PWSA | USA Bi-Weekly Pulse: October 6, 2023



Freya, a 6th-grade student living with PWS, recently embarked on a journey with her class to explore volcances in Lassen Volcanic National Park. Despite arduous hikes and challenging activities, Freya's unwavering confidence and resilience shone through as she conquered Mount Lassen's summit, a 10,457-foot volcano. Her adventures continued with visits to geothermal areas, the ascent of a 700-foot volcano named Cinder Cone, and exploration of Shasta Caverns' tunnels. Freya's remarkable self-belief and her triumphant journey serve as an inspiring testament to the power of determination!

Read the full Spotlight on Hope story, contributed by Anne Fricke, at the button below.

READ MORE HERE

We want to hear YOUR Spotlight on Hope!

Any and all milestones, whether it's first steps or hiking to the top of a volcano, deserve to be celebrated. Click the button below and fill out our online form to share your loved one's milestones and what you're grateful for as we head into the giving season.

SHARE YOUR SPOTLIGHT ON HOPE



Calling all New Englanders - Join the Lens family for a full afternoon of fun activities! The 14th Annual Hunter Lens Golf Tournament will take place

Where: Incarnate Word Academy 2788 Normandy Avenue St. Louis, MO 63121

Saturday, October 7, 2023, at 12:00 p.m. EST at the Back Nine Club (17 Heritage Hill Dr., Lakeville, MA 02347). Enjoy time with family and friends while participating in golf, a silent auction, prize drawings, and delicious dinner. Meet Hunter (24, living with PWS), learn more about PWS and how PWSA | USA is working to help families across the U.S, and enjoy a day on a beautiful course with great company!

LEARN MORE HERE

When: Saturday October 21, 2023

Doors Open at 6:30pm & Trivia Begins at 7pm

"We want to continue to help fund research that will provide life changing treatments for PWS, which is why we are hosting our 3rd annual Trivia Night! We were blown away by the love and support of 2022's Trivia Night and cannot wait for October 21st!" - The Spring Family

LEARN MORE HERE

PWSA | USA Engages in Global Advocacy Awareness



Later this month, Dorothea Lantz, our Director of Community Engagement, will help to represent the PWS community at the <u>Indo US Bridging RARE Summit 2023</u> in Arlington, VA.

Dorothea will be part of a panel of advocates who represent rare disease throughout the U.S. and India to discuss cross-border perspectives on patient advocacy.

For more information on IndoUSrare, their mission, and the 2023 Summit, click the button below.

LEARN MORE HERE

Dates Announced for PWSA | USA's 2nd D.C. Fly-In Event



FAMILY SUPPORT

Joint Announcement: FPWR Partners with PWSA | USA to Provide BOSS Social Skills Groups for Teens and Adults with PWS



PWSA | USA and The Foundation for Prader-Willi Research are teaming up to empower teens and adults living with Prader-Willi syndrome with crucial social skills through the BOSS Curriculum. This program tackles loneliness, teaches emotional understanding, and fosters meaningful connections. Learn more and find out how your loved one can get involved at the button below.

LEARN MORE HERE

CANCELED: October 11th Sibling Community Conversation



PWSA | USA's October 11, 2023 Sibling Community Conversation has been **CANCELED**. Our next Sibling Community Conversation will take place **November 8, 2023**.

This discussion provides a safe space for dialogue between siblings of a person with PWS. All ages are welcome to attend! PWSA | USA team members will also be available to offer any insight and support. Register in advance for future Sibling Community Conversations at the button below.

REGISTER IN ADVANCE HERE

DMI Webinar Recording Now Available



If you missed last week's Dynamic Movement Intervention Webinar, the recording is now available for viewing at the button below.

DMI co-founders Jake and Jo-Anne take a deep dive into all things therapy related. In this webinar, they discuss the goals and use of Dynamic Movement Intervention, intensive scheduling, low muscle tone, and much more. Their years of

experience in therapeutic interventions help caregivers understand the ins and outs of physical therapy and how it is plays a crucial role in overall development in children with PWS.

WATCH HERE

RESEARCH

PWSA | USA's ECHO 4 PWS "Endocrinology 101" Webinar Recording Now Available

We held our second **Healthcare Provider Project ECHO** webinar last month, which focused on "Endocrinology 101." The recording is now available for our community to view and features speakers Jennifer Miller, MD, Professor of Pediatric Endocrinology, Gainesville, FL and Moris Angulo, MD, Pediatrics, Genetics, and Pediatric Endocrinology, Catholic Health Physician Partners.

