Soft Bones
Finding the Key to HPP

WINTER 2022 EDITION

THE BARE BONES

Official Newsletter of Soft Bones, Inc., The US Hypophosphatasia Foundation

Events Recap

2022 Cannonball 5k: Run, Roll or Stroll

This year, Soft Bones hosted its first-ever 5k: The Cannonball Run, Roll, or Stroll! During the month of October, participants were urged to complete their 5k by running, rolling or strolling, any time, anywhere! Some members of our community were even able to complete it together.

With 67 registrants, the HPP community came together to bring global awareness to hypophosphatasia. In total, we raised $1,675.00 and an additional $1,006.50 in donations.

Soft Bones would like to extend a huge thank you to all of the participants.

Rachael Alford
Pat Canada
Dominic Carrano
Maryanne Caruso
Jane Castello
Kristi Culver
Brenda Degnan
Laura Ekas
Linda Eramian
Catherine Fenlon
Jennifer Flanagan
Deborah Fowler
Jason Fowler
Caroline Fox
Kim Fox
Ryan Fox
Carolina G. M.
Bill Goodbar
Chloe Goodbar
Denise Goodbar
Jim Grady
Michael Greco
Judith Harris

Amanda Hayward
Claire Hernon
Megan Hessel
Ann Houston
Daina Janowski
Kelly Jones
Noel Lammey
James Latimer
Laiken Latimer
Preston Latimer
Pamela Legett
Eric Levesque
Caden Lucarelli
Kinsley Lucarelli
Brad MacDougall
Hillary MacDougall
Daniela Martinez
Seametso Maseng
Debbie McGarity
Charlie McGarity
Todd McGarity
Kirsten Mitchell
Mary Mulcahy

Lila Namisnak
Maya Namisnak
Pam Neumann
Deana Ollis
Keith Ollis
Tricia Oppelt
Allison Pishko
Traci Raynor
Cindy Reasor
Christine Salomon
Nellie Sanders
Cannon Sittig
Clark Sittig
Colby Sittig
Grace Sittig
Travis Sittg
Connie Tavanis
Samantha Tracy
Ting Yi
Briley Zibilski
Danielle Zibilski
Grayson Zibilski
Travis Zibilski

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View our Fall 2022 newsletter **HERE**

Events Recap

2022 World HPP Day

This year’s World HPP Day was a smashing success! Celebrated every year on October 30th, the 2022 theme was “HPP ON THE MOVE.” In an effort to create momentum around awareness of hypophosphatasia across the world, Soft Bones members chose to participate in a variety of ways. This year's theme was a celebration of movement, both physically and metaphorically, so while some focused on drawing attention to our rare disease cause, others participated in the 2022 Cannonball Run, Roll or Stroll 5k. By moving together, we started a movement to educate others about HPP. Overall, the activities of World HPP Day were felt around the globe.

To learn more and keep an eye out for news about the 2023 World HPP Day, click **HERE**.

2022 Southeast Region Meeting

The Southeast Region held a meetup in St. Augustine, Florida on October 22, 2022 where patients connected with members of the Soft Bones community. Region Leads Blynda Kellner and Kirsten Mitchell hosted a relaxed and casual meeting at a local library. The meeting allowed for patients and caregivers to reconnect with each other and have great conversations, while also having a representative from Alexion AstraZeneca’s OneSource program to provide an overview of their services. Soft Bones' Patient Programs and Service Manager, Muffy Mulcahy, introduced us to some of the Soft Bones programs and resources. Finally, a holistic health expert attended and provided tips on how to approach life with a healthier mindset. It was a great meeting and wonderful to reconnect with everyone.

Events Recap

EL-PFDD

On November 15th, Soft Bones hosted its very first Externally-Led Patient-Focused Drug Development (EL-PFDD) meeting. EL-PFDD meetings are designed to educate members of the Food and Drug Administration (FDA) and other key stakeholders about rare diseases so that decision makers have a better understanding of these conditions as they consider new therapeutic options submitted for approval. With several treatment approaches in development for HPP, the time was right to host this meeting and continue to foster education through first-hand accounts of patients and caregivers living with HPP.

Over 200 people tuned in for the meeting. The audience was comprised of key stakeholders including patients, caregivers, medical professionals, researchers, industry and representatives from the FDA. The meeting was moderated by Soft Bones President and Founder, Deborah Fowler, and James Valentine from Hyman, Phelps and McNamara. Members of the HPP community participated by sharing their stories, serving as panelists, submitting written comments, calling in and voting in live polling sessions that took place during the meeting. A *Voice of the Patient Report* will be published and shared with the FDA and other key stakeholders in the coming weeks.

To view a recording of the EL-PFDD click **HERE**.

