PROPIONIC ACIDEMIA FOUNDATION

SPRING 2026

SEARCHING FOR A CURE HOPE FOR OUR CHILDREN

MY EXPERIENCE AT GMDI - ANGELA WAITS, PAF BOARD MEMBER AND SECRETARY

I recently had the pleasure of attending the Genetic Metabolic Dietitians International (GMDI) 2026 Conference as an advocacy sponsor/exhibitor on behalf of PAF. The conference brought together industry, sponsors, researchers, and genetic metabolic dietitians from around the world.

This was my first time attending. Not only was it a lot of fun, but I learned so much. I saw a few familiar faces and had the opportunity to meet many new people. I attended several sessions and visited exhibitor booths. I thanked several formula companies for the work they do for our children and families. It was wonderful to see how many people dedicate their careers to improving the lives of individuals living with metabolic disorders.

One presentation that especially stood out to me focused on supporting adult IEM patients through major life stage transitions. As the parent of a 26-year-old daughter with Propionic Acidemia, this topic deeply resonated with me. One of the speakers talked about the importance of genetic dietitians in all aspects of care for our kids. My thoughts immediately went back to Nalani's very first dietitian, Nancy Brody.

I still remember Nancy teaching me how to measure formula on a gram scale and explaining how important it was to follow the diet exactly. Nalani was so fragile, and I was terrified to bring her home. Nancy reassured me that I could do it and she would be there to help when I needed her. Over the years, she walked us down to the lab after appointments, checked on us during hospital stays, and brought such a calming presence whenever Nalani was sick. She encouraged me to stay in school during a time when I was considering dropping out because balancing college and caring for a medically fragile child felt impossible. I honestly do not know where we would be now without her support and guidance.

Nalani has had other dieticians since, and each one has been amazing. When Nalani is sick and I hear the words "we have talked to the genetics dietician" from the hospital staff, there is an immediate sense of relief. It was truly an honor to share a space with so many professionals who have helped guide, comfort, and support our families over the years. I left the conference feeling grateful, encouraged, and hopeful for the future of our metabolic community.

PA REGISTRY

Help move research forward for propionic acidemia.

Participate in the Propionic Acidemia International Patient Registry.

For more information on joining the registry, or to update your information, go to www.paregistry.com

INSIDE

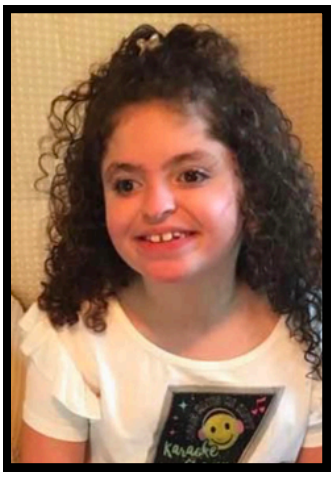
PAF AWARDS $50,000 NEW RESEARCH GRANT

PROJECT: MECHANISMS OF DISEASE IN PROPIONIC ACIDEMIA

PRINCIPAL INVESTIGATOR: MICHAEL J. WOLFGANG, PHD
THE JOHNS HOPKINS UNIVERSITY

SUMMARY: Why does a block in propionate metabolism result in disease? Is an important product of propionate missing or is a product of its incomplete metabolism toxic? What organ systems should be targeted to improve acute or long-term outcomes in Propionic Acidemia (PA)? What biomarkers are important to measure and affect, and which are merely benign passengers and can be ignored? These basic questions are surprisingly difficult to answer given the complexity of cellular metabolism particularly in the context of human physiology and extensive crosstalk between tissues. To begin to answer these fundamental questions, we generated a new mouse model of Propionic Acidemia where we can remove the activity of the effected gene in a tissue-specific and time-dependent manner. Utilizing this unique animal model we have recapitulated important features in PA and begun to provide answers to some of these fundamental questions. We serendipitously discovered that lethality from PA could be reversed by simultaneously inhibiting a second enzyme in carbohydrate metabolism. Here, we will use our extensive genetic models to better understand the mechanism of disease, distinguish the critical biomarkers for preclinical and clinical studies and identify avenues to improve clinical care. Check out his recent publication.

