

Propionic Acidemia Foundation

VOLUME 1, ISSUE 28

SPRING 2020

PAF IS HERE FOR YOU

COVID-19 has challenged us all in ways we never thought possible. You are continually in our thoughts - those affected by PA and those that have other underlying conditions, our caregivers, our healthcare professionals, and essential workers.

PAF was planning on exhibiting and attending the SIMD and GMDI conferences in April which were both canceled. In addition, we have postponed our PA Family Day that was scheduled for April 18th until it is safe to have and when we do have it, it will look different than past events with health and safety continuing to be our number one priority.

We continue to work toward our mission of better treatment and a cure for propionic acidemia and providing information for families and medical professionals.

Please know that we want to hear from you and are here for you. We can be reached by phone, email or mail. **Stay Safe. Stay Healthy.**



PA Registry

Help move research forward for propionic acidemia. Participate in the Propionic Acidemia International Registry.

As of April 1, there are 114 participants. For more information on joining the registry, or to update your information, go to www.paregistry.org.

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OFFICIAL CHARITY PARTNER

TCS NEW YORK CITY MARATHON
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NOVEMBER 01, 2020
2020 TCS NEW YORK CITY MARATHON

JOIN THE PROPIONIC ACIDEMIA FOUNDATION runner's team TO GUARANTEE YOUR ENTRY TO THE 2020 TCS NEW YORK CITY MARATHON

FOR MORE INFORMATION contact teamPAR4@gmail.com.



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.

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

I WANT MY CHILD TO PARTICIPATE IN A CLINICAL TRIAL FOR PA. NOW WHAT?

Quick guide to understand the essential steps that take place in a clinical trial by M. L. Cotrina, PhD

We, the PA community, continuously hear about the arrival of gene therapy for a certain disease, a new cure for another rare disease, a new medication that mitigates the effects of yet another disease. However, it is not unusual that when the moment arrives and we hear that a clinical trial for a new medicine is set to start, families and patients are unsure about what the necessary steps are for the potential new medication to make it to the clinic. Moreover, although we may be interested in participating in the trial, we may not really know what this actually entails.

What is a clinical trial?

In the United States, any new medication or treatment that reaches the clinic needs to be approved first by the Food and Drug Administration (FDA). The FDA sets up very high safety and efficacy standards for every new drug and/or treatment directed to humans. So, before appearing in our pharmacies or clinics, the basic requirement of the FDA is that the safety and efficacy of any new drug or procedure be tested first in a clinical trial.

A clinical trial is a clinical research study designed to test the safety and efficacy of every drug and treatment for humans before it enters the market/clinic.

What are the steps to participate in a clinical trial?

Once a family hears about a clinical trial, there needs to be a detailed conversation between the family and the team in charge of the trial so that there is a clear understanding of what the trial entails and what will be expected from us and our children to take part in it.

The first step to be able to participate in a trial is to meet the “enrollment” criteria for the trial, which are established by the investigators. These criteria must accomplish two aspects: first, no patient is going to have more risk than necessary if he/she participates in the trial (for example, a newborn or a pregnant woman may be at more risk than an adolescent or adult). Second, the participant should be close to



be “the ideal patient” to maximize the chances of success of the trial (for example, if a treatment works preferentially in early stages of the disease, then the trial will likely exclude patients who have lived with the disease for a long time).

Once a participant meets the enrollment criteria, he/she needs to sign a consent form agreeing to participate in the trial. This signed consent indicates that the participant understands the procedures of the study and the risks/benefits involved.

-What happens if the PA affected patient is a child or he/she has some cognitive impairment?

When the patients are children, the parents or legal guardians will be the ones to sign the consent form on behalf of the child. However, from ages 7 and up, the child should also be involved in the decision-making process and may be asked to sign an “assent” form where the study is explained to the child in an age-appropriate language. The parent/s will sign the consent form too, on behalf of the child.

Apart from children, some adults may lack capacity to consent, for example, as a result of trauma, cognitive impairment, dementia, ... and these patients are also considered vulnerable populations. In these cases, only a “legally acceptable representative” (LAR) is authorized to sign the consent form on the patient’s behalf. In the US, each state has its own rules about who is allowed to sign as LAR.

Will the trial hurt my child? Do I want my child to go through the procedures of a clinical trial?