A Day of Pampering to Benefit Soft Bones

Ladies gathered for a day of pampering on Sunday, November 20, 2022 at Enriched Aesthetics in Morristown, New Jersey. It was a peaceful and relaxed get together with plenty of time to sit and chat. Cris Morin, the owner, gave a tour of her beautiful salon and educated attendees on products and services. Soft Bones ultimately raised $400. A special thank you to Cris and Enhanced Aesthetics for a wonderful event.

EA Enriched Aesthetics

Pictured to the left: Deb Fowler receiving the $400 donation from Cris Morin

Program Highlights

2022 Fall/2023 Winter Hippo Squad Box

The fall and winter Hippo Squad boxes were a great success! Our fall Hippo Squad package had a "Join The Movement" theme to coincide with World HPP Day. Inside this package, Squad members found a pedometer, a strength band and the book "Zara Shares Her STRIPES," a story about animals learning to appreciate each others' differences. The kids used a Candy Land-style map to track their progress and join the movement by adding stickers, coloring and writing about their day-to-day movements.

Just in time for the holidays, the winter Hippo Squad package had a baking theme with some festive cookie recipes. Included in the mailing was a snowflake cookie cutter and Squad members learned that snowflakes are like HPPers in that no two are exactly the same. Bakers also received aprons and chef hats. Another educational message compared recipes to HPP, since if you don't have enough of one ingredient for a recipe, it can impact the end result. Similarly, the body has different chemicals that make it work and when one chemical is low, it can impact how the body functions. Kids shared their photos baking holiday goodies and it was wonderful to see everyone's smiling faces while baking their holiday goodies.

Program Highlights

Teen Advisory Council Launches New “Stick It to HPP” Program

The Teen Advisory Council (TAC) is sticking it to hypophosphatasia (HPP) with a new program aimed at supporting kids who are ready to begin administering their own injections. The program, aptly named “Stick It to HPP,”' was created by the members of the TAC, which includes teens who have overcome their own fears of self-injecting, as well as siblings and children of HPPers, and their friends. The goal was to create a program that would encourage children, on enzyme replacement therapy, to learn to self-inject and gain independence. HPP teens, on the TAC, shared that starting their own injections was not only more convenient for them, it also allowed them the freedom to do things like go to camp, travel, or sleep over at a friend’s house.

Kids who sign up for the program will be sent a starter kit containing educational and motivational materials that TAC brainstormed about and felt would be helpful to someone who is just getting started. Materials include a chart of injection goals (with each goal met, they will receive an acknowledgement of their accomplishment), tips for easing the fear of needles/injections, a stress ball, a shot blocker, an ice pack, breathing techniques and other "TAC hacks". The TAC even created a "Stick It To HPP" music playlist to help kids get pumped up for their injections. Participants can also request encouraging videos or calls from other teens to talk about their experiences or just for a pep talk. To learn more, please email **info@softbones.org**.

Join the Conversation: HPP AND ME

If you’re not already a member of HPP AND ME, take some time over the holiday break to check it out. Currently with over 500 registered members, this community is a forum for Soft Bones members to ask questions, find support and answers to questions, share their journeys, and read the latest research, important news and announcements. Unlike Facebook, this community is organized by topic so patients and caregivers can easily navigate content and ask targeted questions. A few of the topics recently discussed have covered enzyme replacement therapy, migraines, genetics, new member stories, research and scientific publication discussions.

HPP AND ME

Click **HERE** to join in on the conversation!

Program Highlights

You Could Be Our Next "Patient of the Month!"

Each month Soft Bones features an HPP patient who shares their journey in our ongoing series "Patient of the Month." These stories highlight the obstacles faced along the way to a diagnosis, and the path forward.

Previous "Patients of the Month" have shared that they found the process of writing their stories to be therapeutic and felt more connected knowing they were not alone along the way.

Nominate yourself or an #HPP patient who inspires you today by emailing **maryelizabeth@softbones.org**.

Give the Gift of Hope

'Tis the season of giving and one way to bring meaning to the holidays is to give the gift of hope to those who need it the most. Managing HPP for patients presents a burden as people need to learn and understand the physical, mental, and emotional components of a complex rare disease. They are often confused, uncertain and scared. Soft Bones gives these families hope by offering a community and support as they navigate their HPP journey. Contributions made today will support scientific advancements, provide important resources and help us continue to build a support community for patients and their families living with HPP. It's not too late to make an impact! As we reflect on this past year filled with successes and new challenges, we are continually grateful for the support and generosity of others.

Our greatest gift is having all of you in our community.

