact that the best way to truly live this special needs life is to walk side by side with others. The inaugural PWS Moms' Hiking Weekend took place in the Adirondack Mountains of New York between June 20-23, 2024. Read Amy's blog at the button below to learn more about this hiking weekend and how you can participate in the next one!

READ HERE

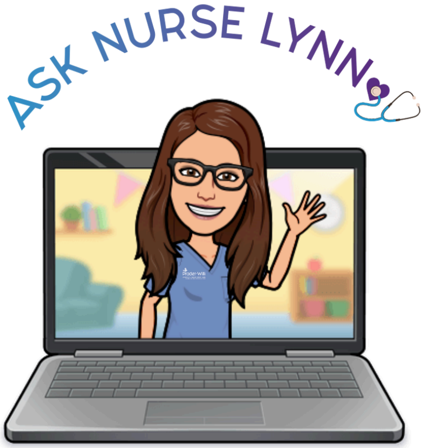
Ask Nurse Lynn

Question *(Male, 33 years-old, Deletion):*

"My son is mostly behaving, and he is not aggressive. However, lately he gets up in the morning upset, anxious, and wants more food. For the rest of the day, he is OK. He used to take Cipralex. He does not want to take it anymore. Not that I have seen much of a difference. How can I help him????"

Visit our blog to read Nurse Lynn's response.

READ NURSE LYNN'S RESPONSE



Do you have a non-emergency medical question about Prader-Willi syndrome? Do you have a non-urgent medical question that keeps coming up, but you forget to ask every time you're at the doctor? Perhaps your question applies to numerous families who will benefit from hearing the answer.

Now you can **Ask Nurse Lynn,** PWSA | USA's very own PWS Nurse.

ASK YOUR QUESTION HERE

Family Support Webinars

Support PWS Research and Create Community
When: Tuesday, October 29, 2024 | 5:00 p.m. PST / 8:00 p.m. EST

Join us for an insightful and informative webinar to learn how you can help advance PWS research while also finding and building community support. PWS parents, Maria Picone and Chris DeFelice, are excited to share how their personal journey with PWS led them to create the **PWS Connect** channel on Discord, a place where caregivers come together to share their journeys, experiences, questions, and advice to support each other and directly impact the progression of PWS research. This webinar will explain the **What, Why,** and **How** of **TREND Community** and the PWS Connect Discord group, and include time for your questions. We hope you join us for a new wave of PWS insights and growing research! Register at the button below.

REGISTER HERE

Watch the recording of our September Family Support Webinar, **Friendship and Beyond: Navigating Relationships and Sexuality in PWS** with Patrice Carroll, LICSW, at the button below.

CLICK HERE TO WATCH

RESEARCH

Calls for Abstracts for 2025 International PWS Conference

The abstract submission process is open for the 2025 International PWS Conference Clinical and Scientific Program and the Professional Providers and Caregivers Program! Read more information about how to apply using the button below. Our United in Hope: International PWS Conference will shape the future of PWS care and research. This 5-day event marks the first joint conference between IPWSO, PWSA | USA, and FPWR, and symbolizes a historic milestone! Register today!

Call for Abstracts

Professional Providers Program Submission
Deadline: November 5, 2024

Clinical & Scientific Program Submission
Deadline: January 31, 2025

APPLY HERE

REGISTER HERE

ECHO 4 PWS Health Care Series:
Three Upcoming ECHO Webinar Opportunities

PWS Health Care Providers!

Register for PWSA | USA's Upcoming ECHO 4 PWS Webinars

PWSA | USA is hosting **two** upcoming **ECHO 4 PWS** webinars.

Date: Thursday, October 24, 2024 at 10:00 AM PST / 1:00 PM EST
Speaker: Deepan Singh, MD
Topic: Psychiatry and PWS
Location: ZOOM

REGISTER HERE

Date: Monday, November 11, 2024 at 2:00 PM PST / 5:00 PM EST
Speaker: Daniela Rubin, Ph.D.
Topic: Exercise Physiology
Location: ZOOM