For a family to decide whether or not to enroll their child into a clinical trial, the most important question is: what are the

(continued next page)

Steps in Drug development



CLINICAL TRIAL PARTICIPATION CONTINUED (FROM PAGE 2)

benefits and the risks of this new drug or procedure?

Every family will respond differently to this question, depending on their child. Children with PA, as is the case for any other rare disease, show great variability on how they manifest the disease. Whereas some children lead almost normal lives on a low-protein diet, others are continuously in hospital or have profound developmental challenges.

So, for some families, the potential benefit of participating in a clinical trial outweighs the risks, given the poor quality of life of their child. For other families, it may not be so clear. Also, some trials only involve taking a pill once a week and doing a blood test once a month, whereas other trials require the child to undergo testing every week for 3 hours or even get surgery, often at a clinic far from home. This is a very personal decision and each family has the responsibility to carefully weigh the pros or cons of every procedure and obtain as much information as possible early on.

Why are there so few clinical trials for PA, and why do they take so long to get a new medicine approved?

Development of new drugs and therapies is a very long and expensive process. When scientists identify a compound that might be effective for the treatment of a disease, a lot of experiments (the “preclinical research”) need to be performed before they are confident that it can be used in people. For example, scientists need to check that the compound is not toxic and works first in cells and then in two animal species, at least, like in mice and monkeys, which increase the cost of any trial.

Once the preclinical phase is complete, the company that is developing the new medicine will present all of their data and their clinical plan to the FDA. If the FDA approves the plan, the compound can be moved into an actual clinical trial with patients. Each clinical trial involves a lot of costly steps like hiring laboratories for testing, securing nurses and doctors to oversee the trial, managing personnel that oversee that the study is done properly and the patients do not take unnecessary risks, and so on.

Some estimates indicate that, out of 10,000 molecules that start the process, only one will become an actual medicine. Moreover, it is estimated that pharmaceutical companies spend between 0.5-1 billion dollars over a 15-year span per each compound that hits the market. This is why there are so few trials at any given time.

For rare disease, the process is still more daunting because the small number of patients available to participate in a clinical trial and the difficulty in proving that a compound or therapy

is actually working. This helps explain why the recent success of gene therapy for spinal muscular atrophy come with a price tag in the order of millions of dollars.

What can be done to accelerate the process of drug testing?

One of the biggest obstacles in approval of new medications is that, often, the success observed in the animals where it has first been discovered does not translate to humans once the clinical trial starts. Indeed, over 50% of clinical trials are abandoned because of lack of enough efficacy, loss of funding or not enough patients enrolled into the trial.

A better design of testing platforms at earlier stages or better animal models for each disease may be necessary for a higher success rate in clinical trials. However, it is also imperative that our community understands and considers participation into the process of clinical research so that new therapies can see the light. Having said this, it is also crucial that our families also understand the risk and benefits of each approach in clinical trials.



Glossary:

Inclusion criteria: the criteria established to be able to participate in a clinical trial, like age, gender and a PA diagnosis.

Exclusion criteria: criteria established to exclude those patients that are at higher risk of having complications during the trial, like smokers, or having a heart pacemaker...

ICF, informed consent form: A document designed to explain to every participant the study per se, how many participants will be involved, which procedures will take place and the risk and benefits in the study; whether any compensation will be involved and how the confidential information of the patient will be protected. By signing the ICF, a patient acknowledges that he/she understands the study procedures and the risks and benefits the study involves. This document is not legally binding and the subject may choose to withdraw consent at any time.

Assent: the child’s affirmative agreement to participate in research, generally obtained through a separate consent form written with age-appropriate language.

Guardian (in the context of obtaining consent for research): an individual who is authorized to consent on behalf of a child or adult to general medical care.

Efficacy: measure of whether the compound or treatment produces the “expected” results under the particular circumstances of the study.

PAF AWARDS \$30,591 CONTINUATION GRANT TO EVA RICHARD

“Cardiomyocytes derived from induced pluripotent stem cells as a new model for therapy development in propionic acidemia.” Eva Richard, Associate Professor, Universidad Autónoma de Madrid

There is an unmet clinical need to develop effective therapies for propionic acidemia (PA). Advances in supportive treatment based on dietary restriction and carnitine supplementation have allowed patients to live beyond the neonatal period. However, the overall outcome remains poor in most patients, who suffer from numerous complications related to disease progression, among them cardiac alterations, a major cause of PA morbidity and mortality. In our research, we developed a new cellular model of PA based on induced pluripotent stem cells (iPSC) with the goal of defining new molecular pathways involved in the pathophysiology of PA which could be potential therapeutical targets.

