PWSA | USA Pulse Newsletter: September 5, 2025

Enhancing the Quality of Life and Empowering those Affected by PWS

PWSA | USA
PULSE

Vol. 130

September 5, 2025

www.pwsausa.org | 941-312-0400

Each year, during National Direct Support Professional Week, we pause to celebrate the incredible individuals who dedicate their careers to supporting people with disabilities. At PWSA | USA, we know firsthand how essential these professionals are to the lives of children and adults living with Prader-Willi syndrome. Direct Support Professionals are the backbone of care. They ensure dignity, safety, independence, and opportunity for those they support. From carefully managing food security, to navigating challenging behaviors, to offering encouragement and joy in daily activities, DSPs make an extraordinary difference every single day.

As we honor DSPs this week, we encourage families and communities to take a moment to recognize the DSPs in their lives. A handwritten note, a kind word of gratitude, or a small token of appreciation can make a lasting impact. At PWSA | USA, we see you, and we thank you. To every Direct Support Professional who supports a person with Prader-Willi syndrome: your work matters, YOU matter. You are making the world a safer, kinder, and more hopeful place for our loved ones and for our entire community.

In 2024, PWS United aired an episode with Stacy Ward, PWSA | USA's CEO. She spoke with direct support professionals who have made a lasting difference in the PWS community. You can listen to that episode at [Ep8 Direct Support Professionals Week Panel Discussion | PWS United](). We will continue this new tradition next week with a new episode of PWS United, once again honoring DSPs in the PWS community. Follow PWS United on your favorite podcast platform or at [PWS United | PWSA | USA]() to be sure you never miss an episode.

You can read Stacy's full DSP Recognition Week blog using the button below.

READ HERE

PWSA MEMORIES

Since our founding in 1975, Prader-Willi Syndrome Association | USA (PWSA | USA) has been more than an organization. We have been a trusted voice, advocate, and lifeline for families and individuals affected by Prader-Willi syndrome. Just as our mission has grown and adapted to meet the needs of our community, our logo and tagline have also evolved over the years to reflect strength, resilience, and hope. We invite you to take a look back on the evolution of our logo and tagline, and the story it tells about 50 years of hope, resilience, and community. Learn more by clicking the button below.

READ HERE

SHARE YOUR MEMORY

PWSA | USA'S

SPOTLIGHT ON HOPE

"Freya's class went on another backpacking trip to the Trinity Alps, a mountain range in northern California. We were slightly concerned about some fires in the area, but rain began to fall heavily as we loaded the packs onto our backs at the trailhead. About a mile in, the skies opened up with thunder, lightning, and hail. Freya was scared. Honestly, I was scared as well. But Freya kept moving forward. She jumped every time thunder loudly clapped and was honest with me about her fears, ranging between fear, panic, and the thought that we were going to die. (She still managed a snack request at one point, to which I pointed out that this was not snack weather.) Toward the end of the hike to the campsite Freya began singing and let me know it was her way of calming herself. The bad weather was lifting, and I could more fully appreciate that she was using a valuable skill - self-soothing.

We got to the campsite cold and soaked through, like everyone else. Freya did not complain. She did not say she wanted to go home or that she had bad feelings about what we had just hiked through. The rest of the trip was glorious and though the hikes were tough, Freya kept a positive attitude for (almost) all of it. Four days later, we hiked out of the mountains singing "We Shall Not be Moved" as the sun rose on what was shaping up to be a beautiful, sunny mountain morning. We talked about how scared we had been on the hike in, but that it felt good to be brave and courageous.

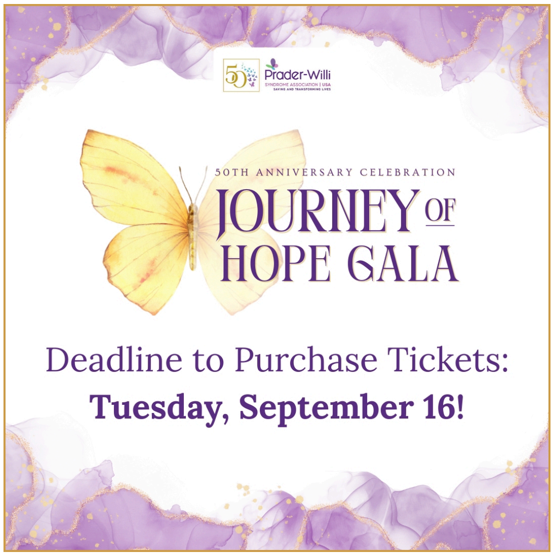
If we had not been on a class trip, I would have certainly turned around once the bad weather started. This was another valuable lesson for me on just how strong, capable, brave, and adventurous Freya can be."

- Submitted by Anne Fricke (mom to Freya, 13, living with PWS)

SHARE YOUR SPOTLIGHT

EVENTS & FUNDRAISERS

Journey of Hope Gala – Ticket Deadline Approaching!



Prader-Willi
SYNDROME ASSOCIATION | USA
SAVING AND TRANSFORMING LIVES

50TH ANNIVERSARY CELEBRATION
JOURNEY OF HOPE GALA

Deadline to Purchase Tickets:
Tuesday, September 16!

We are just weeks away from PWSA | USA's 50th Anniversary Journey of Hope Gala on Friday, September 26, 2025, at The Ritz-Carlton in St. Louis, MO! Don't miss this unforgettable evening as we celebrate five decades of impact, hope, and community. Here are a few important event announcements:

- **Registration Deadline:** Tickets must be purchased by Friday, September 19th. Secure yours today at the button below!
- **Can't attend in person?** You can still be part of this milestone by making a tax-deductible donation in honor or memory of a loved one. **Click here to make a donation**.
- **Silent Auction:** Our virtual Silent Auction will be live soon and is open to the public - anyone, anywhere, can bid on more than 25 incredible items! Stay tuned for the official launch link.
- **Hotel Update:** Our discounted room block at **The Ritz-Carlton, St. Louis**, is now full. If you'd like to stay onsite, you may call the hotel directly at (314) 863-6300 or book online at their standard rate. We also have a list of alternate hotel options in the Clayton area. You can find this list at the button below.

We cannot wait to gather with our community and honor 50 years of progress. See you in St. Louis!