ALESSIA CARMELA MARIA ESTEPHAN 4/21/09-5/27/26



Our precious angel. Her smile would captivate a room before she ever made a sound. The warmth in her eyes-a spark of wonder and joy-that drew people to her made everyone feel special. She had a unique charm expressed through subtle gestures that became her language: subtly signing “all done”, the gentle reach of her hand toward those that captured her fascination, an almost unnoticeable wave “good-bye” or pulling you close to be near you. She loved all kinds of sounds, some bringing tears of joy. Through countless medical crises she showed a fighter's spirit that amazed us all. Her hands were always busy, whether stimming with excitement, swinging her toys or suggesting the car ride continue. She loved the sun, spending hours at the park or on the deck. She loved babies, watching house tasks, getting dressed for Halloween, being pushed in her chair or stroller and many other simpler joys of life that made being with her an escape.

ROAR KICK-OFF MEETING

On May 26-27th, the Rare Organic Acidemias Research (ROAR) Consortium held their kick-off meeting in Houston, Texas. ROAR is funded by the National Institutes of Health as part of the Rare Disease Clinical Research Network.

Sites include
- Baylor College of Medicine
- Children’s National Hospital
- University of Colorado
- University of Minnesota
- University of Pittsburgh

Disorders include
- Cobalamin C (and other disorders of MMA and HCU)
- Glutaric Aciduria Type 1
- Isovaleric Acidemia
- Isolated Methylmalonic Acidemia
- Propionic Acidemia

For more information, check out https://roar.rarediseasesnetwork.org/

ANGEL'S STORY

LA VIDA CON ACIDEMIA PROPIÓNICA: LA HISTORIA DE ÁNGEL ANTONIO

Ser madre siempre implica aprender sobre la marcha, pero cuando la vida nos coloca frente a una enfermedad rara, el camino se vuelve aún más desafiante. Mi hijo, Ángel Antonio, fue diagnosticado con Acidemia Propiónica a los dos meses de nacido, después de un inicio lleno de incertidumbre.

A los 28 días de vida, Ángel dejó de comer y comenzó a presentar clonos. Lo llevamos de urgencia al hospital, donde inicialmente se le diagnosticó una sepsis neonatal. Sin embargo, su estado no mejoraba y fue necesario trasladarlo al Hospital de Alta Especialidad de Mérida, Yucatán. Allí pasó días en terapia intensiva, y aunque finalmente nos dieron el alta, aún no teníamos un diagnóstico claro. Gracias al compromiso y apoyo de la doctora Leticia Belmot, y tras diversas pruebas, logramos finalmente ponerle nombre a lo que enfrentábamos.

Ese fue solo el inicio de un camino lleno de hospitalizaciones y consultas con múltiples especialistas. En nuestra ciudad, Ángel es el primer bebé con esta condición, lo que significó que muchos pediatras y médicos no sabían cómo tratarlo. Además, nació con una hidronefrosis congénita en el riñón izquierdo, que requirió cirugía. Su primer año de vida transcurrió entre hospitales de Campeche y Yucatán, con la esperanza de darle la mejor atención posible.

La vida con Acidemia Propiónica es compleja. No sólo por los retos médicos, sino también por las implicaciones económicas, legales y emocionales. Nadie nos prepara para enfrentar una enfermedad rara. Aprendemos a fuerza de experiencia, entre gastos constantes, largas estancias hospitalarias y la necesidad de ser proveedores y cuidadores al mismo tiempo.

LIFE WITH PROPIONIC ACIDEMIA: ANGEL ANTONIO'S STORY

Being a mother always involves learning as you go, but when life places us face-to-face with a rare disease, the journey becomes even more challenging. My son, Ángel Antonio, was diagnosed with Propionic Acidemia at two months of age, following a start filled with uncertainty.