Traditionally, disease pathophysiology has been studied in immortalized or human cell lines and in animal models. Unfortunately, immortalized cells often do not respond as primary cells and animal models do not exactly recapitulate patients' symptoms. So far, patients-derived fibroblasts have been mainly used as cellular models in PA due to their availability and robustness, but they have important limitations.

The ability to reprogram somatic cells to iPSCs has revolutionized the way of modeling human disease. To study rare diseases, stem cell models carrying patient-specific mutations have become highly important as all cell types can be differentiated from iPSCs. We have generated and characterized two iPSC lines from patients-derived fibroblasts with defects in

the PCCA and PCCB genes. These iPSC lines can be differentiated into cardiomyocytes that mimic the tissue-specific hallmarks of the disease. The presence of cardiomyocytes has been easily established by visual observation of spontaneously contracting regions, and the expression of several cardiac markers. PCCA iPSC-derived cardiomyocytes exhibited an alteration of autophagy process with an accumulation of residual bodies and mitochondrial dysfunction characterized by reduced oxygen consumption and alteration of mitochondrial biogenesis due to a deregulation of PPARGC1A. We also evaluated the expression of heart-enriched miRNAs previously associated with cardiac dysfunction and several miRNAs were found deregulated. Furthermore, we found increased protein levels of Herp, Grp78, Grp75, sigma-1R and Mfn2 suggesting ER stress and calcium perturbations in these cells.

We are planning to analyze PCCB cardiomyocytes to compare the results with PCCA and control data. We are working to obtain mature cardiomyocytes in order to perform electrophysiology studies (K⁺ currents) using a whole-cell patch clamp method. We are interested in the study of the tissue-specific bioenergetic signature comparing cardiomyocytes derived from control and PA patients' iPSCs by reverse phase protein microarrays (RPPMA). Future work also includes testing the effect of the mitochondrial biogenesis activator, MIN-102 compound (PPAR agonist, derivative of pioglitazone) and of the mitochondrial targeting antioxidant MitoQ in PA cardiomyocytes.

We would like to sincerely thank the Propionic Acidemia Foundation for supporting our research.



NOTE: Due to COVID-19, the research has been put on hold.

GRANT & SEBASTIAN'S STORY



For most of us COVID-19 has brought the world into our shoes for the first time. The world has started to understand what cold and flu season is like for families who have

a child with Propionic Acidemia. For once we weren't the only ones that were "paranoid" about our children getting ill and having to be hospitalized.

In some ways this is comforting, but in others it totally brought me to my knees. We quickly not only became responsible for our child's medical wellbeing, but also for their social emotional and academic well being. While most of us have had to provide support to our children to ensure that they are progressing, I think few of us have had to take on the role of teacher, therapist, mom, nurse and also work a full time job all day everyday.



By occupation I am a teacher, but I'm not a special education teacher or a physical therapist, occupational therapist and speech teacher. Even though we fight for our children every day, I think this has been a difficult

journey. It's set a new normal not only for us, but for the world as a whole. It's brought a new fear into our lives, with many unknowns. Now even the typical hospital stay has become unnavigated waters, with new protocols put into place daily, sometimes hourly. How do we fit all of it into 24 hours? For many of us the extended support system we have built has been cut, our home health nurses, therapists, teachers etc. have been removed, except for Zoom, and who are

we kidding Zoom isn't the same as people being in our homes and helping us, giving us an hour here or there of adult interaction.

Yes, these times are rough, but I know brighter days are ahead, or at least warmer ones. Which brings about a whole new round of changes. During the summer the park and pool are our go to places to keep the boys entertained. Those options are gone for this summer. What are we to do? We've already been on an extended period of summer-like routine. How are we going to keep these kids cool and entertained for another 3 months? For our family the answer was to redo, our luckily small, backyard into an entertainment area for the boys. One where they could go and be outside, but not have to be exposed to germs. The backyard has been mulched, the blow up island has been inflated and the shade has been brought in.