Thank you to Dr. Miller and Dr. Angulo for taking the time to educate healthcare professionals in the PWS space! Watch the recording by clicking the image below.



Harmony Biosciences Reveals Encouraging Data from Phase 2 Pitolisant Study in PWS Patients

<u>Harmony Biosciences</u> has presented new secondary endpoint data concerning the use of pitolisant, an investigational drug, for the treatment of excessive daytime sleepiness (EDS) in Prader-Willi syndrome.



According to a recent press release share by Harmony Biosciences, the Phase 2 signal-detection study showed improvements in behavioral disturbances, especially in the higher-dose pitolisant group, as well as reductions in caregiver ratings of EDS severity and some improvements in hyperphagia. The Phase 3 registrational TEMPO study, expected to begin in Q4 2023, will further assess pitolisant's safety and efficacy in PWS patients.

Learn more about these findings at the button below.

READ THE PRESS RELEASE HERE

Acadia Pharmaceuticals Unveils New COMPASS PWS Study Website



Click here to visit the new COMPASS PWS website





This new website gives in-depth details about the COMPASS PWS study, including a brief video, eligibility for participation, what to expect at study visits, and a research site locator. Visit the website by clicking the button below.

The COMPASS PWS study focuses on evaluating carbetocin nasal spray, also known as ACP-101, a new treatment for individuals with Prader-Willi syndrome who experience excessive appetite (hyperphagia).

ICYMI: Soleno Therapeutics Reports Positive Results from DCCR Study C602 for Prader-Willi Syndrome





Exciting News! Soleno Therapeutics has announced positive results from the randomized withdrawal phase of Study C602 for Prader-Willi syndrome (PWS) with DCCR. This is a significant step towards delivering an effective therapy for PWS individuals.

Key Results:

Hyperphagia-related behaviors improved significantly with DCCR
Secondary endpoints also showed favorable trends for DCCR
No new safety concerns were identified

Find more details about this announcement by clicking the button below.

LEARN MORE HERE

Research Opportunity: Play Skills & PWS

The Chicago School of Professional Psychology is seeking survey study participants to contribute to their research on the play skills of children with PWS. If you are 18 years or older, have a child aged 2 to 12 with PWS, are proficient in English, have internet access, and an internet-connected device, you're invited to participate.

By completing the survey, you will have the chance to win a \$25 Amazon gift card through a raffle. Please share the flyer at the button below with anyone who might be interested in joining this study. If you have any questions, contact Kelly Krukowski, M.A., BCBA, at Krukowski@ego.thechicagoschool.edu.



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PWSA | USA ANNOUNCEMENTS

Are You or is Someone You Know a Current or Retired Federal Employee? You Can Make a Difference!



Between **NOW** and January 15, 2024, you can support PWSA | USA during this year's **Combined Federal Campaign (CFC)**! The CFC is a program through which you can give to the charity of your choice. The campaign's mission is to provide "all federal employees the opportunity to improve the quality of life for all." Please consider supporting PWSA | USA as we continue to provide help and hope to families around the world.

PWSA | USA's CFC ID # is 10088

LEARN MORE HERE

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



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PWSA | USA Bi-Weekly Pulse: October 20, 2023



<u>Click Here</u> to View the 2023 Moms' Retreat Photo Gallery

Moms' Retreat Testimonials

"I loved every part of this trip. I have memories that will last the rest of my life."

"The highlight of my experience was relaxing and connecting with so many amazing moms who are going through the same thing - I just loved it all."

"Jessica's talk was on point and spoke to me right where I am right now. Allowing myself to lean into intentionality is priceless."

"Getting to be in the company of other moms that understand the daily struggles and adventures was my highlight."

PWSA | USA'S SPOTLIGHT ON HOPE



Maria, mother to George, shares a heartfelt **Spotlight on Hope** about her family and son's journey with PWS.

"Over time, the diagnosis that had initially felt like a weight became a badge of honor – a testament to love, resilience, and unwavering dedication. We were all on a mission to show the world that every child, regardless of their challenges, has the capacity to shine brightly and inspire others."

George's story serves as a reminder that with love and determination,

nothing is impossible. Read the full article at the button below.

READ MORE HERE

SHARE YOUR SPOTLIGHT ON HOPE HERE

EVENTS

Calling All Missouri Residents! Join for a Night of Fun at the 3rd Annual Answers for Audrey Trivia Event



Where: Incarnate Word Academy 2788 Normandy Avenue St. Louis, MO 63121

When: Saturday, October 21, 2023

Doors open at 6:30 p.m. and Trivia begins at 7:00 p.m.