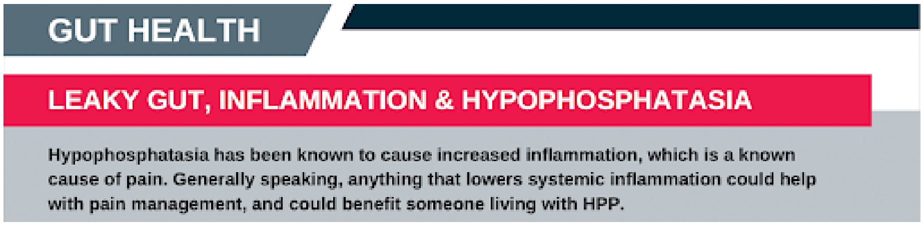
DONATE TODAY

We could never do what we do without each and every one of you. Thank you in advance for your generous support!

Leaky Gut Fact Sheet

As a result of feedback received from patients, last month Soft Bones created a new resource on leaky gut, detailing important information regarding gut health, inflammation and hypophosphatasia. This fact sheet provides facts, figures and statistics on this condition, including approaches about what can be done to help a leaky gut, top foods to avoid, and more.

To learn more, click **HERE**.



GUT HEALTH

LEAKY GUT, INFLAMMATION & HYPOPHOSPHATASIA

Hypophosphatasia has been known to cause increased inflammation, which is a known cause of pain. Generally speaking, anything that lowers systemic inflammation could help with pain management, and could benefit someone living with HPP.

Exciting News

HPP Awareness Week on TikTok

Our very own Deb Fowler decided to hop on the TikTok train and participate in the viral #OneThingAboutMe trend. If you're not familiar with this trend, participants write and sing their own rap to the tune of the popular Nicki Minaj song "Super Freaky Girl." The lyric "One thing about me, I'm the baddest alive" from Minaj's song inspired the phenomenon. Over 800,000+ TikTok users have jumped on the trend and used this sound to rap about their personal lives. Deb's spin on the trend gave us insight into her and her family's own personal experience with hypophosphatasia while also bringing awareness to World HPP Day, Soft Bones and hypophosphatasia in general. On top of that, she raised nearly $4,500 for Soft Bones because of this video!

To view the TikTok, click **HERE**.

Maher Family Research Grant Winner

Soft Bones recently announced the recipient of the 2022 Maher Family Research Grant. As an organization committed to bringing forward advancements and education to those affected by HPP, it was an honor to award this grant to Nan Hatch, DMD, PhD. As an associate professor of dentistry at the University of Michigan School of Dentistry, this $25,000 grant will support Dr. Hatch's studies to establish essential roles for tissue-nonspecific alkaline phosphatase (TNAP) in muscles and determine how TNAP modulates muscle function. Dr. Hatch's previous findings led her to question if cell cycle and/or energetic abnormalities might also be relevant to the muscle weakness and fatigue found in some individuals with HPP.

When asked for a comment, Dr. Hatch stated that, "This grant will provide me with the resources to investigate muscle development and function in HPP, which is a new line of research for us. I previously focused on the role of TNAP, the enzyme that is deficient in hypophosphatasia, on bone cells. This opens a new avenue for me to determine how TNAP deficiency leads to muscle weakness and fatigue, which are prominent features of hypophosphatasia for some individuals."

Soft Bones funds research to better understand HPP with the goal of finding a cure and has awarded over $475,000 in research grants since 2014. The organization supports HPP investigators through annual grants such as the Maher Family Annual Hypophosphatasia Research Grant.

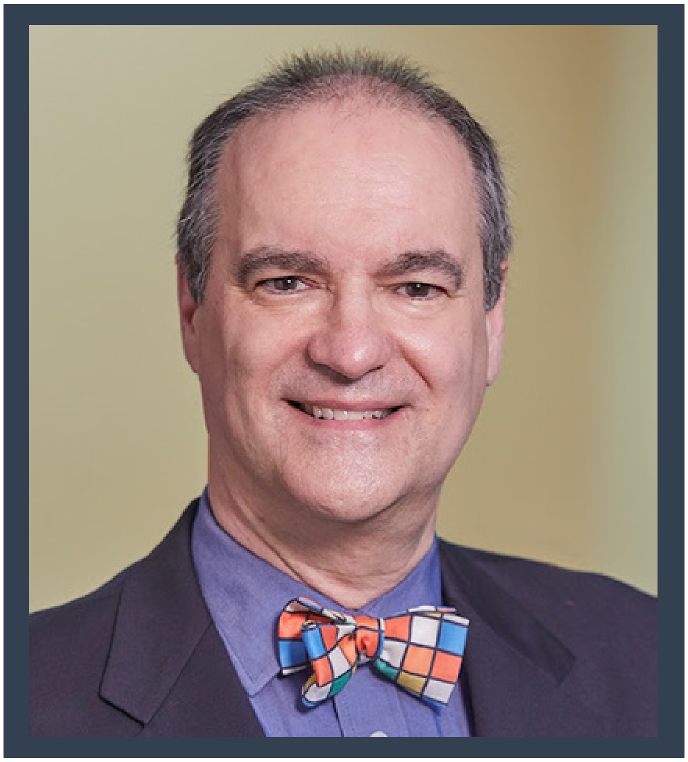
More information about hypophosphatasia, as well as existing and ongoing research can be found **HERE**.

