PURA Connect

PURA Syndrome FOUNDATION

September 2026

Message from our Co-Presidents

Hello PURA Family,

As we move into the second half of the year, we're focused on making connections and raising awareness at some major rare disease conferences, while continuing our current initiatives to move research forward and find treatments for PURA Syndrome. Some of the conferences we will have representatives at are the Global Genes Rare Drug Development Symposium, COMBINEDBrain Summit, and American Epilepsy Society Annual Meeting -- the largest epilepsy focused event in the U.S. We look forward to growing our knowledge and partnerships that will help us to better serve all of you.

Our conference in June brought PURA families from all over the world to Munich, Germany. It was a wonderful weekend spent sharing insights, learning from our speakers and one another, and most importantly, connecting as a PURA perfect community. Thank you to all those who joined us in person, virtually or anyone who takes the time to watch the videos on our conference website. Next year we are headed to San Diego, California, USA.

For the second year in a row, we held an open scientific meeting prior to the conference for any scientist, clinician, or organization focusing on PURA Syndrome and PURA Syndrome related issues. We welcomed participants from ten countries and over ten U.S. states. We couldn't be more grateful to witness the growth and collaboration in PURA research happening around the world. We look forward to continuing these in the future.

At our conference, we kicked off of the **2026 Together for PURA Campaign** with the goal of raising $300,000. Our theme this year, "Empowering Families, Advancing Research" really encapsulates what we are planning on doing with the funds raised -- to continue funding the research and infrastructure that move us toward treatments and empowering families with knowledge and assistance. We are thrilled to announce that our generous donors have already provided a $25,000 matching gift for the month of September. Scroll down later in this newsletter to see how we can come together to achieve this goal!

The new PURA Syndrome Registry was officially launched at our PURA Conference as well. Together, the Registry and Biobanks will give researchers the data and samples they need to deepen our understanding of PURA Syndrome and prepare the community for future clinical trials. We cannot express enough how important this Registry is to the future of PURA research and treatments, so please consider participating. See page 2 on how you can get involved.

Our partnership with COMBINEDBrain has allowed us to participate in several research opportunities including an upcoming EEG Bank and Mortality Study. We've already begun a Disease Concept Model, a two-year project that is focused on gathering direct feedback from our community that will help guide care and determine the outcome data needed to measure progress in future clinical trials.

We couldn't be more excited about what is to come and are so grateful for your continued support. Stay tuned for more updates coming soon!

Sincerely,
Liz Astridge & Eva Tucker
Co-Presidents, PURA Syndrome Foundation

Our mission is to support and drive research, raise public awareness and connect, educate, serve, and empower families impacted by PURA Syndrome

NEW PATIENT REGISTRY

Help Advance PURA Research

Join the PURA Syndrome Patient Registry

For a rare condition like PURA syndrome, every family's experience helps researchers better understand the condition, identify meaningful outcomes, develop biomarkers, and prepare for future clinical trials.

The PURA Syndrome Foundation is proud to launch the PURA Syndrome Patient Registry in partnership with COMBINEDBrain and Matrix. This new registry will bring together information shared by families, clinical data, and research resources to help build a stronger foundation for future research and therapeutic development.

Why Participate?

- Help researchers better understand PURA syndrome across the lifespan
- Support natural history and biomarker research
- Accelerate clinical-trial readiness
- Contribute to future treatment development efforts
- Ensure the experiences of individuals with PURA syndrome are represented in research

Why is this Important?

With only a small number of identified individuals worldwide, every participant matters. The more families who participate, the more powerful this resource becomes for researchers, clinicians, and future industry partners working toward treatments.

Check PURA Syndrome Foundation **blog** for our big announcement with details on how YOU can move PURA Syndrome research forward.

Together, we are stronger — and together, we make a difference.

U.S. BIOBANK ROADSHOWS

Donate a Biospecimen to our U.S. Biobank

We are working on setting up sample collection at our 2027 PURA conference, but in the meantime, families have the opportunity to participate in roadshows organized by other Patient Advocacy Groups. This allows individuals to contribute samples even if a dedicated event has not been scheduled for their community.

COMBINEDBrain Roadshows bring professional biospecimen collection teams directly to patient conferences and community events. These events make it easier for families to contribute samples while connecting with others in their community. Roadshows allow Patient Advocacy Groups to coordinate sample collection for their communities in a convenient, supportive setting. Collections are conducted under an Institutional Review Board–approved research protocol and coordinated with participating Patient Advocacy Groups.