"We want to continue to help fund research that will provide life changing treatments for PWS, which is why we are hosting our 3rd annual Trivia Night! We were blown away by

the love and support of 2022's Trivia Night and cannot wait for October 21st!" -The Spring Family

LEARN MORE HERE







The **14th Annual Hunter Lens Golf Tournament** was a hole in one! You could feel the love and sense of community among the crowd, all gathered to play golf in support of PWSA | USA and in honor of Hunter Lens, 25, living with PWS. Thank you to the Lens family for hosting this event and continuing to support our mission. We are incredibly grateful.

If you were not able to attend and want to show your support, click the button below.







We want to share a big THANK YOU to those who hosted, sponsored, attended, and donated to the **2nd Annual No Gimmes for Jimmy** golf tournament in memory of Jim Worthington, a PWS dad who passed away in 2021. It was an incredible turnout and PWSA | USA was grateful to have been there in person to share details about our mission.

If you were not able to attend and want to show your support, click the button below.

DONATE HERE





It's almost that time of year to sign up as a **PWSA | USA Selfless Elf** and give the gift of hope and support to PWS families!

Tell your family and friends that taking action for PWS is at the very top of your holiday wish list. Leading up to the kick-off of our 2023 Angel Drive campaign (November 1st), we will share how YOU can become a Selfless Elf to start encouraging donations in lieu of gifts and in honor of your loved ones. It only takes five minutes to create your own Selfless Elf page! Funds raised during our Selfless Elf campaign will support the 2023 Angel Drive. Be on the lookout for more details soon.

If you are interested in receiving more information ahead of the sign up link becoming available, please email <u>hopeunited@pwsausa.org</u>.



We are Getting Ready for a Busy Week on Capitol Hill!

Next week, Community Engagement Director Dorothea Lantz and several PWS advocates will travel to Washington, D.C. for multiple Rare Disease events, meetings, and hearings.

During the D.C. trip, Dorothea will speak at the <u>Rare Disease Company</u> <u>Coalition's</u> briefing "The Risks of Rare: Obstacles and Opportunities in Rare Disease Drug Development." It will take place Wednesday, October 25, 2023 at 12:00 PM EST at 1539 Longworth House Office Building in Washington, D.C. Dorothea will give a 10-15 minute overview on PWS advocacy, life as a caregiver, and how policy and regulatory uncertainty affect rare disease drug



development. By clicking the button below, you can register to attend the event in-person or virtually.

ABOUT THE BRIEFING

Join the Rare Disease Company Coalition (RDCC) on October 25, 2023 as they unveil their new white paper. This research explores the financial realities of rare disease drug development and the implications of federal policies on an already challenging drug development process.

This briefing will provide a deeper analysis of the incentives and risks

associated with clinical and commercial stage rare disease research and development.

Questions? Reach out to <u>yoko@rarecoalition.com</u>.

LEARN MORE AND REGISTER HERE

PWS Awareness will be Broadcast on a National Stage



Next on the agenda - a groundbreaking opportunity for the PWS community you won't want to miss! On Thursday, October 26, 2023, PWSA | USA advocates will take part in a press conference, drawing national media attention in anticipation of the Senate Committee on Aging's hearing on the **Promising Pathway Act (PPA)**. The press conference will allow us to share information about PWS and the participation of our dedicated advocates. They will then have the chance to share their compelling stories directly with the

Senate Committee on Aging during the subsequent congressional hearing. We will share a link to watch the live broadcast on our website and social media pages next Thursday so you can tune in! Ahead of the PPA hearing, you are also encouraged to share your story with Congress to amplify our community's voice. If you're interested in participating, click <u>HERE</u> to learn more. <u>ABOUT THE PROMISING PATHWAY ACT</u> Introduced by Senator Mike Braun (R-IN), Senator Kirsten Gillibrand (D-NY), Senator Lisa Murkowski (R-AK), Senator Kevin Cramer (R-ND) and Senator Roger Wicker (R-MS), this bill would expedite beneficial outcomes for patients by requiring the FDA to establish a rolling, real-time, priority review pathway for drugs intended to treat, prevent, or diagnose serious or life-threatening diseases or conditions. Learn more by clicking the button below.