While some of this virus has familiarity to it because we deal with PA, much of it brings about new questions and possible issues. And that can be scary, we are fortunate to have the PA community to reach out to. Keep reaching out, share your joys, frustrations, fears, with us. Ask for help when you need it and above all know for once, we aren't the only one living in a sea of unknowns. We are all in this together. Our family hopes you have a wonderful adventure of some sort this summer.

Amber and John,
Grant and Sebastian, Age 7



FIGHT COVID-19 RIGHT WHERE YOU ARE

Propionic Acidemia Foundation, working with you, can help fight this virus. Whether you've had COVID-19 or not, you can help researchers understand the full human experience. You are the best source of information. The community's power to come together to solve big problems has never been clearer. Our short questionnaire will help researchers understand long-term health effects, treatments, and how we can best prepare for future pandemics. <https://peer.lunadna.com/paf-org/>



COVID-19 Information for Patients with Propionic Acidemia and their Families

COVID-19, the coronavirus currently causing global concern, is of particular concern for the elderly and those with underlying health issues. We acknowledge that this is a difficult time with much uncertainty and recommend that taking precautions is currently the best strategy. In the following **FAQs**, we have attempted to provide answers to some of the questions that are relevant to those affected with propionic acidemia (PA) and their families.

Coronavirus (COVID-19) is a respiratory illness caused by a novel (new form) of coronavirus. Individuals affected with COVID-19 typically develop fever, cough, and shortness of breath. There may be mild cases that may not have all of these three key features. Others may have general body aches or flu-like symptoms. Complications of COVID-19 can include development of pneumonia in the lungs, respiratory failure, multi-organ failure and, in some cases, death.

In order to avoid complications that may trigger instability in PA such as elevated ammonia levels, we recommend that individuals with propionic acidemia and their families be particularly careful about exposure and contact their physicians immediately if they suspect they are symptomatic. This is the time to be in close contact with your metabolic specialist about any concerns that you may have about risks of exposure, and to have a plan in place in the event that there is an COVID-19 exposure in your family.

COVID-19 is highly contagious and can spread from person to person through coughing, sneezing, or touching surfaces. The World Health Organization (WHO) has declared the coronavirus outbreak a pandemic, meaning a worldwide epidemic. COVID-19 has been reported in countries on all continents except Antarctica. In the US and Europe, clusters of COVID-19 outbreaks through community transmission have been identified. This is an evolving situation and there are still many unanswered questions. We encourage you to refer to sources that provide updated and reliable information like the ones listed below:

<https://www.cdc.gov/coronavirus/2019-nCoV/index.html>

<https://www.who.int/emergencies/diseases/novel-coronavirus-2019>

Please also check for area-specific resources available on the official websites of your Local and State Government. Additionally, hospitals that provide care for you or your PA-affected family member should have information on their preparedness and policies regarding COVID-19. Please make sure to get your information from official and reliable sites, as there are many “websites”, Facebook pages, and other social media sites that are spreading misinformation.

A. Risks of infection with COVID-19 and precautions that may help reduce the risk of infection

Are individuals with PA at higher risk of complications from COVID-19?

- 1) Currently, it is not known whether individuals with PA or other inborn errors of metabolism are at increased risk for developing COVID-19 or its respiratory complications. Individuals of any age or health status can become infected and develop complications. Experts believe that although healthy children are generally less severely affected by the virus than adults, they can be carriers of COVID-19 and infect vulnerable PA children and adults.

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The Propionic Acidemia Foundation Newsletter is designed for educational purposes only and is not intended to serve as medical advice. The information provided should not be used for diagnosing or treating a health problem or disease. It is not a substitute for professional care. If you suspect you or your children may have Propionic Acidemia you should consult your health care provider.

The Propionic Acidemia Foundation does not recommend nor endorse any particular products, companies, or manufacturers.

2) **An acute viral (infectious) illness in individuals with PA can trigger a metabolic or hyperammonemic crisis.** Therefore, individuals with PA and their caregivers/families should take extra precautions. This is especially important in PA individuals who have a history of episodes of metabolic. We advise that you contact your metabolic physician and have a plan in place in the event that the PA-affected individual needs emergency care during the pandemic.