REGISTER HERE

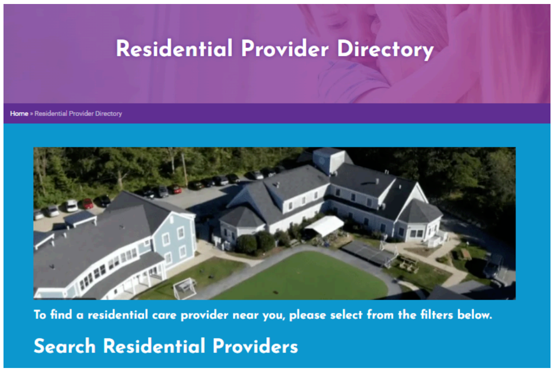
These webinars are for health care professionals to attend only. We will have recordings of these webinars available on PWSA | USA's YouTube channel following the presentations at this link. Find more information about ECHO 4 PWS at the button below.

PROJECT ECHO

PWSA | USA ANNOUNCEMENTS

PWSA | USA Resource Spotlight

Are you considering a residential living setting for your loved one with PWS but don't know where to start? PWSA | USA offers a comprehensive list of residential facilities around the country that can help support families with PWS. The decision to move your loved one to a residential facility will likely spark a lot of questions and considerations. We're here to help you determine why and how this might be the right path for your family. Visit our website at the button below to explore this essential tool for families.



Residential Provider Directory

Home » Residential Provider Directory

To find a residential care provider near you, please select from the filters below.

Search Residential Providers

RESIDENTIAL PROVIDER DIRECTORY

Your gift to PWSA | USA ensures individuals and families living with Prader-Willi syndrome have the connection, guidance, and hope they so desperately need. Thank you!

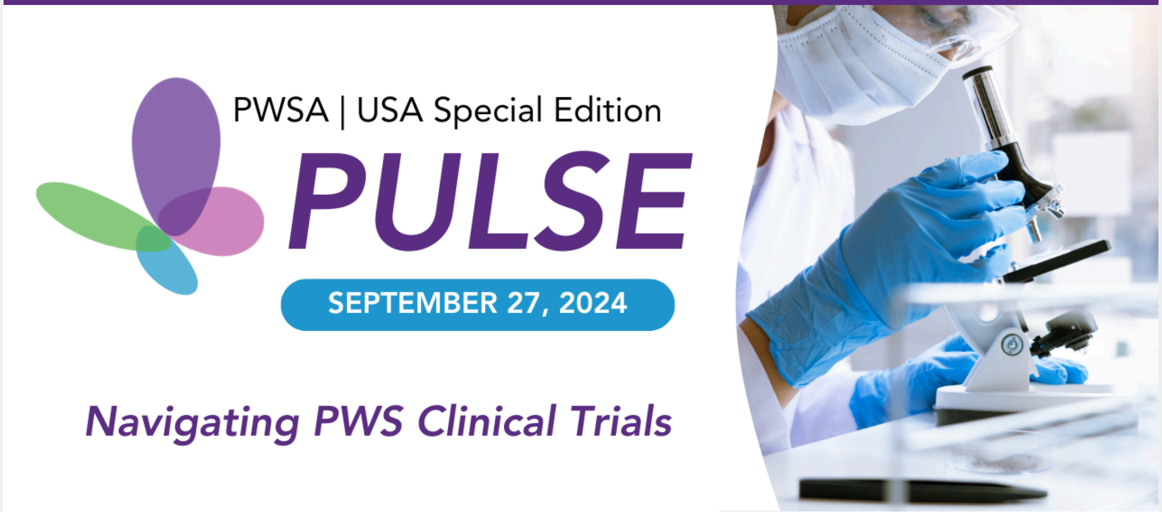
Donate

Privacy Policy | Unsubscribe
Prader-Willi Syndrome Association | USA
1032 E Brandon Blvd #4744 Brandon, FL 33511

PWSA | USA Special Edition Pulse: September 27, 2024

www.pwsausa.org | 941.312.0400

Donate



What is a clinical trial? It's a dry term for incredibly valuable research that can further understanding of PWS and get new treatment options into the marketplace. A clinical trial is a *"study in which one or more human subjects are prospectively assigned one or more interventions (which may include placebo or other control) to evaluate the effects of those interventions on health-related biomedical or behavioral outcomes."* (NIH's Definition of a Clinical Trial | Grants & Funding)

But what does that actually look like for families?