PURCHASE GALA TICKETS

OTHER HOTELS

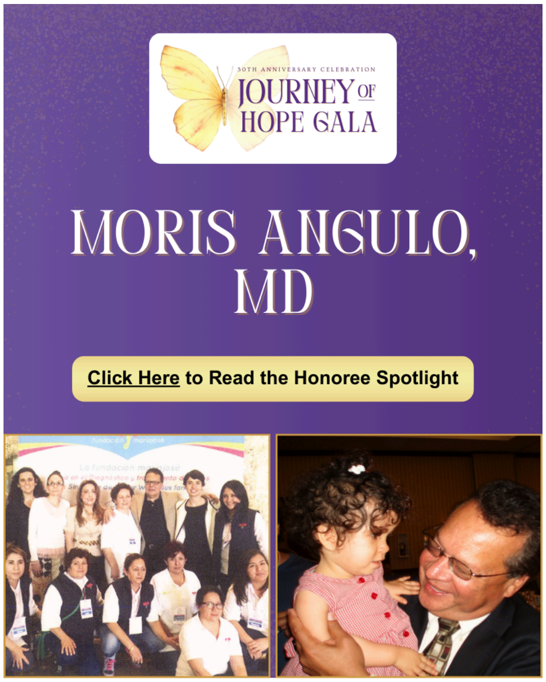
Journey of Hope Gala Honoree Spotlight Series

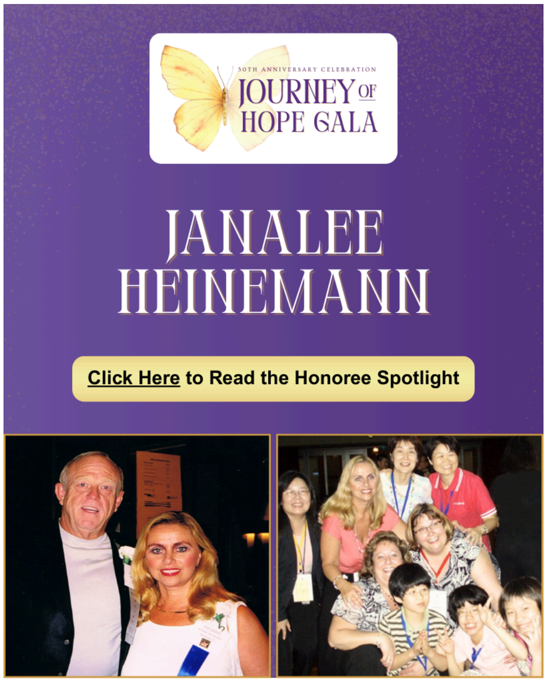
This year, as PWSA | USA celebrates **50 years** of hope, advocacy, and impact, we’re proud to recognize the pioneers and visionaries who helped lead the way. In the weeks leading up to our *Journey of Hope Gala* in St. Louis, MO, we will share a series of blog spotlights to honor the 12 incredible individuals being recognized at the event.

These honorees have shaped the Prader-Willi syndrome landscape in profound and lasting ways – through groundbreaking research, tireless advocacy, compassionate care, and unwavering support for families and individuals living with PWS.

We hope you’ll join us in celebrating their legacies by reading their stories and attending our Journey of Hope Gala. Click the images below to read spotlights, featuring Moris Angulo, MD, and Janalee Heinemann.

Visit our Journey of Hope Gala website to find the full list of honorees.





THANK YOU, JOURNEY OF HOPE GALA SPONSORS

LEGACY SPONSORS

SOLENO THERAPEUTICS

SEMINOLE Hard Rock HOTEL & CASINO HOLLYWOOD, FL

PIONEER SPONSORS

5 BROTHERS PRODUCE

ACADIA

Get Your Tickets Now for Cocktails for a Cause!

When: **Saturday, October 18 @ 6:30 PM EDT**
Where: **Brown's Brewing Company Malt Room** *(417 River St, **Troy, NY** 12180)*

Join PWSA | USA CEO Stacy Ward and her family for an unforgettable evening of support, hope, and community. Hosted at the scenic and historic Brown's Brewing Company Malt Room, this event will bring together families, friends, and supporters for an inspiring evening of cocktails, conversation, and compassion, all aimed at raising awareness and funds for Prader-Willi syndrome.

Guests will enjoy signature cocktails, delicious hors d'oeuvres, and the unique ambiance of the Malt Room, known for its charm and exceptional service. The night will feature engaging speakers, including families impacted by PWS, as well as opportunities to learn more about the syndrome and how funds raised will directly benefit those affected by it. The event will also offer a raffle, silent auction, and other interactive ways to contribute to this important cause. Click the button below to purchase tickets and to learn more!

COCKTAILS FOR A CAUSE

Thank You, Cocktails for a Cause Sponsors

PLATINUM SPONSOR

SOLENO THERAPEUTICS

GOLD SPONSOR

ACADIA

SILVER SPONSOR

CATHOLIC CHARITIES
CELEBRATING 40 YEARS
DISABILITIES SERVICES

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

A Podcast for the PWS Community

PWS United podcast just turned 1! We decided to celebrate with a look back at our top 10 episodes from this past year. From nutrition advice to sibling support, adults with PWS and advocacy work, the BIPOC community and Nurse Lynn submissions, Carrie and Anne share some clips to celebrate this growing library of information and support and inspire new listeners to take advantage of this incredible resource for the PWS community. Share this podcast with a friend or family member to help spread PWS awareness.

Listen to PWS United on your favorite podcast app or by clicking the button below.

LISTEN ON PODBEAN

ADVOCACY

Call to Action for PWS Families!

State P&T (Pharmacy and Therapeutics) Committees and DUR (Drug Utilization Review) Boards need to hear directly from you – the parents, caregivers, and family members who understand the real-life impact of hyperphagia (excessive hunger) in PWS. One of the medications under review is Vykat XR, the first-ever hyperphagia treatment specifically for individuals living with PWS that was approved by the FDA in March 2025.

We're asking families to submit written testimony to help decision-makers understand why access to this treatment is so urgently needed. If you'd like to learn more and submit a testimony for your state listed, please click on the links below.

Minnesota Meeting: Wednesday, September 17, 2025
Montana Meeting: Wednesday, September 17, 2025
Alaska Meeting: Friday, September 19, 2025

Mental Health Advocacy and Femi's Heart Foundation

This past weekend, I had the opportunity to attend a powerful panel on mental health hosted by **Femi's Heart Foundation**. I was honored to speak about the importance of support groups and the vital role they play for families and individuals navigating challenges, especially in the world of special needs.

In many of our cultures, there is still a deep taboo around both mental health and special needs. This silence often prevents families from seeking early intervention or accessing the resources that can truly transform lives. Without these supports, individuals are left without the tools they deserve to thrive. Breaking this cycle begins with open conversations, awareness, and compassionate community support.

The story of **Femi's Heart Foundation** itself is an inspiring reminder of resilience and hope. Femi was only 10 years old when she received a heart transplant. Doctors said she wouldn't survive, but she proved them wrong, living five more beautiful years. After her passing, her mother channeled her grief into purpose by creating this foundation to help others, honor Femi's memory, and share her story of courage.

Her legacy continues to inspire, and this panel was a reminder that through storytelling, breaking taboos, and building community, we can bring light and resources to those who need them most.

-Submitted by Sarah Kasaby, mom to Khaled, living with PWS

FAMILY SUPPORT

Grandparent Perspectives

In honor of the upcoming Grandparents Day (September 7), we reached out to our community of grandparents to learn more about their experiences, joys, and advice. We invited them to respond to prompts on how they support parents, spend time with their loved one with PWS, what brings them joy as a grandparent, and any advice they may have for other grandparents. Some shared how they are able to help in day-to-day tasks, and others who don't live in

the same area shared how they enjoy their visits and offer support from afar. To all of our grandparents on this upcoming Grandparents Day – we thank you for your love, your compassion, your support, and for continuing to show up in our lives. Our lives are better because you're here.
You can read this blog using the button below.

READ HERE

PWSA | USA is recruiting residential homes or programs to help pilot a new PWS Residential Training Program!

PWS Home Base

WHAT IS THE PROGRAM ABOUT?