3) Individuals with PA who have received an organ transplant typically take medications that suppress their immune system. Individuals with liver transplant also need to take extra precautions and should seek the advice of the medical professionals managing their posttransplant care.

What steps can we take to prevent infection in individuals with PA?

The virus is thought to spread mainly between people who are in close contact with one another (within about 6 feet) through respiratory droplets produced when an infected person coughs or sneezes. These viral particles may also stay suspended in the air. An infected person without symptoms can also carry the virus and infect others. A person could get COVID-19 by touching a surface or object that has the virus on it and then touching their own mouth, nose, or eyes. COVID-19 has been reported to live on surfaces, like plastic and metal, for several days.

The following precautions may help decrease the chance of infection. The most effective preventions include hand hygiene and social distancing:

- Wash your and your child's hands with soap for at least 20 seconds frequently. Scrub the palms, fingernails, between the fingers and the backs of the hands. If soap and water are not available, use hand sanitizers that contain at least 70% alcohol. These measures are especially important before eating, touching your face, or after touching shared surfaces such as doorknobs, faucets, keyboards, etc. Frequently disinfect surfaces with Lysol spray or disinfecting wipes.
- Teach children to avoid touching their face as much as possible.
- Avoid (and teach your child) touching surfaces outside your home that carry viruses, such as doorknobs, hand railings, etc. Use hand sanitizer or wash hands immediately.
- Avoid large crowds. If you need to go out, try to maintain a distance of at least 6 feet from others. Don't shake hands or hug others. Avoid using public transportation.
- Ask everyone entering your home to wash their hands. Do NOT be shy about asking individuals whether they have viral symptoms or have been exposed and, if so, telling them to stay away.
- If someone in your household develops signs of viral illness, have them wear an earloop mask and isolate them to avoid exposing the individual with PA and other family members.

Should my PA-affected child stay home from school/day care and other activities? The Centers for Disease Control (CDC) is recommending social distancing as a measure to curb the spread of the virus. Please discuss this with your metabolic doctor, and work with your school. Schools are working hand-in-hand with their local departments of health to determine if schools need to be closed. Most schools are developing online curriculums and programs that can be accessed from home.

We are planning travel, is it safe to travel with a child or adult with PA?

Please discuss any travel plans with their metabolic doctor. The CDC advises refraining from non-essential travel. We recommend NOT taking a PA-affected person on a cruise and to avoid airports and air travel. Read the CDC's travel advisory at: <https://www.cdc.gov/coronavirus/2019-ncov/travelers/index.html>

If you or a family member have traveled in the last 2 weeks, call your doctor to determine if a quarantine is needed.

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B. Management of PA during the COVID-19 pandemic Is it safe to take my child for a clinic appointment in the hospital?

1) The healthcare facility or region where you receive care may have specific policies regarding infection control and screening for COVID-19. Healthcare facilities may also have limitations on the number of caregivers who will be allowed to accompany children during these clinic appointments. Please refer to these policies before you go take your child to the appointments. Some PA clinics are planning for telemedicine visits. Ask about the availability and appropriateness of a telemedicine visit.

What additional measures should we take for management during these times?

- 1) Contact your metabolic team and have a plan in place for evaluation and management of a potential metabolic crisis. Emergency department visits may not be recommended during the pandemic and an alternative plan should be in place.
- 2) The COVID-19 outbreak could potentially affect supply chains. Work with your physician's office and pharmacy to maintain an adequate supply of medications, supplements, and metabolic formula that are critical for management of a child or adult with PA. Don't forget gloves, saline and other supplies needed for tube feed care, ports, etc.
- 3) Ask your PA physician what type of fever-reducing medicine the PA-affected individual can take, and have a supply on hand, along with oral rehydration solutions to use as recommended.
- 4) Have a plan for where and how family members with symptoms of COVID-19 will be isolated in the home, and how you would prevent their contact with the PA-affected family member.
- 5) In the event the primary caregiver has symptoms of COVID-19, have a plan for who will take over the care of the PA-affected person, measure and administer their PA medications, formula and supplements, and monitor their PA diet and protein intake. Make sure to post emergency contact information for the PA metabolic physician in a conspicuous place (like on the refrigerator).

What should I do if a PA-affected child or adult develops symptoms of COVID-19?

Call your primary healthcare provider and your metabolic physician's office to inform them of the symptoms so that they can advise you regarding the next steps for evaluation and treatment.