Blood collections are provided at no cost, however participation may be limited based on event capacity. Below are the remaining dates and cities. Please contact biorepository@combinedbrain.org if you'd like to participate in one of the Roadshows.

Boston, MA (October 8-9)
Philadelphia, PA (October 9-10)
Nashville, TN (November 4)
Denver, CO (December 3-4)

COMBINED

PURA Syndrome FOUNDATION

Biobank: A global collection and distribution point for PURA research

DISEASE CONCEPT MODEL

Are you a caregiver of someone with **PURA syndrome?**

Your family's experiences can help

improve future care and clinical trials.

Participate in our Disease Concept Model Study

Every family experiences PURA syndrome differently. By sharing your experience, you can help researchers better understand what life with PURA is really like, and ensure that future care and clinical trials focus on what matters most to families.

Researchers, COMBINEDBrain, and the **PURA Syndrome Foundation** invite caregivers to participate in a research interview.

Who can participate?
Parents or caregivers(18+) of someone diagnosed with PURA syndrome.

What will you do?
One virtual audio-recorded interview about your child's symptoms, treatments, and how they impact your family.

How long does it take?
60-90 minutes

Why does it matter?
Your experience will help researchers understand what matters most to PURA families and improve future care and clinical trials.

We're inviting parents and caregivers of individuals with PURA syndrome to participate in a one-time virtual interview (60–90 minutes) as part of a research study led by Vanderbilt University in partnership with COMBINEDBrain and the PURA Syndrome Foundation.

Interested in participating? Email the study team at kailee.s.ward@vanderbilt.edu. Research like this is made possible through the generosity of our community.

Thank you for supporting the PURA Syndrome Foundation and helping fund studies that move us closer to better care and treatments. Together, we're building a future where every story helps create better care for the next generation.

PURA Syndrome FOUNDATION COMBINED VANDERBILT UNIVERSITY

Together, we move closer to better care and treatments.

FUNDRAISING FOR A PURPOSE

TOGETHER for PURA 2026

Empowering Families, Advancing Research

Our 2026 Together for PURA Campaign launched in June with a goal of raising $300,000 — to support critical research, empower families, and strengthen advocacy worldwide. We are excited to share that our generous donors have already provided a $25,000 matching gift, doubling the impact of early donations.

Here's how you can help and take part:

- **Become a Team Captain.** The biggest campaigns are powered by people who rally their own networks — family, friends, coworkers, neighbors. When you start a team, your community becomes part of ours, and that's how we reach $300,000. Getting started is easy: visit our 2026 Together for PURA Fundraising page, scroll to "Team Members," and click the blue "Fundraise" button. Once your team is set up, invite others to join or share your personal link or QR code.

- **Watch Jack and Sara Manning** (Team Hattie's Heroes) share how they planned their October fundraiser and have already raised $25,000. Get inspired and see how you can make a difference too!

- **Offer or Secure a Matching Gift.** A matching commitment — from a family foundation, a local business, or an individual donor — doubles every gift made during the campaign. Matches are one of the most effective ways to motivate giving, and we're working to line them up throughout the campaign. If you can offer a match or help us secure one, please reach out to Brian at fundraising@pura-syndrome.org.

Reaching $300,000 will take all of us — families and extended families, friends and neighbors, local businesses and corporate partners, restaurants, creators, and community leaders. Rare does not mean alone, and when we come together, our impact compounds. We can do this. And you can be part of it.

Here's where this money will go:

- Based on the results of the four grants we funded earlier this year, each of the grants will likely require additional funding to continue the research.
- Growing and expanding our biospecimen collection based on what is needed by researchers
- Expanding our resources for families across the globe
- Ensuring the success of our Patient Registry

Click to join the
2026 Together for PURA Fundraising Campaign

Together, we are advancing research and empowering families.

2026 CONFERENCE RECAP

What a memorable weekend!

Our 2026 PURA Syndrome Conference in Munich, Germany, welcomed 105 in-person attendees, including 17 PURA Perfect individuals, along with 83 registered virtual participants. Over three inspiring days, families, researchers, and advocates connected, learned, and shared in support of everyone affected by PURA Syndrome.