WHAT DOES PARTICIPATION INVOLVE?
- Completing online questionnaires about your work experiences, stress, and use of support strategies
- Attending training sessions (synchronous and asynchronous) delivered via telehealth
- Joining follow-up meetings to share feedback on the program and its implementation

BENEFITS OF PARTICIPATING
- Free access to structured training and resources tailored for residential programs serving individuals with PWS for 12 months.
- Potential improvements in skills, support systems, and overall quality of life for residents.

WHO CAN PARTICIPATE?

✓ Program Administrators or Residential Managers who:
- Are 18 years or older
- Supervise at least two residential staff members
- Work in a program serving at least one adult with PWS
- Speak and read fluent English

✓ Residential Staff who:
- Are 18 years or older
- Work in a program serving at least one adult with PWS
- Speak and read fluent English

INTERESTED?
Contact: Stacy Ward
sward@pwsausa.org

Prader-Willi
SYNDROME ASSOCIATION | USA
SAVING AND TRANSFORMING LIVES

We are inviting residential administration, managers and staff to take part in the PWS Home Base Pilot Program, a new multi-tiered support model designed to strengthen training, improve positive behavior supports, and provide resources to reduce stress and burnout among caregivers working with adults with Prader-Willi syndrome (PWS).

Benefits of participating:

Free access to structured training and resources tailored for residential programs serving individuals with PWS for 12 months. Potential improvements in skills, support systems, and overall quality of life for residents.

Who can participate:

Program Administrators or Residential Managers who:

- Are 18 years or older
- Supervise at least two residential staff members
- Work in a program serving at least one adult with PWS
- Speak and read fluent English

Residential Staff who:

- Are 18 years or older
- Work in a program serving at least one adult with PWS
- Speak and read fluent English

If you're interested, please contact Stacy Ward at sward@pwsausa.org.

Ask Nurse Lynn

Male, 23 years old, unknown subtype

I have PWS. I'm 23, 5'7". I do not have diabetes, but my grandfather and dad do. I was wondering what my blood sugar should be every day. I just got a glucose monitor so I want to make sure I'm doing good. I do workout every single day in the gym with my trainer and also doing additional cardio. I weigh right now in the 150s. If you can let me know I would appreciate it. Thank you.

Visit our blog to read Nurse Lynn's response and find links to connected resources.

READ NURSE LYNN'S RESPONSE

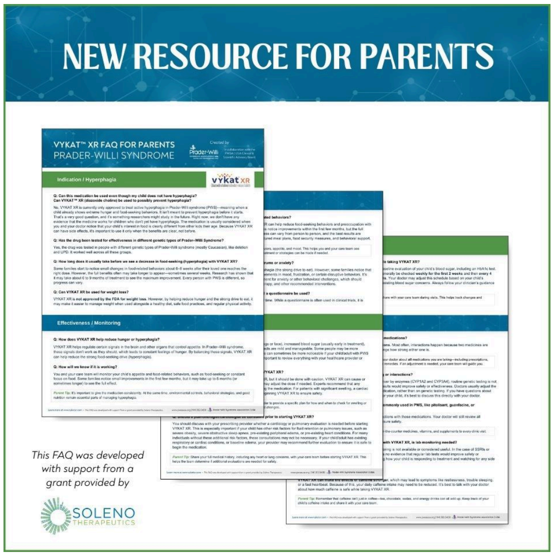
Do you have a non-emergency medical question about Prader-Willi syndrome? Do you have a non-urgent medical question that keeps coming up, but you forget to ask every time you're at the doctor? Perhaps your question applies to numerous families who will benefit from hearing the answer.

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

ASK YOUR QUESTION HERE

RESEARCH

New Resource: VYKAT™ XR (diazoxide choline) FAQ Document



In March 2025, the FDA approved VYKAT™ XR (diazoxide choline), the first-ever treatment for hyperphagia (excessive hunger) in Prader-Willi syndrome. Since then, families and caregivers have had many important questions about this new option.

To help, PWSA | USA, in collaboration with our Clinical & Scientific Advisory Board, has created a comprehensive FAQ document that addresses the most common questions and concerns.

Topics covered include:

- Indication / Hyperphagia
- Effectiveness / Monitoring
- Side Effects / Safety
- Interactions / Pharmacology
- Diet / Lifestyle
- Administration / Dosing
- Storage / Pharmacy

Click below to access and download the full FAQ. This resource was developed with support from a grant provided by Soleno Therapeutics. To learn more about VYKAT XR, visit **www.vykatxr.com**.

DOWNLOAD THE FAQ

Social Skills and PWS Research Study

The purpose of this study is to learn about the perspectives of caregivers on social skills, challenges, and strengths in children with Prader-Willi syndrome

(PWS).

What You'll Do:

Complete a short online survey via QuestionPro (15-20 minutes). Option to participate in a follow-up Zoom or phone interview (30-45 minutes). Participants who complete the survey will be entered into a raffle for a $50 Amazon gift card.

To participate, you must:

- be at least 18 years of age
- Have a child with PWS between the ages of 2 and 15
- Speak and read English
- Have access to the internet and an internet connected device

If interested, contact: Kelly Krukowski, PhD, BCBA-D at KKrukowski1@thechicagoschool.edu. Learn more by clicking the button below.

LEARN MORE

Webinar Recording: Harmony Biosciences At-Home TEMPO PWS Study

We recently hosted a webinar for representatives from Harmony Biosciences to share an exciting update to their TEMPO PWS Study, investigating pitolisant, a potential treatment for excessive daytime sleepiness in individuals aged six and older with Prader-Willi syndrome. This new addition is a game-changer: an **at-home clinical trial opportunity** - no travel required!

Learn more about the TEMPO PWS study by visiting https://www.tempopwsstudy.com/. You can also learn more by watching the webinar using the button below.

WATCH HERE

Aardvark Therapeutics HERO Phase 3 Trial Now Enrolling!

New Site Open

Aardvark Therapeutics recently launched **HERO**, a global Phase 3 clinical trial investigating ARD-101, an innovative, orally administered treatment designed to help reduce hyperphagia (excessive hunger) and food-seeking behaviors in individuals with Prader-Willi syndrome (PWS). This randomized, double-blind, placebo-controlled trial is an important step toward identifying a potential new treatment option for the PWS community, and now, trial sites across the United States are actively enrolling participants.

New Trial Site:

Gainesville, Florida
UF Shands Childrens Hospital
Contact: Jennifer Miller, MD
Phone: (352) 294-8229
Email: millejl@peds.ufl.edu

Click on the button below to learn more about the open trial sites. To learn more about the HERO Study, visit www.heroforpws.com.

LEARN MORE

Recursos en Español

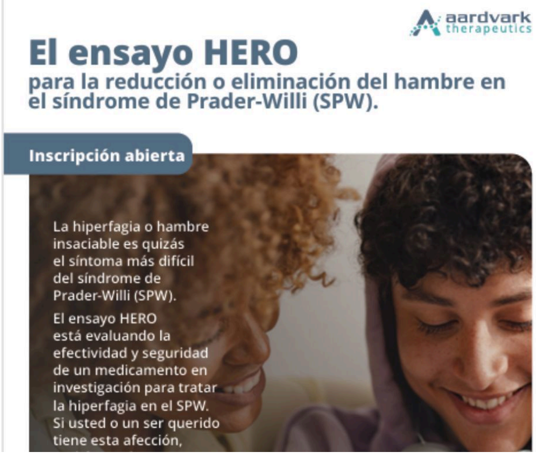
Aardvark Therapeutics has information on their HERO Clinical Trial in Spanish. You can find the material on our clinical trial webpage, or by clicking the links below. Learn more about the HERO Clinical Trial at www.heroforpws.com

Aardvark Therapeutics tienen información sobre el Ensayo Clínico HERO en Español. Puedes encontrar el material sobre nuestra página web del ensayo clínico, o haciendo clic en los enlaces a continuación. Obtenga más información sobre el Ensayo Clínico HERO en www.heroforpws.com.