Cloth Face Covering Do's & Don'ts:

DO:	DON'T:
<div style="display: flex; justify-content: space-around; align-items: center;">    </div> <ul style="list-style-type: none"> ✓ Make sure you can breathe through it ✓ Wear it whenever going out in public ✓ Make sure it covers your nose and mouth ✓ Wash after using 	<ul style="list-style-type: none"> ✗ Use on children under age 2 ✗ Use surgical masks or other personal protective equipment (PPE) intended for healthcare workers



cdc.gov/coronavirus

ZENTE'S STORY

Life has slowed us down as the pandemic of Covid19 keeps our family lockdown. Thanks to God, we are all fine. Particularly, protecting our Zente (8,5 PA), we really take it seriously to stay home and not to meet anyone. Our sons are studying through digital education, their teachers are doing their best. My husband had to close the restaurant he had been running... this may bring financial difficulties. It is so hard not seeing our parents either. I'm praying for my father (over 65) who is working as a pharmacist at a hospital that is being used now to treat corona patients. He cannot stay home being the only pharmacist there.

There are a lot of secrets that let themselves be revealed though. What a self-made bread tastes like, how exciting a bike tour in our small backyard can be, how the forest nearby smells, and how our older son, Pál, managed to make Zente drink his whole glass of water by mouth – it does not happen everyday, as Zente is 98% tube-fed. There is no need to worry about the news that nowadays are spread about Hungary either. Our family is grateful for our government's wisdom and humanity. We are praying for the soonest possible end of the pandemic and the safety of all our families.

Ágnes, Hungary
April, 2020



CHASE'S STORY

Hello Everyone,

It has been four years since I have written an update on Chase. He is 22 years old now and I am happy to report he is doing great and staying healthy during this very difficult time of the pandemic.

During these past four years Chase has required two hospitalizations. In April of 2017, Chase became very ill with pneumonia. He was on a ventilator for three weeks. We weren't sure he was going to make it, but Chase is a fighter and we had so many people praying for him. Many of you were part of those prayers, and I cannot thank you enough. He has been metabolically stable for the most part except, of course, when he is ill. In addition to the genetics doctor and the neurologist, we are seeing a cardiologist for long qt syndrome. We also see a nephrologist and learned recently that his kidneys are only functioning at fifty percent. Despite these challenges, Chase remains relatively healthy.

Chase graduated from high school last spring. It was so exciting for him. I loved his school so much, but it was time for him to move on. The biggest challenge was finding an appropriate



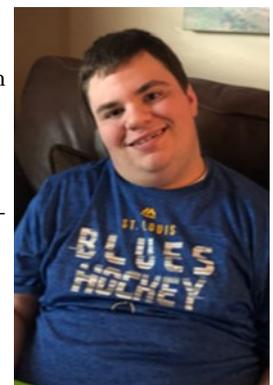
program for him post-graduation. I was able to find a program that could support all his needs and was able to obtain the funding for the program. That was a huge relief!

Chase is such a happy person and brings a smile to everyone he meets. He loves getting the birthday cards from the warrior birthday club. He enjoys spending time with his family, especially, his older brother Kyle. He likes to watch sports on tv and really enjoys going on walks being pushed in his wheelchair. He is able to walk on his own but he tires pretty easily.

I hope that during this very difficult

time all of our family members remain healthy. It is such a scary time but having an organization like this is so helpful. I cannot thank the PA foundation and the OAA enough for everything they do.

Amy
Mom to Chase, 22 PA



PAF EVENT & FUNDRAISING SPOTLIGHT

UPCOMING/ONGOING EVENTS

- **Fall 2020 - 15th Annual Tailgate Party & Corn Hole Tournament for PAF**, GwenForACure.com for details
- **Fall 2020 - PA Family Day**, See pafoundation.com for updates
- **10/18/2020 - Team PA Runners, Nationwide Children's Hospital Columbus Marathon, Ohio**
- **11/1/2020 - NYC Marathon**

PAST EVENTS

- **In honor of Rare Disease Day, The Villainettes made a donation of \$1330 in honor of Diego Cardona Corporate Matching**

GIFTS: This may enable you to double your donation. Check with Human Resources to see if your employer matches. It makes a big difference.