At 28 days old, Ángel stopped eating and began exhibiting clonus. We rushed him to the hospital, where he was initially diagnosed with neonatal sepsis. However, his condition did not improve, and it became necessary to transfer him to the High-Specialty Hospital in Mérida, Yucatán. He spent days there in intensive care, and although he was eventually discharged, we still lacked a clear diagnosis. Thanks to the dedication and support of Dr. Leticia Belmot—and following various tests—we were finally able to put a name to what we were facing.

That was just the beginning of a journey filled with hospitalizations and consultations with multiple specialists. In our city, Ángel is the first baby with this condition, which meant that many pediatricians and doctors did not know how to treat him. Furthermore, he was born with congenital hydronephrosis in his left kidney, which required surgery. His first year of life was spent moving between hospitals in Campeche and Yucatán, in the hope of providing him with the best possible care.

Life with Propionic Acidemia is complex - not only because of the medical challenges, but also due to the economic, legal, and emotional implications. No one prepares us to face a rare disease. We learn through sheer experience, amidst constant expenses, long hospital stays, and the need to be both providers and caregivers at the same time.

(cont. PAGE) 6

IAN'S STORY

De una crisis a la esperanza: la historia de resiliencia de un bebé con aciduria propiónica

Ian Jesús Lara Tarazona nació el 6 de noviembre de 2025 en la ciudad de Los Ángeles, Chile. Sus primeros dos días de vida parecían normales. Sin embargo, tras un episodio de hipoglicemia en la maternidad se le administró una fórmula láctea estándar. Poco después comenzaron a aparecer signos que nos preocuparon profundamente: somnolencia marcada, dificultad para despertarlo, respiración inusual y una hipotermia que llegó a 35 °C. Como madre, noté de inmediato que algo no estaba bien.

Ian fue trasladado a la unidad de cuidados intermedios, donde los exámenes mostraron una acidosis metabólica severa. Durante la hospitalización presentó convulsiones y comenzó tratamiento con fenobarbital, L-carnitina intravenosa y bicarbonato mientras los médicos intentaban entender la causa de su estado. Los estudios revelaron una hiperamonemia extrema, con niveles de amonio que alcanzaron 1250 µmol/L, una situación crítica para un recién nacido.

Debido a la gravedad del cuadro fue trasladado a la Unidad de Cuidados Intensivos Neonatales de la Pontificia Universidad Católica de Chile. Allí se evaluó realizar hemodiálisis, pero el equipo médico decidió continuar con tratamiento médico al observar que los niveles de amonio comenzaban a descender. Aun así, Ian entró en coma durante aproximadamente diez días y presentó edema cerebral. Fueron días de enorme incertidumbre para nuestra familia. Afortunadamente, los estudios neurológicos no mostraron infartos cerebrales ni daño estructural evidente.

Con el tiempo Ian comenzó a despertar. Inició entonces un proceso de rehabilitación con kinesiología, fonoaudiología y terapia ocupacional, ya que necesitó alimentación por sonda nasogástrica durante parte de su hospitalización. Recibió tratamiento metabólico con L-carnitina y suplementación de aminoácidos, permaneciendo hospitalizado durante un mes y seis días.

From Crisis to Hope: The Resilience Story of a Baby with Propionic Acidemia

Ian Jesús Lara Tarazona was born on November 6, 2025, in the city of Ángeles, Chile. His first two days of life appeared normal. However, following an episode of hypoglycemia in the maternity ward, he was given a standard milk-based formula. Shortly thereafter, signs began to emerge that deeply alarmed us: marked drowsiness, difficulty waking him, unusual breathing, and hypothermia that reached 35°C. As a mother, I immediately sensed that something was wrong.

Ian was transferred to the intermediate care unit, where tests revealed severe metabolic acidosis. During his hospitalization, he experienced seizures and began treatment with phenobarbital, intravenous L-carnitine, and bicarbonate while doctors attempted to determine the cause of his condition. Diagnostic tests revealed extreme hyperammonemia, with ammonia levels reaching 1250 µmol/L—a critical situation for a newborn.

Due to the severity of his condition, he was transferred to the Neonatal Intensive Care Unit at the Pontifical Catholic University of Chile. There, hemodialysis was considered, but the medical team decided to continue with medical management after observing that his ammonia levels were beginning to decline. Even so, Ian fell into a coma for approximately ten days and developed cerebral edema. These were days of immense uncertainty for our family. Fortunately, neurological examinations showed no signs of cerebral infarction or evident structural damage.