"Hoja de información del estudio clínico HERO"

"El ensayo HERO"

una folleto "Estudio HERO"



El ensayo HERO

para la reducción o eliminación del hambre en el síndrome de Prader-Willi (SPW).

Inscripción abierta

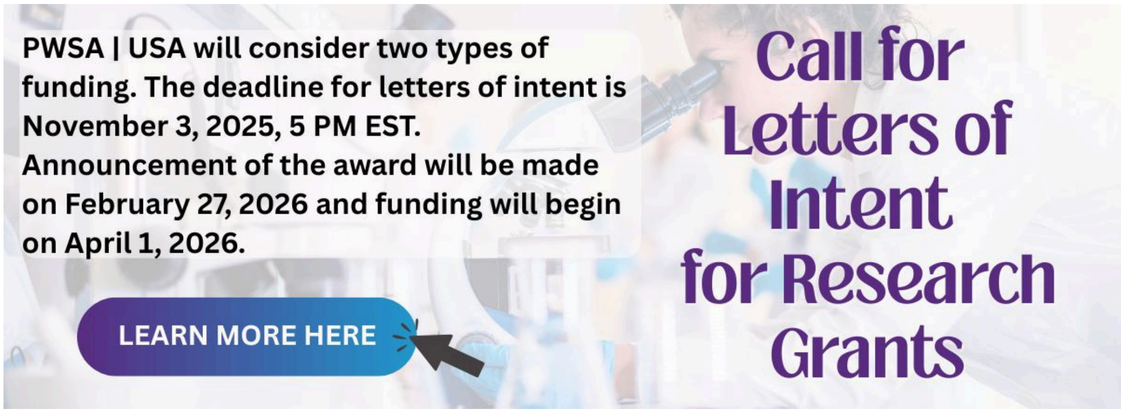
La hiperfagia o hambre insaciable es quizás el síntoma más difícil del síndrome de Prader-Willi (SPW).

El ensayo HERO está evaluando la efectividad y seguridad de un medicamento en investigación para tratar la hiperfagia en el SPW. Si usted o un ser querido tiene esta afección,

Supporting PWS Research

Prader-Willi Syndrome Clinical Scholarships Available

$25,000 USD to support providers in enhancing their understanding of PWS through clinical proctorships with experts in the field. Available to providers currently in practice in the US and who presently care or plan to care for patients with PWS.



PWSA | USA will consider two types of funding. The deadline for letters of intent is November 3, 2025, 5 PM EST. Announcement of the award will be made on February 27, 2026 and funding will begin on April 1, 2026.

Call for Letters of Intent for Research Grants

PWSA | USA ANNOUNCEMENTS

PWSA | USA Resource Spotlight

Sleep disorders are a common occurrence in the PWS community. Disordered sleep issues like sleep apnea, cataplexy, narcolepsy, and excessive daytime sleepiness can have profound and lasting effects on our loved ones with PWS. It is important for caregivers to learn more about these issues, recognize the signs, and bring any concerns to medical professionals in charge of your loved one's care. PWSA | USA offers an incredible webpage dedicated to resources for sleep issues. There you will find webinar session recordings, presentations, and downloadable PDFs. Please use the button below to gain access to these valuable resources.

VISIT WEBPAGE

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 Pulse Newsletter: September 19, 2025

Enhancing the Quality of Life and Empowering those Affected by PWS

PWSA | USA
PULSE

Vol. 131
September 19, 2025

www.pwsausa.org | 941-312-0400

We are just one week away from PWSA | USA's 50th Anniversary Journey of Hope Gala on Friday, September 26, 2025, at The Ritz-Carlton in St. Louis, MO! Don't miss this unforgettable evening as we celebrate five decades of impact, hope, and community. Here are a few important event announcements:

- **Registration Deadline:** Tickets must be purchased by **Friday, September 19th**. There will be no tickets available on-site/day of. Secure yours today at the button below!
- **Can't attend in person?** You can still be part of this milestone by making a tax-deductible donation in honor or memory of a loved one. **Click here to make a donation**.
- **Silent Auction:** Our virtual Silent Auction is NOW LIVE! See information and links below.
- **Hotel Update:** Our discounted room block at **The Ritz-Carlton, St. Louis**, is now full. If you'd like to stay onsite, you may call the hotel directly at (314) 863-6300 or book online at the standard rate. We also have a list of alternate hotel options in the Clayton area. You can find this list at the button below.

We cannot wait to gather with our community and honor 50 years of progress. See you in St. Louis!

PURCHASE GALA TICKETS

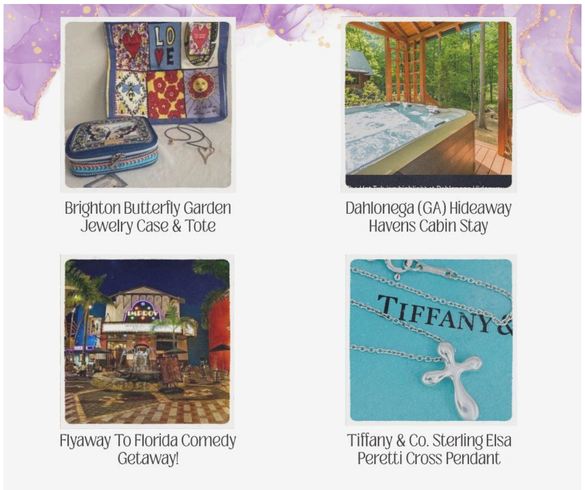
OTHER HOTELS

Virtual Silent Auction Now Live!

Bidding ends Friday, September 26, 8:15 PM CDT. Learn how to bid **HERE.**

Travel destinations, handbags, jewelry, events, and more, you're sure to find something to bid on!

VISIT AUCTION SITE



THANK YOU, JOURNEY OF HOPE GALA SPONSORS

LEGACY SPONSORS

SOLENO THERAPEUTICS

SEMINOLE Hard Rock HOTEL & CASINO HOLLYWOOD, FL

PIONEER SPONSORS

5 BROTHERS PRODUCE

ACADIA

PWSA MEMORIES

At the 4th Annual PWSA Conference, psychologist Dr. John Rustemeyer sat down with several families to discuss how they cope with PWS. These interviews were created as a video format and put onto VHS and Betamax. September-October 1982 issue of The Gathered View offered families the opportunity to rent these videos. The costs of rental ($20 for members | $50 for outside sources) were there to offset the cost of productions, postage, insurance for mailing, and replacing the worn-out copies. VHS and Betamax tapes had a limited number of times they could be viewed before the quality diminished.