VOLUNTEER HOURS: Some companies have a volunteers program and will donate based on your volunteer hours. PAF is always looking for volunteers. Please check with Human Resources to see if they have a program.

INTERNET

Thank you for using Igive, Goodsearch and AmazonSmile and designating Propionic Acidemia Foundation as your charity and setting up Facebook Fundraising Pages. Every dollar counts.

PA RUNNERS UPDATE

Support the effort to improve treatments and create new therapies for PA or join the 2020 team with a distance and race of your choice. All abilities welcome!!! Contact Marisa Cotrina for more information: teamPAR4@gmail.com.



FACEBOOK: Thank you to all of our Facebook Fundraisers and people that donated to their fundraising pages: Thank you for making a difference. Lao Marie Robertson-Sherwood's Birthday, Kierstin Berg's Birthday, Jason Vandergriff's Birthday, Elisabeta Riverqueen's Birthday, Brittany Smith, Debbi Buck, Selvi Pragasm, Jaime Lynn's Birthday, Brayden Murphy's Birthday, Cindy Davis's Birthday, Kelli Johnston's Birthday, Sonia Phillips' Birthday

STOCK DONATIONS: PAF is now accepting stock donations. Email paf@pafoundation.com with any questions.

DEDICATED GIFTS FROM INDIVIDUALS:

Among the many contributions received, the following is a list of some that were dedicated to those who have inspired the giver.

- **In Honor Of:** Kristin Boecker, Nila Branch, Kaitlin Burns, Diego Cardona, Lucy Harding, Dylan Jaehnke, Nalani Johnson's birthday, Zente Kiss, Hogan Koehl, Laura Lemire, Riley and Judson Lenert, Steve and Judy Lenert, Gabriel Lopez, Kate Lowry, Dania and Edgar Jr. Martinez, Zach Matz, Trent McKinley, Gabrielle (Gabby) Millett, Grant & Sebastian Moss, Gwen Mouat, Brandon Napiwocki, Carolyn Schlein, Benjamin Sweetman, Isabella Velazquez, Brett Young
- **In Memory Of:** Bob Buck, Alice and John Dawe, Jordan Franks, Vincent Philip Franze, Kerrie Fessler, Connor McKillop, Nicholas Phillips, Margaret Reardon, Abraham and Amanda Sleiman, Talli Smith,, Angelica Stageman, Kirstyn Tripp, Denis Willens

Thank you for all donations and the kind notes we receive throughout the year. Your support overwhelms us and continues to be a source of inspiration. PAF couldn't do what we do without your incredible support.

HEMOSHEAR DONATION

HemoShear is a company developing an investigational oral drug treatment for PA and MMA. Their team stepped out in February for Rare Disease Day by running/walking and biking 2500 miles to raise \$2,500 to help support the important work of the Propionic Acidemia Foundation. Thank you HemoShear!



GWEN AND ALLISON'S ZOOM CALL

Gwen and Allison are friends who both have PA. They recently did a Zoom call to stay in touch because they cannot visit each other. Here are some topics and tips they recommend if you choose to have a Zoom call with your friends!

TIPS:

- Contact friends to set it up
- Sing songs together
- Play games together
- You can use headphones
- Sometimes fun backgrounds will work

TOPICS:

- What do you do during the day?
- Do you play Disney Magic Kingdoms?
- What's your favorite movie?
- Do you watch "Barney"?
- Look up "Muppet Treasure Island"!
- Talk about "Full House".
- Sing "*The Green Grass grows all around, all around...*"



Thank you for all donations and the kind notes we receive throughout the year.

Your support overwhelms us and continues to be a source of inspiration.

PAF couldn't do what we do without your incredible support.

We want to hear from you! Have a PA story to tell, event to promote or news?

Fall newsletter submissions due by August 1, 2020.



Warriors Birthday Club

The students at Oak Lawn-Hometown Middle School made such amazing and memorable cards last year for participating families. We are thankful they have volunteered to do it again this school year. Please sign up a patient or sibling for the Warriors Birthday Club at <http://www.pafoundation.com/warriors-birthday-club/>. If you signed up last year, you will need to sign up again, so we have current information.



SEARCHING FOR A CURE
HOPE FOR OUR CHILDREN

Propionic Acidemia Foundation
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PROPIONIC ACIDEMIA FOUNDATION

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