Over time, Ian began to wake up. He then started a rehabilitation process involving kinesiology, speech therapy, and occupational therapy, as he required nasogastric tube feeding for part of his hospitalization. He received metabolic treatment with L-carnitine and amino acid supplementation, remaining hospitalized for one month and six days.

(CONT. PAGE 5)

IAN'S STORY CONTINUED FROM PAGE 4

Tras el alta, su seguimiento continuó en el Instituto de Nutrición y Tecnología de los Alimentos (INTA) de la Universidad de Chile, donde se confirmó el diagnóstico de aciduria propiónica y se ajustó su tratamiento con L-carnitina, biotina y una fórmula metabólica especializada. Desde entonces Ian se mantiene bajo un régimen nutricional estricto y con seguimiento médico multidisciplinario.

Hoy, a sus cuatro meses de vida, Ian se encuentra estable, sin nuevas descompensaciones metabólicas. Ha aumentado de peso y talla, se mantiene alerta, despierto y con una evolución neurológica alentadora gracias a las terapias de rehabilitación. Continúa en seguimiento con distintos especialistas y realiza terapias en Teletón.

Como madre, esta experiencia ha sido un camino lleno de incertidumbre, miedo y aprendizaje. También ha estado marcado por la profunda gratitud hacia los equipos médicos que acompañaron cada paso: desde el hospital donde nació hasta los especialistas en enfermedades metabólicas que hoy siguen su evolución. Ian es hoy un bebé alegre, rodeado de cuidados y esperanza, y su historia refleja no solo los desafíos de las enfermedades metabólicas raras, sino también la importancia del diagnóstico oportuno, el tratamiento adecuado y el acompañamiento humano en la medicina.

Sarat Tarazona, Chile

Following his discharge, his follow-up care continued at the Institute of Nutrition and Food Technology (INTA) at the University of Chile, where his diagnosis of propionic acidemia was confirmed and his treatment was adjusted to include L-carnitine, biotin, and a specialized metabolic formula. Since then, Ian has remained on a strict nutritional regimen under multidisciplinary medical supervision.

Today, at four months of age, Ian is stable, with no new metabolic decompensations. He has gained weight and height, remains alert and awake, and shows encouraging neurological progress thanks to his rehabilitation therapies. He continues to be monitored by various specialists and attends therapy sessions at Teletón.

As a mother, this experience has been a journey filled with uncertainty, fear, and learning. It has also been marked by profound gratitude toward the medical teams who supported us every step of the way—from the hospital where he was born to the specialists in metabolic diseases who continue to monitor his progress today. Ian is now a joyful baby, surrounded by care and hope; his story reflects not only the challenges posed by rare metabolic diseases but also the critical importance of timely diagnosis, appropriate treatment, and compassionate human support in medicine.

Sarat Tarazona, Chile

ANGEL'S STORY CONTINUED FROM PAGE 3

Hoy, Ángel está por cumplir su primer año. Su desarrollo es más lento que el de otros niños, perosu fortaleza nos inspira cada día. Él se aferra a la vida, y yo me aferro con él, encontrando ayuda en grupos de apoyo que han recorrido este mismo camino, así como en pediatras y especialistas que siempre están pendientes de su evolución.

La historia de Ángel es la historia de muchos niños con enfermedades raras: un recordatorio de que detrás de cada diagnóstico hay familias que luchan, aprenden y se reinventan para darles a sus hijos la oportunidad de crecer y sonreír.

Karen Berenice Ayala, México

Today, Ángel is about to turn one year old. His development is slower than that of other children, but his strength inspires us every day. He clings to life, and I cling right alongside him, finding support in groups that have walked this very path, as well as in pediatricians and specialists who are always closely monitoring his progress.

Ángel's story is the story of many children with rare diseases: a reminder that behind every diagnosis, there are families who struggle, learn, and reinvent themselves to give their children the opportunity to grow and smile.