How do you find a clinical trial, and then a site that works for you? How long does a trial last? Who plans and pays for the travel if needed? What is the difference between Phase 1 and Phase 3, or a Phase 5? What paperwork and tests are required? What happens when the trial is over? Who receives the information and what happens to the data collected? How does a trial drug get to the FDA?

We know that families have a lot of questions about clinical trials. We hope to answer some of those questions in this Special Edition Pulse and in our new ongoing series that follows a family through their process of participating in a clinical trial.

While clinical trials are important to further PWS research and develop treatment options for families, enrolling your loved one in a trial may not be an easy decision. The power to make good decisions for your family comes from knowledge. We strive to provide families with that knowledge.

"Participating in a trial is a very personal decision. What works for one family may not work for another – be it the drug, the travel, the administration of the medicine, etc. The drug may not work for your child, whereas it may work for another child. The research will help others with PWS, but that does NOT mean you have to participate. You also may do the screening with the hopes of being accepted, only to be told you do not qualify. It takes a big commitment, with time and schedules. We chose to wait until something local opened up, as we have other children, and we were unable to travel and find care for our other children. Again, it's a personal decision, with no right or wrong answer."

- Susan Fries, mom to Roselyn (7) in the Carbetocin trial

Freya's Clinical Trial Journey:
How We Got Here

The decision to enroll in a clinical trial involves the consideration of many factors. Some families may be open to the idea from the start. Other families may never consider it an option. Most families fall in between.

Our communications coordinator, Anne Fricke, and her daughter, Freya (12), have recently begun their clinical trial journey with Harmony's Pitolisant trial, aimed at fighting excessive daytime sleepiness. In an effort to help other families have a clearer understanding of what that process and experience may look like, Anne and Freya have both consented to sharing their journey with the PWS community. "How We Got Here" is the first in a series documenting one family's experience as they hesitantly enter the realm of medical research. You can begin reading the series by clicking the button below.

READ MORE HERE

Freya's Clinical Trial Journey: First Appointment

In part 2 of our Clinical Trial series, Anne recounts the details of how they began the process of enrolling in the Pitolisant clinical trial and what those steps are. She then offers a detailed list of how they prepared for the first appointment and what happened at that initial screening appointment. Families can learn more about who makes the travel plans and pays for it, what paperwork is needed, what tests are done at the appointment, the clinical trial app, and expectations for caregivers in the initial part of the process. If you are interested in reading a firsthand account of what a clinical trial appointment may look like, please click the button below.

LEARN MORE HERE

Lessons Learned: PWS Families Reflect on the Clinical Trial Experience

"Enrolling in a clinical trial can seem overwhelming. The unknowns can be scary for the parent and the child. We have learned to ask questions to find out exactly what is going to happen at the appointments to help ease any fears and anxiety. The clinical trial sites have all been extremely helpful to minimize added stress."

- Kristi Rickenbach, mom to Justice (20) enrolled in Pitolisant trial

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"I feel like we are all waiting for that magic fix, and if it works for someone else then my kid must drink the Kool-Aid and it'll work for them too. But we deep down know it doesn't work that way as much as we all want it to. And it ultimately may take a cocktail to help. It's so close we can touch it. We are driven by not only the hope that's staring us in the face, but also the fear of that hope being ripped away in an instant, just like it has been over and over again the last few years. It's terrifying living in a constant state of hyper-everything - awareness, hope, anxiety, fear, vigilance, protectiveness, research, fight or flight, etc."

Susan Fries, mom to Roselyn (7) enrolled in the Carbetocin trial (aimed at treating hyperphagia and anxiousness), shared part of her family's experience with the clinical trial. You can read more at the button below.

If you have a loved one enrolled in a clinical trial and would like to share your experience, please email africke@pwsausa.org.

READ HERE

"Gavin is non-speaking. We explained what we were going to be doing and why. I don't know that he fully understands the trial, but we have told him how proud we are of him and that he is helping his friends. He always gets a big smile."

- Amanda DiPascale, mom to Gavin (7) enrolled in Carbetocin trial

Clinical Trials 101

What is a Clinical Trial?

A clinical trial is a research study assessing the safety, effectiveness, and benefits of new treatments or interventions. Designed with strict protocols involving human participants, these trials compare new approaches to existing ones. They're essential for advancing medical knowledge and enhancing patient care.

How do Clinical Trials Work?