PWSA | USA offered rental videos for VHS and Betamax users.

Read the September-October 1982 issue of The Gathered View by clicking the button below.

THE GATHERED VIEW

SHARE YOUR MEMORY

PWSA | USA'S

SPOTLIGHT ON HOPE

In May of 2024, Heather James, mom to Giovani (living with PWS), presented on PWS to her Kiwanis Club. This was the beginning of her public journey as a PWS parent. Because of her presentation and hard work, PWSA | USA was invited to apply for a grant through her club, which we received. Heather was recently invited to present to her club on the impact of the grant's support. The Kiwanis Club of Texarkana recently honored Heather as a "Rising Star". We are grateful to Heather for her dedication and creativity in spreading awareness and support for PWS.

Read the full Volunteer Spotlight using the button below.

SHARE YOUR SPOTLIGHT

EVENTS & FUNDRAISERS

Check Out These Incredible Events Coming Up in October!

We are so honored and blessed to have many wonderful fundraising events happening in October! From dance parties and trivia to cocktails and comedy, the PWS community is showing up in style. We want to spread our gratitude all around. Thank you to all of the families and community members who have taken the time to create such fun and creative events, thank you to those who are donating and participating, and thank you to all the sponsors helping these families create their visions and put the FUN in FUNdraising.

Please click on the images to learn more about the specific events and how to participate. Use the button below to explore events coming up in the next few months.

EVENTS

Saturday, October 11, 2025
@ 4 PM

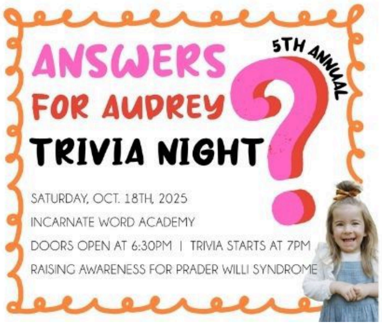
Trumbull Career and Technical Center
Warren, Ohio

ACADIA SOLENO THERAPEUTICS

Saturday, October 18, 2025
@ 6:30 PM

The Malt Room at Brown's Brewery
Troy, New York

SOLENO THERAPEUTICS ACADIA



Saturday, October 18, 2025
@ 6:30 PM

Incarnate Word Academy
St. Louis, Missouri

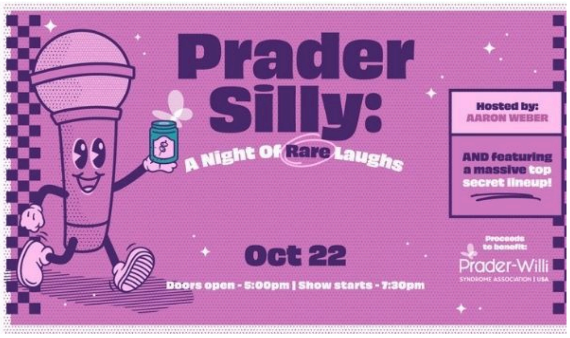
ACADIA SOLENO THERAPEUTICS

Knowledge Bomb Jordan Craft

Wednesday, October 22, 2025
@ 5 PM

Zanie's Comedy Night Club
Nashville, Tennessee

NATELAND Anne and Mike Sinsheimer ZANIES COMEDY NIGHT CLUB



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

A Podcast for the PWS Community

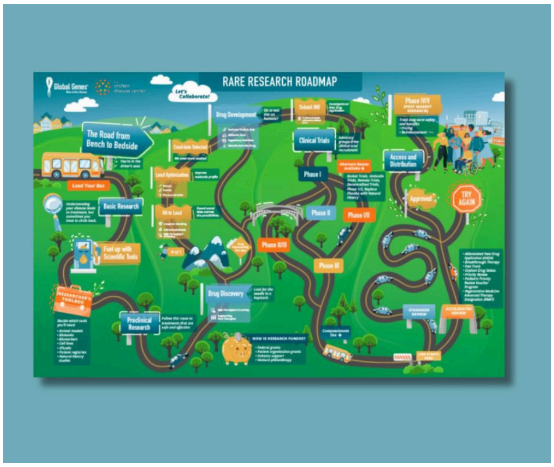
This week our CEO, Stacy Ward, sat down with three direct support professionals (DSP) from the community to discuss what it is like working with individuals with PWS, how these individuals affect their lives, advice they have for DSPs coming into the community, and more. DSP Week comes every September, but we encourage our families to celebrate and appreciate their DSPs throughout the year. Thank you, DSPs!

Listen to PWS United on your favorite podcast app or by clicking the button below.

LISTEN ON PODBEAN

ADVOCACY

Global Genes RARE Drug Development Symposium



On September 3-4, Elaine Towle, Advocacy Specialist with PWSA|USA, had the opportunity to attend the Global Genes RARE Drug Development Symposium in Boston, MA. Attendees included advocates/representatives from many rare disease communities, as well as clinicians, physician scientists, and researchers from the rare disease community. It was a great opportunity to learn more about the drug development world and the important role organizations like PWSA|USA have in that process.

One session focused on navigating the drug development roadmap and introduced attendees to key stages in that process. Critical areas for engagement by advocates and people living with rare disease were highlighted.

One big focus throughout the Global Genes conference was the importance of data to drug development endeavors. There are several important sources of data for research purposes including natural history data, biomarkers (when available), genetic information, biospecimens, and even medical records data. The Global PWS Registry is a source of natural history data and is a powerful tool for the PWS community to advance understanding of PWS, areas of unmet need, standards of care, and new therapies. Data from this registry is shared back to the PWS community and is also used by researchers and scientists. By participating, families of those with PWS become part of the research team, helping uncover trends in causes and diagnoses as well as new directions in therapies and treatment. This is an important way for all of us to contribute to the science and drug development in PWS for our loved ones.

Global Genes has developed a visual of this roadmap which is included here. Click the button below for a detailed description of the process.

GLOBAL GENES RARE RESEARCH ROADMAP

GLOBAL PWS REGISTRY

Advocacy Ambassadors Help Advance PRV Reauthorization on Capitol Hill!

This past week, PWSA | USA’s Advocacy Ambassadors (Dorothea Lantz, Elaine Towle, Jennifer Garzia, Jennifer Andrews, Pia Dorsen, Christina DeFeo, Kristi Rickenbach, and Justice Rickenbach) traveled to Washington, D.C. to champion an issue of critical importance to our community: the reauthorization of the Rare Pediatric Disease Priority Review Voucher (PRV) program.

Why PRVs Matter

For children and families impacted by PWS and other rare diseases, PRVs are more than just a policy tool—they are a lifeline. The PRV program incentivizes pharmaceutical companies to invest in developing treatments for rare pediatric conditions by offering a voucher that can accelerate the FDA review of another drug. This added incentive has already spurred innovation, bringing forward new therapies that might otherwise have been overlooked due to the small patient populations they serve. For the PWS community, where treatment options remain limited, the continuation of PRVs means more opportunities for urgently needed therapies to reach families faster.

A Unanimous Step Forward

We are thrilled to share that the Give Kids a Chance Act, which includes reauthorization of the PRV program, passed unanimously (47-0) in the House Energy & Commerce Committee this week. This decisive vote demonstrates strong bipartisan support for children living with rare diseases and sends a powerful message: our voices are being heard.