Karen Berenice Ayala, México

REQUESTS FOR PROPOSAL

REQUEST FOR

RESEARCH PROPOSALS

PROPIONIC ACIDEMIA FOUNDATION

PAF: ADVANCING RESEARCH AND IMPROVING LIVES

PAF is a non-profit organization that is committed to advancing research and finding better treatments, and ultimately a cure, for propionic acidemia. Our primary aim is to fund projects which will accelerate new knowledge about PA, promote the discovery of biomarkers and co-morbid conditions, and develop and evaluate therapeutics that can help improve the lives of those affected by PA.

PAF will entertain any proposal with the potential to advance treatments and improve the lives of those with PA

APPLICANT QUALIFICATIONS:

To be considered, candidates must possess a PhD, MD, or equivalent degree, and currently hold a full-time position at an established academic or research institution, regardless of their current rank (post-doctoral, research scientist, professor, etc.).

DEADLINE: OCTOBER 1, 2026

GRANT SPECIFICATIONS

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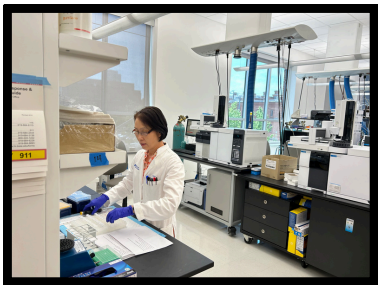
HOW PROPIONIC ACIDEMIA REPROGRAMS LIVER METABOLISM: INSIGHTS FROM A HUMAN CELL MODEL

Guofang Zhang
Duke Molecular Physiology Institute,
Duke University, Durham, NC, USA, 27701



Fang Lu at the Duke Lab

Propionic acidemia (PA) is a rare inherited metabolic disorder caused by deficiency of propionyl-CoA carboxylase (PCC), an enzyme required for the breakdown of propionyl-CoA derived from propiogenic amino acids and gut microbiota–derived propionate. Disruption of this pathway leads to the accumulation of toxic metabolites and impaired cellular energy homeostasis. The liver plays a central role in propionyl-CoA metabolism and is a key target for transplantation in severe PA; however, the mechanisms by which PA disrupts hepatic metabolism remain incompletely understood.

To address this gap, we developed a human hepatocyte model of PA by deleting the PCCA gene in HepG2 cells (PCCA-null HepG2), in collaboration with Dr. Xiaoxin Luke Chen at the Coriell Institute for Medical Research. These PCC-deficient cells lack enzyme activity and recapitulate key biochemical features of PA, including accumulation of propionyl-CoA, propionylcarnitine, and methylcitrate, supporting the fidelity of the model.

Using stable isotope tracing, we systematically characterized metabolic reprogramming caused by PCC deficiency. In control cells, propionyl-CoA contributes to the tricarboxylic acid (TCA) cycle through anaplerosis, supporting energy production and biosynthesis. In contrast, this anaplerotic input is nearly abolished in PCC-deficient cells. This block drives a profound shift in energy metabolism: PCC-deficient cells rely more heavily on glucose oxidation, while pyruvate carboxylase activity is markedly reduced, limiting a major alternative anaplerotic pathway. As a result, these cells lose metabolic flexibility, becoming increasingly dependent on glucose while their biosynthetic capacity declines.

Functionally, these changes impair gluconeogenesis and lipid synthesis—consistent with observations in mouse models of PA—and provide a mechanistic explanation for the susceptibility of PA patients to metabolic crises during fasting. In parallel, mitochondrial fatty acid oxidation is significantly reduced, further limiting lipid-derived energy production. Branched-chain amino acid metabolism is also suppressed, indicating broader metabolic disruption beyond propionate handling. Notably, isotope tracing shows that propionate—primarily derived from the diet and gut microbiota—is the dominant source of propionyl-CoA, whereas amino acids contribute minimally under these conditions in HepG2 cells.

Collectively, these findings demonstrate that PA is not simply a single-enzyme deficiency, but a systems-level metabolic disorder involving coordinated reprogramming of glucose, lipid, and amino acid metabolism. This integrated dysfunction results in a metabolically inflexible liver that is unable to adapt to physiological stress. Our study provides a systems-level framework for understanding hepatic metabolic dysfunction in PA (see reference below).