LEARN MORE HERE

PWSA | USA Engages in Global Advocacy Awareness at the Indo US Bridging RARE Summit



To conclude the week, Dorothea will help to represent the PWS community at the Indo US Bridging RARE Summit 2023, which will take place October 29th and 30th in Arlington, VA. Dorothea will be part of a panel of advocates who represent rare disease throughout the U.S. and India to discuss cross-border perspectives on patient advocacy. For more information on IndoUSrare, their mission, and the 2023 Summit, click the button below.

LEARN MORE HERE

PWSA | USA Volunteer Advocates Jennifer Garzia and Charles Conway Jr. Share Presentations at Harmony Biosciences Conference

PWSA | USA volunteer advocates Charles Conway Jr. and Jennifer Garzia recently had the opportunity to share their compelling insights from a PWS parent perspective at Harmony Biosciences' annual all-employee meeting in Philadelphia.



"Advocating is a very powerful and necessary initiative to PWS awareness, education, and treatment development, whether with local, state, or federal agencies (including pharmaceutical companies). This event provided me the opportunity to share my story and challenges to an organization who is assisting in turning our PWS community's dreams to a reality. Remember, our loved ones' abilities are 'Delaved not Denied' as long as you have the 'Hope' of a mustard seed!!!!!" -Charles Conway Jr., dad to Angelica (3)

"I am thankful for the opportunity to share Rocco's story with Harmony. Rocco's life journey is unique and full of difficult twists and turns, but with the help of so many talented individuals, each day we are filled with more hope that helps us stay on course. I am more optimistic than ever that as impacted families and medical communities listen to each other and work together, Rocco and his peers will be provided a more peaceful and manageable way to live with Prader-Willi syndrome." - Jennifer Garzia, mom to Rocco (19)

Read more about their experience at the button below.

LEARN MORE HERE

FAMILY SUPPORT

October is Mental Health Awareness Month

October is nationally recognized as **Mental Health Awareness Month**, and offers the perfect opportunity to remind our community that you are not alone. We know access to information, connection, and help is crucial to supporting our families and individuals living with PWS.

PWSA | USA's Family Support team is available to talk. Call 941.312.0400 to speak with someone today or at any point in your journey. We are here 24/7/365.

To find additional resources, we encourage you to download the *PWS Mental Health Guidebook* at the button below.



DOWNLOAD THE BOOKLET HERE

Next Sibling Community Conversation: November 8th



Prader-Willi Sundrome Association | USA

Siblings of individuals living with PWS are invited to join PWSA | USA for our next Sibling Community Conversation on **Wednesday**, **November 8, 2023 at 8:00 p.m. EST.**

This discussion provides a safe space for dialogue between siblings of a person with PWS. All ages are welcome to attend! PWSA | USA team members will also be available to offer any insight and support. Register in advance at the button below.

REGISTER IN ADVANCE HERE

RESEARCH

Pitolisant Shows Positive Secondary Outcomes in Phase 2 Study



Exciting news for the PWS community! New data shows promising results for pitolisant in treating excessive daytime sleepiness (EDS) in individuals affected by PWS. <u>Harmony</u> <u>Biosciences</u> plans to initiate a phase 3 study, marking a significant step forward in this treatment. The study's findings highlighted significant positive effects, particularly in the higher-dose group. In children aged 6 to 12, improvements were noted in various behavioral domains, including irritability and social withdrawal, painting a hopeful picture

for the PWS community. Learn more at the button below.

LEARN MORE HERE

Research Opportunity: Play Skills & PWS

The Chicago School of Professional Psychology is seeking survey study participants to contribute to their research on the play skills of children with PWS. If you are 18 years or older, have a child aged 2 to 12 with PWS, are proficient in English, have internet access, and an internet-connected device, you're invited to participate.

By completing the survey, you will have the chance to win a \$25 Amazon gift card through a raffle. Please share the flyer at the button below with anyone who might be interested in joining this study. If you have any questions, contact Kelly Krukowski, M.A., BCBA, at KKrukowski@ego.thechicagoschool.edu.



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PWSA | USA ANNOUNCEMENTS

Welcome to the Team, Anne Fricke!



We are excited to announce a new addition to our PWSA | USA staff! Anne Fricke will join our team as the new **Marketing and Communications Coordinator**.