Recognition from the Dais

During the markup, Congressman Gus Bilirakis (R-FL-12) took a moment to specifically recognize Dorothea Lantz and Jennifer Garzia, two of our dedicated Advocacy Ambassadors, for their work and presence on Capitol Hill. Their recognition reflects the power of lived experience and the importance of patient and caregiver advocates in shaping federal policy.

The Road Ahead

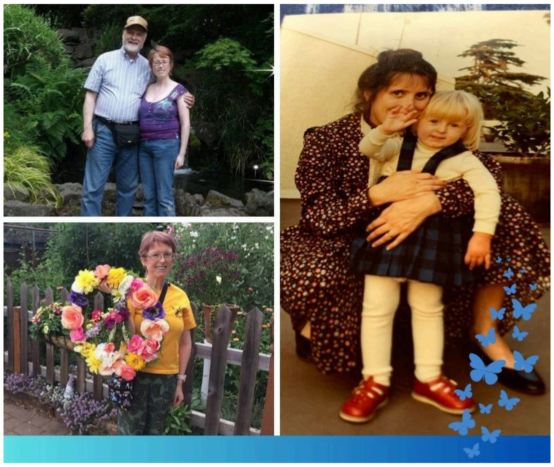
While this milestone is cause for celebration, the work is not done. The bill must still move through the full House and Senate before becoming law. PWSA | USA will continue to work alongside our partners and advocates nationwide to ensure this essential program is reauthorized and that the voices of the PWS community remain central in the conversation.

We extend our deepest gratitude to our Advocacy Ambassadors, the families who shared their stories, and all members of Congress who are standing with rare disease communities. Together, we are moving closer to a future where children with PWS have access to the treatments—and hope—they deserve.

FAMILY SUPPORT

PWS in Adulthood Blog Series

Last year we began a blog series to highlight adults with PWS. We love hearing about the lives of our loved ones with PWS, especially when it comes from them directly. Emma Niedermeyer, a 44-year-old individual living with PWS, submitted her own responses to our PWS in Adulthood blog form. She shared with us a bit of her story, from living arrangement to romantic relationships and advice for parents. Thank you, Emma, for giving us a glimpse into your world.



Click on the button below to read more about Emma in "Living a Happy, Healthy Life."

READ HERE

PWS IN ADULTHOOD FORM

PWS Egypt and Middle East Conference

October 1-4, 2025, PWS Egypt and Middle East will be hosting the Nile Hope Workshop and Family Camp. This conference is the first PWS conference in the area. The first two days of the conference, October 1-2, will be held online.

Prader-Willi Egypt & Middle East
مؤسسة برادر ويلي مصر والشرق الأوسط

NILE HOPE WORKSHOP & FAMILY CAMP
ورشة ومعسكر نيل الأمل

Empowering Families

1-4 OCTOBER 2025

Beit El Wady- Wady El Natroon

ورشة عمل عن بُعد ١-٢ أكتوبر
معسكر عائلي في بيت الوادي ٣-٤ أكتوبر

PRADER-WILLI SYNDROME EGYPT& MIDDLE EAST

Free Registration
التسجيل مجاناً

IPWSO International Prader-Willi Syndrome Organisation

Some of the speakers include Dr. Farah Almadhoun, Dr. Mohamed Hamdy, Dr. Mona Salem, Dr. Shokery Awadalla, along with professionals and staff from the PWSA | USA community.

Families will gather in person October 3-4 in Wady El Natroon-Cairo- Egypt for a PWS Camp with activities for the whole family. These will include some live sessions on mental health, along with games and other fun and engaging activities to support and engage the entire family.

This conference is supported by a grant from Friends of IPWSO (International Prader-Willi Syndrome Organisation).

Beyond High School: Creating Meaningful Futures for Young Adults with PWS

The C-15 Foundation, a registered 501(c)(3) organization dedicated to creating opportunities for individuals living with PWS, is announcing the creation of a Live, Work, Play, and Thrive community in Chattahoochee Hills, GA, approximately 20 minutes from the Atlanta Airport.

This first-of-its-kind, dedicated PWS development will be home to “high-functioning” PWS young adults (current ages of 20-30) who have the desire and ability to reach greater heights of independence and self-development. The 108-acre C-15 campus is proposed to provide a genuine opportunity to live a more self-supportive life, while still receiving guidance and light oversight from on-campus coaches. Additionally, all residents will be required to have a part time job on or off campus.

The campus will offer 24 private apartments consisting of a large private bedroom and bathroom with connected common areas. Ample entertainments are included in these common areas, such as games, televisions, and “hang-out” zones. These apartments will not be constructed with kitchens or furnished with refrigerators.

A free-standing and ultra-secure dining hall will serve a catered menu of healthy and nutritious, campus-grown foods. This dining hall will be the ONLY place on campus where food will be accessible.

The on-campus Activities Center, located within walking distance, will include an indoor pool, walking track, and yoga room. Fun and engaging activities and classes curated to stimulate movement will be available to all residents.

The campus’s working farm will be the main source of ingredients for the Dining Hall. Fruits, vegetables, and healthy starches will be grown and tended by campus residents and prepared by kitchen staff for meals. This farming area will provide daily jobs for some residents with an opportunity to care for a few small animals (i.e. dogs, chickens, etc.).

If you believe your loved one with PWS has the desire and ability to live in a semi-independent environment, we encourage you to reach out to the C-15 Foundation. They expect to begin conducting resident interviews in 2026 with a goal to open in mid-2027.

Use the button below to learn more or email Scott Arant, Founder at Arant0867@gmail.com

LEARN MORE

Ask Nurse Lynn

Female, 22 years old, Deletion subtype

My daughter is not sleeping…day or night. She sleeps maybe 2 hours at night and no naps. That means I don’t sleep. We have tried OTC and Ambien, and they don’t work. We need help, I am emotionally and physically exhausted. I will reach out to her psychiatrist but wanted to know if there are things you know that may possibly work.
Thanks!

Visit our blog to read Nurse Lynn's response and find links to connected resources.

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

RESEARCH

New Research Opportunity for Parents

Research Opportunity:

Calling parents of children who received a genetic diagnosis in the hospital (NICU, PICU, or CCU)

Questions? Contact:
Annika Paulsen at anpaulsen@unmc.edu
Kristen Fishler Malone kristen.fishler@unmc.edu

This study has been approved by the UNMC Office of Regulatory Affairs. (IRB 03 85-25 -EX)

Would you like to share your thoughts about your experience receiving your child’s genetic diagnosis?

Researchers at the University of Nebraska Medical Center are recruiting parents and caregivers with a child who received a genetic diagnosis while in the neo-natal intensive care unit (NICU), pediatric intensive care unit (PICU), or critical care unit (CCU) to share their experiences in a 10- to 15-minute online survey. Responses from this survey may be used to guide medical providers in giving genetic diagnoses and support to families in the inpatient setting.

To participate, you must be 19 years or older and have a child who received a genetic diagnosis while in the NICU, PICU, or CCU. Thank you for considering sharing your experiences!

Click the button below to take the survey.

TAKE THE SURVEY

Aardvark Therapeutics HERO Phase 3 Trial Now Enrolling!