Fang Lu, Chorlada Paiboonrungruang, Wentao He, Zhaohui Xiong, Pingchuan Tang, Takhar Kasumov, Xiaoxin Chen, Guofang Zhang. Loss of Propionyl-CoA Carboxylase Reprograms Hepatic Metabolism by Suppressing Mitochondrial Pyruvate Carboxylation and Fatty Acid Oxidation. bioRxiv. 2026 April 15. doi: https://doi.org/10.64898/2026.04.13.718201.

NEW ZEBRAFISH KNOCKOUT MODELS FOR PCCA AND PCCB: TOOLS FOR PROPIONIC ACIDEMIA RESEARCH

Melissa N. Hinman, Marnie A. Preston, and Trisha J. Brock

Two new zebrafish knockout lines for **PCCA** and **PCCB** are now available through the Zebrafish International Resource Center (ZIRC) for academic research. These models are intended to support studies of propionic acidemia and to enable further investigation into disease mechanisms and potential therapeutic approaches.

The **PCCA** knockout line contains a ~92 kbp deletion of the putative PCCA locus (5' end of gene remains undefined; deletion is from ~100bp upstream of exon 1 of pcca-201 to 443bp downstream 3'UTR). While animals carrying two copies of this alteration have not been extensively characterized to date, initial observations indicate that they do not persist beyond early developmental stages, highlighting the importance of PCCA function during development.

The **PCCB** line carries a 55 base pair deletion that removes the start codon (ATG) and part of the 5′ untranslated region. This alteration disrupts normal gene expression and provides a model to study the consequences of PCCB deficiency.

Phenotypic observations include:

- **Survival and Development:** Animals with complete loss of PCCB function show markedly reduced survival and do not progress to later life stages under standard conditions.
- **Growth**: Individuals that develop beyond early larval stages are substantially smaller than their wild-type and heterozygous siblings, reflecting the impact of impaired metabolic function on growth.
- **Biochemical Features**: Mutant fish exhibit elevated levels of propionyl-coenzyme A in tissues, consistent with the accumulation of this metabolite observed in individuals with propionic acidemia.

These models recapitulate key aspects of the underlying metabolic disruption and may serve as valuable tools for understanding disease biology and evaluating therapeutic strategies.

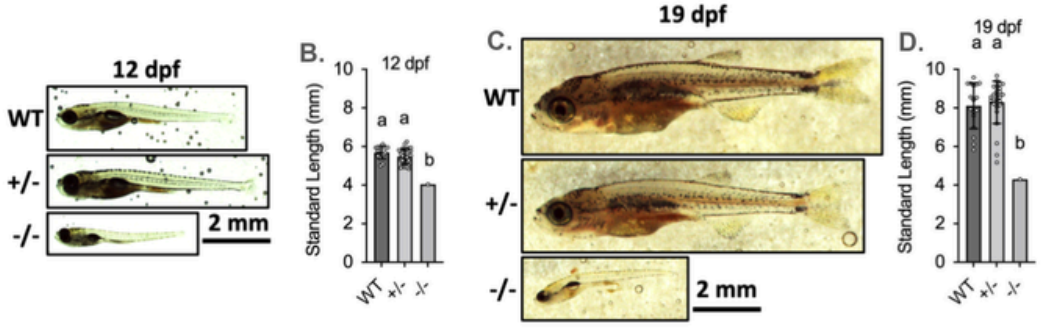


Figure 1. Zebrafish pccb mutants are dramatically smaller than WT at mid-larval stages. Representative images of A. 12 dpf larval fish and C. 19 dpf larval fish. Standard length of B. 12 dpf larval fish and D. 19 dpf larval fish. Each dot represents one fish, and data are presented as mean +/- s.d. Data were analyzed by ordinary one-way ANOVA with Tukey's multiple comparisons test. Data bars with different letters above them are significantly different from one another.