Participants, selected by criteria, are split into treatment and control groups. The treatment group receives the experiment, while the control might get a placebo or standard care. They're monitored for safety and outcomes, with committees overseeing progress. After the trial, data is reviewed to gauge the intervention's safety and efficacy.

Clinical Trial Timeline

Phase 1: Safety & Dosing Information

In this initial phase, the treatment's safety, dosage, and potential side effects are assessed with a small group of healthy volunteers or affected individuals. Researchers monitor participants closely.

Phase 2: Efficacy Studies

In Phase 2, the treatment is tested on a larger group with the specific condition, like Prader-Willi Syndrome. The focus is on evaluating its effectiveness and monitoring for side effects or adverse reactions.

Phase 3: Comparative Effectiveness Study

In this phase, the treatment is compared to current standards or placebos. The goal is to further evaluate its effectiveness, safety, and benefits against other options. Researchers maintain close monitoring and data collection.

Phase 4: Post-Market Monitoring

In Phase 4, the treatment is implemented in clinical practice. Long-term studies assess its real-world effectiveness and longevity beyond trials. These studies offer insights into the treatment's impact on patient outcomes and quality of life.

Phase 5: Implementation and Follow-up Studies

Phase 5 involves the implementation and dissemination of the treatment in clinical practice. Long-term follow-up studies track patients' progress and outcomes over an extended period to assess the treatment's real-world effectiveness, durability, and benefits beyond the clinical trial setting. These studies provide valuable insights into the treatment's performance and its impact on patients' lives.

PWS Clinical Trials Currently Enrolling

HARMONY BIOSCIENCES

Drug: Pitolisant

Phase: 3
Drug targets: Excessive Daytime Sleepiness

CLICK HERE TO LEARN MORE

FOUNDATION FOR PRADER-WILLI RESEARCH
VNS4PWS

Drug: VNS4PWS

Phase: 3
Drug targets: Behavior

CLICK HERE TO LEARN MORE

ACADIA™

Drug: Carbetocin

Phase: 3
Drug targets: Hyperphagia

CLICK HERE TO LEARN MORE

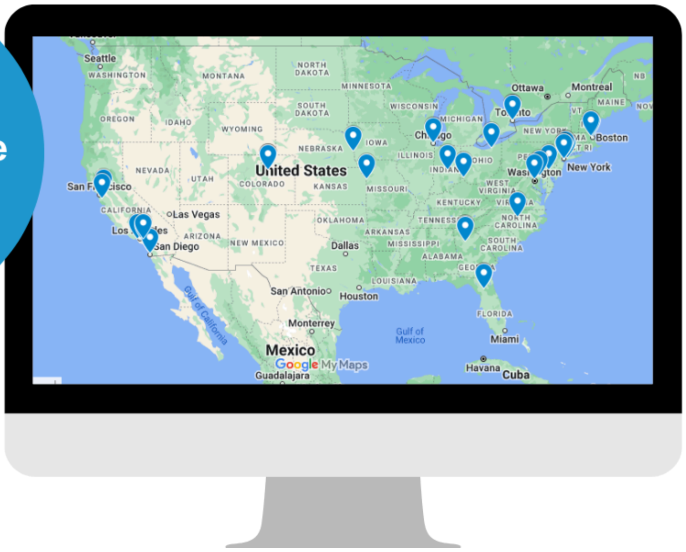
Montefiore
Inspired Medicine

Drug: Cannabidivarin (CBDV)

Phase: 2
Drug targets: Behavior

CLICK HERE TO LEARN MORE

Click Here to find a trial site near you!



PWS Clinical Trials Not Currently Enrolling

aardvark therapeutics

Drug: ARD-101

Phase: 2 *(complete)*
Drug targets: Hyperphagia

CLICK HERE TO LEARN MORE

RG
GEDEON RICHTER

Drug: RGH-706

Phase: 2 *(enrollment complete)*
Drug targets: Hyperphagia

CLICK HERE TO LEARN MORE

SOLENO
THERAPEUTICS

Drug: DCCR

Phase: 3 *(complete - New Drug Application submitted to the FDA)*
Drug targets: Hyperphagia

CLICK HERE TO LEARN MORE

Prader-Willi
SYNDROME ASSOCIATION | Ohio
SAVING AND TRANSFORMING LIVES

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