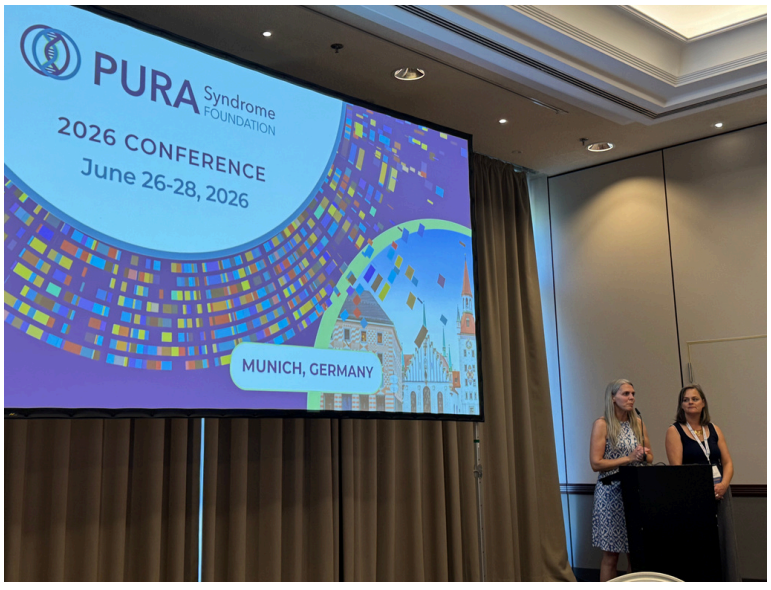
A conference highlight was a visit to Prof. Dierk Niessing's research lab at the Helmholtz Center Munich. Attendees received an update on current PURA research, toured active laboratories and the Biobank—including the PURA Biobank—and had the opportunity to meet with researchers, ask questions, and gain firsthand insight into the important work advancing PURA research.

Conference videos are available by going to **puraconference.org** and clicking on the Schedule tab.

We want your conference feedback. Help us make next year's conference the best one yet! Please click **here** to fill out our quick survey.

Alone we are rare, together we are strong.

2026 CONFERENCE PHOTOS



You are not alone.

2027 CONFERENCE

June 25-27, 2027
San Diego, California, USA

Mark your calendars for the 2027 PURA Syndrome Conference—you won't want to miss it!
We're excited to gather at the beautiful Bahia Resort Hotel, where our PURA community will come together for an unforgettable weekend of learning, connection, and fun.
Take a sneak peek at our incredible host venue below!

BAHIA
RESORT HOTEL

WATCH VIDEO

Join the PURA family.

REMINDERS

PURA Syndrome Clinician Directory

A common question we hear from new families is: "*How can we find doctors who are familiar with PURA Syndrome?"* To help answer that, we've been building a **Clinician Directory** — a growing list of healthcare providers who have experience treating multiple individuals with PURA Syndrome and are experts in their respective specialties.

This directory serves a dual purpose:

✔️ Helping families connect with knowledgeable providers

✔️ Enabling clinicians to connect with one another to improve care and advance research

📍 You can find the **Clinician Directory** in the *For Families* section of our website. If you know of a clinician who should be added to the directory, please let us know by contacting us at president@pura-syndrome.org.

Get Involved in PURA Research

The PURA Syndrome Foundation encourages all PURA caregivers and individuals to consider participating in research studies as they become available. In addition to our Disease Concept Model, we currently have two additional research opportunities. While the Foundation is not always affiliated with or sponsoring these studies, we share them with the hope that participation will contribute to better understanding, care, and outcomes for our children and community.

🔍 To explore current opportunities, click here. Your participation can help drive progress and make a lasting impact!

PURA Syndrome Foundation Needs You!

The PURA Syndrome Foundation is powered almost entirely by volunteers. Every program we offer, every family we support, and every step we take toward advancing research is made possible by people who generously share their time, talents, and passion.

As our PURA community continues to grow, so does the need for helping hands. Whether it's assisting with fundraising, planning events, supporting family outreach, advocating for our community, or lending professional skills in marketing, graphic design, communications, or fundraising, there are many meaningful ways to make a difference.

If you'd like to get involved—or know someone who would—we'd love to hear from you. Together, we can continue building a stronger future for every individual and family affected by PURA Syndrome.

Please fill out the **volunteer form** on our website or email us directly at volunteer@pura-syndrome.org.

We need you.