New Site Open

Aardvark Therapeutics recently launched **HERO**, a global Phase 3 clinical trial investigating ARD-101, an innovative, orally administered treatment designed to help reduce hyperphagia (excessive hunger) and food-seeking behaviors in individuals with Prader-Willi syndrome (PWS). This randomized, double-blind, placebo-controlled trial is an important step toward identifying a potential new treatment option for the PWS community, and now, trial sites across the United States are actively enrolling participants.

New Trial Site:

Seattle, Washington
Seattle Children's Hospital

HERO, Phase 3 Clinical Trial for ARD-101, Now Enrolling

New Site Now Open

Seattle, Washington: Seattle Children's Hospital

Contact: Isabella Niu, MD / Stephanie Purdy
Phone: (206) 987-2640
Email: stephanie.purdy@seattlechildrens.org

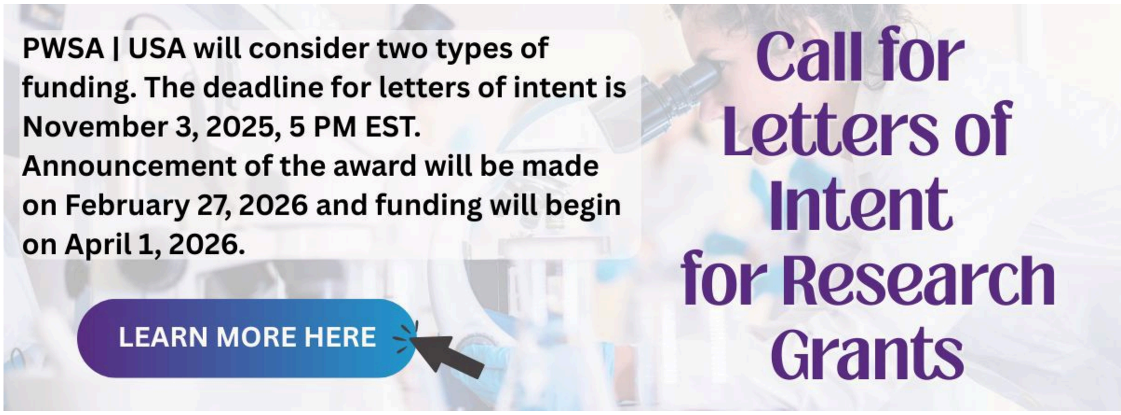
Click on the button below to learn more about the open trial sites. To learn more about the HERO Study, visit www.heroforpws.com.

LEARN MORE

Supporting PWS Research

Prader-Willi Syndrome Clinical Scholarships Available

$25,000 USD to support providers in enhancing their understanding of PWS through clinical proctorships with experts in the field. Available to providers currently in practice in the US and who presently care or plan to care for patients with PWS.



PWSA | USA will consider two types of funding. The deadline for letters of intent is November 3, 2025, 5 PM EST. Announcement of the award will be made on February 27, 2026 and funding will begin on April 1, 2026.

Call for Letters of Intent for Research Grants

PWSA | USA ANNOUNCEMENTS

Colors of Hope Welcomes New Organizer

Colors of Hope is growing — and with growth comes new leadership, new stories, and fresh energy! Colors of Hope is excited to welcome **Sarah Kasaby,** PWSA | USA's Information and Referral Specialist, as a new Colors of Hope organizer!

Sarah is originally from Egypt, North Africa, and currently lives in Nebraska with her husband, who is from Burkina Faso, West Africa, and their 5-year-old son, Khaled, who has PWS. Their family loves road trips and exploring new places together.

Sarah joined PWSA | USA in 2021 as a member of both the Family Support Team and the International Support Team, where she focuses on supporting families in the U.S. and around the world—especially those who do not speak English. She feels blessed to be able to use her language skills to help other families on their journey.

Sarah truly embodies the spirit of Colors of Hope, which is rooted in culture, compassion, and community. We are thrilled to welcome her to our leadership team and excited for the heart, talent, and global perspective she brings to our shared mission.

As we continue to grow this movement, we're always looking for organizers with a serving heart, a strong commitment to inclusion, and a passion for uplifting our diverse PWS community. There's a place for you here.

If you feel called to contribute—or know someone who would be a great fit—please email bipocpws@gmail.com. Together, we're making this journey brighter, in every color, language, and story.

Estimada comunidad,

¡Colores de Esperanza está creciendo — y con el crecimiento vienen nuevos liderazgos, nuevas historias y una energía renovada! Colores de Esperanza se complace en dar la bienvenida a Sarah Kasaby, Especialista en Información y Referencia de PWSA | USA, como una nueva organizadora de Colores de Esperanza!

Sarah es originaria de Egipto, África del Norte, y actualmente vive en Nebraska con su esposo, originario de Burkina Faso, África Occidental, y su hijo de 5 años, Khaled, quien padece el síndrome de Prader-Willi (SPW). A su familia le encantan los viajes por carretera y explorar nuevos lugares juntos.

Sarah se unió a PWSA | EE. UU. en 2021 como miembro del Equipo de Apoyo Familiar y del Equipo de Apoyo Internacional, donde se enfoca en apoyar a familias en EE. UU. y en todo el mundo, especialmente a aquellas que no hablan inglés. Se siente afortunada de poder usar sus habilidades lingüísticas para ayudar a otras familias en su camino.

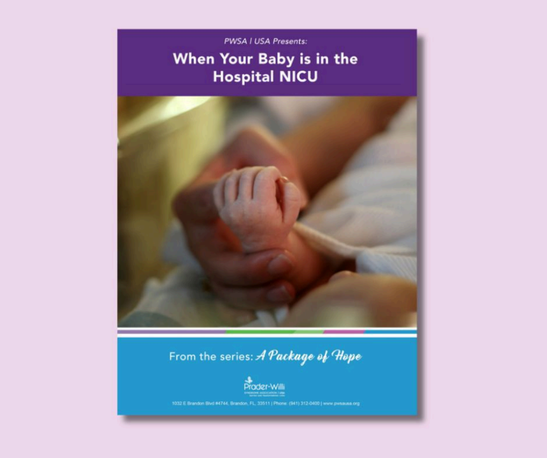
Sarah encarna verdaderamente el espíritu de Colors of Hope, arraigado en la cultura, la compasión y la comunidad. Nos complace darle la bienvenida a nuestro equipo de liderazgo y nos entusiasma su corazón, talento y perspectiva global, que aporta a nuestra misión compartida.

A medida que continuamos expandiendo este movimiento, buscamos constantemente organizadores con un espíritu de servicio, un fuerte compromiso con la inclusión y pasión por impulsar a nuestra diversa comunidad de personas con síndrome de Prader-Willi. Hay un lugar para ti aquí.

Si te sientes llamado a contribuir, o conoces a alguien que podría encajar perfectamente, enviar un correo electrónico a bipocpws@gmail.com. Juntos, estamos haciendo este viaje más brillante, en todos los colores, idiomas e historias.