NYC MARATHON TEAM - NOVEMBER 1, 2026

OFFICIAL CHARITY PARTNER
tcs NEW YORK CITY MARATHON

The 2026 TCS NEW YORK CITY MARATHON TEAM
#TCSNYCMARATHON

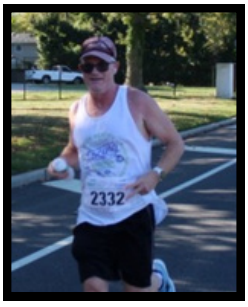
Our 5th year of PARUNNERS!
Meet our amazing team for the NYC Marathon 2026. Show your love; support our runners!

ERIKA ZANNOU, New Jersey. "I am a drug developer and runner. I have been working on rare diseases and gene therapy for the past few years. I have joined the PARUNNERS team this year to raise funds to help researching a treatment for this devastating disease. I have been a runner all my life and have completed multiple races, including the New York City Half marathon and a marathon."

We are thrilled that Erika is joining our team this year in her first New York City Marathon!

ERIN LOWREY, Virginia. "My son, Nash was born in October 2023 and diagnosed with a severe case of PA. He received a liver transplant at 5 months, and despite a lot of challenges and complications of both transplant and PA, he is now thriving. He is talking, walking and is the happiest little dude you'll ever meet. We are very grateful for the support of the entire PA community (medical experts and families alike) in our journey so far and will be honored to run representing the PA team".

We are also very honored to have you in the team, Erin!



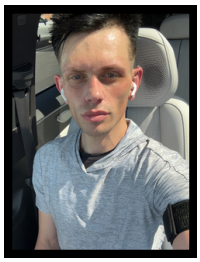
JOHN MALONEY, New York. "I am a real estate salesperson based out of Suffolk County, NY. My connection with PA is baby Jordan Simmons, who has been diagnosed with this rare disease. In 2024, Jennifer was rushed to the hospital and Jordan was born premature. It was clear to the family that they needed to pool their resources to provide Jordan with around the clock care. It was an honor to be there to guide the families through this incredibly stressful time to find a home large enough for the whole family to live in. They are people with hearts of solid gold, and I would do anything for them. I feel very grateful for the opportunity to accept this bib and use the NYC marathon experience to raise awareness and raise funds for a charity that could help our beloved Jordan".

So grateful for your commitment to help Jordan and all the PA families, John!

DEBORAH SLIPETZ, Massachusetts. Deborah is a biotech and pharma executive dedicated to developing medicines that improve the lives of people with serious medical conditions. She is also a runner and fellow parent who understands the uncertainty and strength that come with raising a child with a complex illness. When her daughter developed a chronic kidney disease and needed a transplant, Deb became her living donor – an experience that forever reshaped her perspective on resilience, hope and the power of community. She ran the NYC Marathon in 2021 in support of cystic fibrosis, and again in 2022 for PAF – the very community she is proud to run for again. Deb is honored to continue raising awareness and critical funds for families like yours. Every mile she runs is for parents navigating the unknown, the children fighting every day, and the believe that no family should face this journey alone.

Thank you so much, Deb, for running for Gabriel and all the PA families once again!

PA RUNNERS CONTINUED



TYLER RYDER, New York. "My name is Tyler and I am 27 years old. When I was 18, I made the decision to completely change my life and take control of my health. Through determination, consistency, and a commitment to becoming the best version of myself, I was able to lose more than 250 pounds naturally. Along the way, I discovered something that would completely change my life: running. What started as a way to stay active quickly became a true passion. Running became my outlet, my motivation, and a reminder of how far I had come. Today, I run between 8-12 miles every other day, something I once never thought my body would be capable of doing. Since the beginning of my journey, I've dreamed of running the New York City Marathon. For me, it represents perseverance, growth, and the belief that with hard work anything is possible. Running it in support of families affected by Propionic Acidemia through the Propionic Acidemia Foundation would make this dream even more meaningful. This cause is deeply personal to me because of my connection to Jennifer Simmons and her incredible son, Jordan Simmons. I have personally witnessed Jordan's strength as he continues to fight this disease, and I've seen the unwavering love and determination his parents show every single day. Their resilience is incredibly inspiring, and it reminds me why raising awareness and supporting research for this rare disease is so important."