Anne has been passionate about educating the broader community on PWS since the diagnosis of her daughter, Freya, in early 2012. She knew then that her daughter's safety depended on the education of their community. It began with a blog and a fundraiser but transitioned to a much larger project in 2018 when she created the podcast *Walking*

with Freya. In 2023, she turned her sites to caregiver support by creating an online space of varied resources for parents and families in the disability community. She is the mother of three daughters, wife to a commercial fisherman, poet in both performance and the written word, freelance writer,

novelist, and an avid gardener who loves living in far northern California nestled amongst the redwoods along the Pacific coastline.

Are You or is Someone You Know a Current or Retired Federal Employee? You Can Make a Difference!



Between **NOW** and January 15, 2024, you can support PWSA | USA during this year's **Combined Federal Campaign (CFC)**! The CFC is a program through which you can give to the charity of your choice. The campaign's mission is to provide "all federal employees the opportunity to improve the quality of life for all." Please consider supporting PWSA | USA as we continue to provide help and hope to families around the world.

PWSA | USA's CFC ID # is 10088

LEARN MORE HERE

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



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PWSA | USA Special Edition Pulse: October 27, 2023



PWSA | USA's 2023 Angel Drive campaign, **Give the Gift of HOPE**, kicks off next **Wednesday**, **November 1st**!

YOU can help be a positive force in our work and propel this momentum forward by supporting our end of year campaign to ensure PWSA | USA can continue **TAKING ACTION** in the new year! Whether it's working with volunteer advocates to raise our voices with legislators in Washington, D.C. about issues that matter most or helping you navigate the challenges associated with PWS, your yearend, tax-deductible gift ensures that PWSA | USA can continue to provide **HOPE** to families 24/7/365!

A SNAPSHOT OF OUR 2023



Supported 3,447 families with comprehensive PWS guidance and assistance



Trained at 94 schools, enhancing PWS education and support



Organized historic FDA meeting, uniting 350 voices in PWS advocacy



60 volunteers held 150+ advocacy meetings with elected officials nationwide



Established May 15th as National PWS Awareness Day through Resolution



Hosted record-breaking Orlando Convention, uniting nearly 1,000 attendees





Make a gift online at <u>www.pwsausa.org/angeldrive2023</u>.



Scan the QR code to complete your donation.



Mail a check! Your mailed donation can be sent to:

1032 E Brandon Blvd #4744 Brandon, FL 33511

Please put "Angel Drive" in the check's memo.



Tell your family and friends that **taking action for PWS** is at the very top of your holiday wish list. Click the button below to sign up as a **Selfless Elf** and start encouraging donations in lieu of gifts and in honor of your loved ones. It only takes five minutes to create your own Selfless Elf page!

Questions? Contact us at hopeunited@pwsausa.org.



CLICK HERE TO BECOME A SELFLESS ELF



P.S. Did you know your Selfless Elf page can integrate seamlessly with Facebook to help you reach more friends and family this holiday season?

Learn More About Becoming a Selfless Elf at One of Our Upcoming Webinars

If you are interested in becoming a Selfless Elf but don't know where to start, here's the perfect opportunity! PWSA | USA staff and past participants will be on-hand to share their experiences and encourage others to host their own peer-to-peer fundraiser this holiday season. We will offer TWO webinar opportunities - Thursday, November 9, 2023 at 12:00PM EST and Tuesday, November 14, 2023 at 7:00PM EST. You only need to join one on the date and time that fits your schedule best. Register for the webinar of your choice at the buttons below. We hope to see you there!

Thursday, November 9, 2023 at 12:00PM EST

REGISTER HERE

Tuesday, November 14, 2023 at 7:00PM EST

REGISTER HERE

Share Your Hope



Join the heartwarming movement by sharing your **HOPE**! Help us understand the impact PWSA | USA has had on you and your family's PWS journey. Your unique story is invaluable, and we invite you to click the button below, guiding you to our simple and user-friendly form. There, you'll find a couple questions, along with an opportunity to share a cherished photo that encapsulates your journey. As we embark on our Angel Drive campaign, these testimonials will serve as powerful beacons of inspiration, fostering hope and solidarity within our cherished community. We sincerely appreciate your participation!

CLICK HERE TO SHARE YOUR HOPE

Download our Gift of Hope Certificate!

NEW THIS YEAR! Upon your Angel Drive contribution, you will be prompted to instantly download our beautifully crafted and customizable **"Gift of HOPE" certificate**. This token of goodwill is the perfect embodiment of the spirit of giving, making it a thoughtful keepsake for you or an extraordinary present for your loved ones during this festive season. Simply print and personalize the accompanying space with your own message, transforming this already special gesture into a memory that resonates with warmth and affection.







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