Con mucho respecto
Los Organizadores de Colores de Esperanza

PWSA | USA Resource Spotlight



PWSA | USA Presents:
When Your Baby is in the
Hospital NICU

From the series: *A Package of Hope*

Prader-Willi

1032 E Brandon Blvd #4744, Brandon, FL, 33511 | Phone: (941) 312-0400 | www.pwsausa.org

Spending time in the NICU is a common experience for many families in the PWS community, whether or not they have yet received the PWS diagnosis. The beginning of the PWS journey is often very challenging for families, accompanied by feelings of fear, worry, grief, and countless questions. This booklet, "When Your Baby is In the Hospital NICU," written by Colette, R.N., and Eric Joncas, R.N., (parents of a child with PWS) and edited by PWS medical specialists, is a guide to help parents know what to expect, what to be aware of, and when to ask for help. By providing answers to some of the questions parents and caregivers have while in the NICU, we hope to relieve some of the fears and concerns. If you are a caregiver in the NICU needing support, please reach out to us at (941) 312-0400.

Click the button below to view the booklet.

NICU BOOKLET

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

PWSA | USA Pulse Special Edition Newsletter: September 26, 2025

Donate

www.pwsausa.org | 941.312.0400

VYKAT XR is Approved by FDA, Now What?
PWSA | USA and the PWS community actively participated in the journey of Soleno's VYKAT XR toward FDA approval. VYKAT XR is designed to treat hyperphagia in individuals with PWS ages 4 and older. Now that VYKAT XR has been approved, our advocacy needs move to the state levels to determine coverage and access. We have been putting out the call for families in each state as the review boards come up. There are several states still waiting for those reviews and we wanted to make sure families understand the request when the call goes out. With continued research and advocacy, the PWS community will hopefully continue to see treatment options approved by the

FDA, and this review process will repeat. This Special Edition Pulse is dedicated to helping families better understand P&T committees and DUR board. When we are educated, we can more effectively advocate for our loved ones with PWS.

What are P&T and DUR Boards and why do they matter for families with Prader-Willi syndrome?

For families living with PWS, access to new therapies isn't just about FDA approval. Once a drug is approved, there's another critical step that determines whether patients can actually receive treatment: coverage decisions made by Pharmacy & Therapeutics (P&T) Committees and Drug Utilization Review (DUR) Boards.

These groups may sound like behind-the-scenes bureaucracies, but the decisions they make directly impact whether a treatment is available through Medicaid or private insurance. For our community, understanding their role is essential as we work to ensure coverage of the growing pipeline of therapies designed to treat the symptoms of PWS.

P&T Committees

Pharmacy & Therapeutics Committees are state- or health-plan-based panels of physicians, pharmacists, and sometimes patient representatives. Their job is to evaluate new drugs and decide whether to place them on the formulary—the official list of medications that insurance will cover. When reviewing a therapy for PWS, P&T Committees consider:

- Clinical trial data showing safety and effectiveness.
- Cost-effectiveness compared to other treatments.
- Input from medical experts and patient advocates.

Their decision determines whether a new therapy is “preferred,” “restricted,” or sometimes excluded altogether.

DUR Boards

Drug Utilization Review Boards, often working alongside or after the P&T Committee, monitor how drugs are being prescribed and used once they're on the market. They set policies such as prior authorization requirements (extra steps a doctor must take before prescribing) or limits on who can receive the treatment.

For families, this can mean the difference between smooth access or frustrating delays. DUR Boards help ensure safe, appropriate use of therapies, but their restrictions can sometimes create unnecessary barriers to care. That's why patient voices in this process are so important.

Elaine Towle, PWSA | USA's Advocacy Specialist and mom to James (living with PWS), recently attended her state's review of VYKAT XR in New Hampshire. Read what she had to say about the experience.

“VKKAT was the last of seven new drugs being considered by the New Hampshire DUR Board. It was presented to the Board and for the first time at the hearing, members had comments. The Chair asked me if the genetic testing requirement would be too onerous for PWS families; I assured him that genetic testing is the standard of care. And another Board member, a psychiatrist, commented from the heart (and a little teary) that she had treated a couple of patients living with PWS in the past, that it was a terribly difficult condition, and how pleased she is for the PWS community that there is now a drug to help patients and families living with PWS. VYKAT passed unanimously.”

Different States, Different Rules

One of the most challenging aspects of this process is that every state runs its P&T Committees and DUR Boards differently:

- Membership: Some states allow patient testimony, while others do not.
- Meeting Schedules: Some meet monthly, others quarterly or even less often.
- Decision Processes: Some states vote immediately on coverage, while others delay decisions for further review.

This means that access to the same FDA-approved therapy may look very different depending on where you live.

Timelines You Should Know

Initial Review: After FDA approval, states typically review a new drug within 3–6 months, though some move faster or slower.

Ongoing Reviews: Even after coverage is approved, DUR Boards revisit policies to monitor safety, costs, and usage patterns.

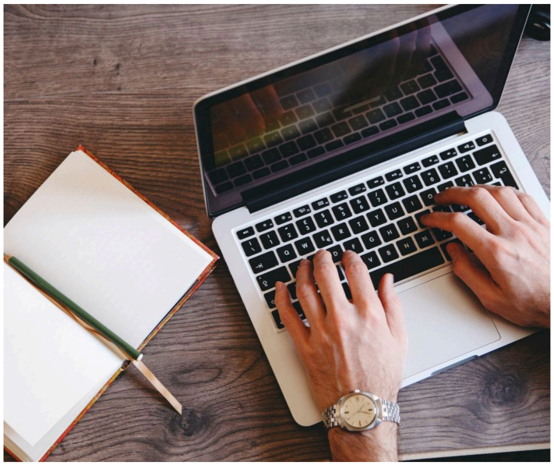
Public Input: Many states allow patients and caregivers to provide comments–either in person, virtually, or through written testimony–before final decisions are made.

Key Takeaways

1. Coverage is not automatic. FDA approval is only step one; states still need to decide whether Medicaid and insurers will pay for the therapy.
2. Your voice matters. Testimonies from families and advocates help committees understand the real-world impact of these treatments beyond the numbers.

3. Expect differences across states. A therapy might be covered in one state but heavily restricted in another.
4. Timelines vary. Stay informed about when your state's committees meet so you can engage at the right time.

How You Can Help



PWSA | USA is actively participating in these processes by submitting written and oral testimony across the country. We encourage families to share their stories with us so we can bring the lived experience of PWS to the decision-makers shaping access. Together, we can help ensure that when safe and effective therapies for PWS are developed, they don't just exist on paper—they actually reach the people who need them most.

Writing Guide:

- My name is:
- I live in:
- My child living with PWS is ___ old and receives Medicaid in the state of (name your state).
- Thank the members of the committee for the work they do.
- How do the unmet needs of PWS affect your family?
- If your son/daughter IS taking VYKAT XR, explain how access to the drug has changed your lives?

· If your son/daughter IS NOT taking vykat XR, how will having access to an approved drug to treat hyperphagia in PWS change their lives and what their future looks like?

· What is your hope for the approval of drugs to treat hyperphagia in PWS?

Review:

If you'd like help drafting your story or want someone to review it with you, PWSA | USA is here to support you. Reach out to a fellow parent or contact us directly advocacy@pwsausa.org or (941) 312-0400.

Remember, the experts will cover the science; your testimony will touch the hearts of the decision-makers. Bring them to tears with the reality of our challenges and the promise of what could be. **This is where the power lies.**

Prader-Willi
SYNDROME ASSOCIATION | Ohio
SAVING AND TRANSFORMING LIVES

DONATE HERE