Tyler, we are inspired by your determination and commitment too. Thanks for running with us!

HANNAH CENEDELLA, New York.

"My name is Hannah, and I am currently a junior at Colgate University. I have been running my whole life and have always dreamed of running the NYC marathon. Running is such a privilege and I look forward to using it as a way to give back to a community. I am honored to be running for the Propionic Acidemia Foundation and be given the opportunity to bring awareness to the cause".

So awesome you decided to join the team, Hannah. Thank you!

MISSION: The Propionic Acidemia Foundation is dedicated to finding improved treatments and a cure for Propionic Acidemia by funding research and providing information and support to families and medical professionals.

ViISION: To create a future where Propionic Acidemia can be prevented, and any affected individual can be cured and live a productive life.

PAF ACTIVITIES AND FUNDRAISING SPOTLIGHT

UPCOMING EVENTS

- OH Families Fall Fest - TBD
- The TCS New York City Marathon 2026 - 11/1

PAST EVENTS

- Ohio Families Fall Fest 2025 raised $13,605
- Team Tadgh - $13,894
- The TCS New York City Marathon Runners 2025 raised $34,225
- PA Family Day - 11/22/25 Wheeling, IL
- PKU Organization of IL and Allied Disorders Low Protein Annual Meeting - 11/15/2025
- Advocacy Webinar Series - Advocacy 101 - What advocacy is and How you can get involved - 3/3/26 Advocacy 101: Tell your story - Drive Change 3/19/26
- GMDI Conference - 4/22-4/25, St. Louis, MO - Advocacy Sponsor/Exhibitor (see PAGE 1)
- ASGCT/BGTC Annual Meeting Attendee - 5/10-5/14 Boston, MA
- SIMD 2026 Annual Meeting - 5/17-5/20, Rio Grande, Advocacy Sponsor/Exhibitor
- ROAR Consortium Kick-off meeting - 5/26-5/27

MATCHING DONATIONS & VOLUNTEER HOURS

This may enable you to double your donation. Check with Human Resources to see if your employer matches. Some companies have a volunteer program and will donate based on your volunteer hours. PAF is always looking for volunteers.

STOCK DONATIONS

PAF accepts stock donations. Please email paf@pafoundation.com with any questions.

DEDICATED GIFTS FROM INDIVIDUALS

- IN HONOR OF: Alexa Faith Cardone, Allison Ellis, Lucy Harding, Dylan Jaehnke, Reuben Kleckley, Kate Lowry, Reily and Judson Lenert, Leah Masten, Trent McKinley, Gabrielle Millett, Gwen Mouat, Sharlene and Annette Weaver, Chase Workman
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Thank you to all of our Facebook Fundraisers and people that donated to their fundraising pages for birthdays, #GivingTuesday or just because: Jill Chertow, Meagan Blachly, Sarah Mullins, Julia Spitzer Landers, Katie Rodgers, Jenn Simmons, Linda Rogers, Loca Gal, Margaret Ramsook McCoy

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Thank you for using iGive, Goodsearch, Bing and selling on eBay. Every dollar helps.

ADVOCACY

On behalf of the MSUD Family Support Group, HCU Network America, and Propionic Acidemia Foundation, thank you for joining us for our Advocacy Webinar Series.

A special thank you to the Everylife Foundation for all their hard work and for providing these informative webinars to our community. Shannon and her colleague Kendly provided insights on what advocacy is and how to get involved, helpful tips, and helped us start to develop our stories. We are truly appreciative of their time, expertise and passion they shared with our communities.

Feel free to email us to get the links to watch the webinars.

Jill Chertow at SIMD

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Publication Note: The PAF Newsletter is published twice a year. Readers may subscribe by writing to PAF, registering online or calling 847-497-0977. Letters and article submissions are welcome for consideration and may be sent to paf@pafoundation.com or mailed to Propionic Acidemia Foundation, P.O. Box 151, Deerfield, IL 60015-4421. If you would like to be removed from our mailing list or receive the newsletter via email, please contact us.