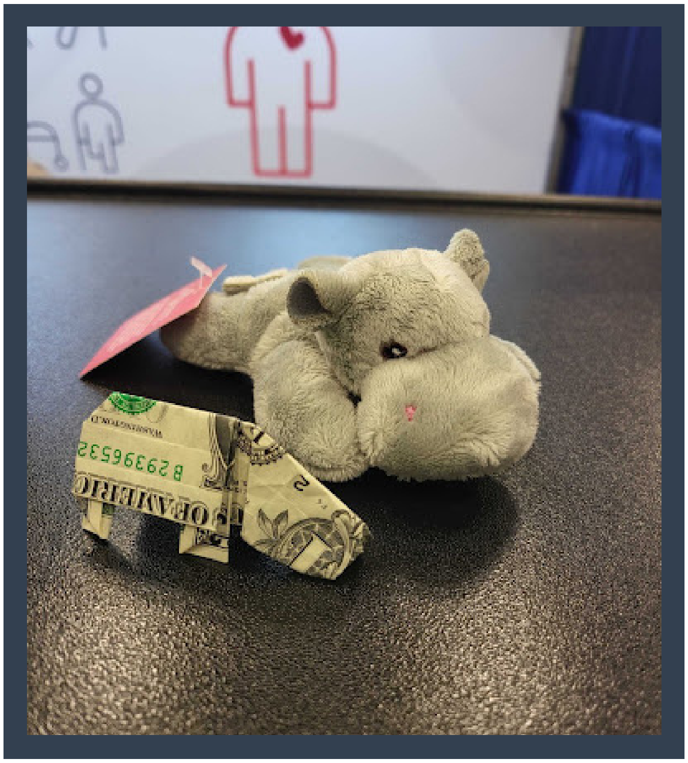
Nan Hatch, DMD, PhD

Exciting News

Origami Doctor: Gary Gottesman

Dr. Gary Gottesman of Washington University School of Medicine (WUSM) in St. Louis has been fondly nicknamed the "Origami Doctor" by his hypophosphatasia (HPP) patients. Not only is he globally renowned for his work with rare skeletal and metabolic disorders, but his patients also adore him for his creativity and kindness. He is known for presenting his patients with origami designs he has created out of dollar bills. These creations give patients and their families the opportunity to forget, even if for just a moment, why they were seeing him in the first place. His latest origami specialty is a hippo in honor of World HPP Day on October 30th.





As a Professor of Pediatrics and Medicine at WUSM in St. Louis in the Divisions of Endocrinology & Diabetes, Genetics & Genomic Medicine and the Division of Bone & Mineral Diseases, Dr. Gary S. Gottesman is helping Soft Bones pave the way to find the key to unlock information about HPP. In addition to his time at WUSM, he worked at the Saint Louis University (SLU) School of Medicine, the National Human Genome Research Institute and the National Institute on Aging. He also served on the SSM Health Cardinal Glennon Children's Medical Center Ethics Committee and was Academic Chair of the Department of Physician Assistant Education and Program Director of the Physician Assistant Program at SLU. Currently, he is also on the Scientific Advisory Board at Soft Bones, Inc.

Exciting News

Centers Of Expertise - A Giant Leap for the Establishment of an HPP Network

Soft Bones is excited to share our progress in the establishment of a framework for a HPP Network (referred to as Centers of Expertise in last year's annual report). A note of thanks to the diverse steering committee (SC) and working group (WG) members inclusive of patients, caregivers, nurses, geneticists, physicians, physical therapists, and others, for their commitment and contributions over the past few months. Their insight and guidance provide a clear blueprint for the creation of a HPP Network, which will assist the HPP community and healthcare providers to successfully obtain or provide appropriate comprehensive care. The SC identified five critical tactics to be addressed. Executing these tactics will enable greater education, navigation, and consultation for treating and those impacted by HPP. Early next year, the SC and WG will continue to refine and produce concrete deliverables to launch the Soft Bones HPP Network, including a HPP Patient App. We look forward to sharing further updates in the next newsletter.

Cannon - Finalist for Rare Voice Award

Exciting news in our HPP community: Our very own Cannon Sittig was named as a 2022 Teen Advocacy Finalist for the EveryLife Foundation for Rare Diseases Rare Voice Awards! Cannon was recognized for his work on the Ensuring Lasting Smiles Act.

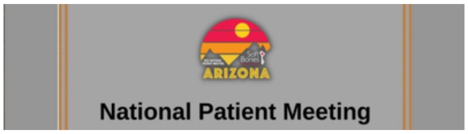
Read more by clicking HERE.

Upcoming Events

2023 National Patient Meeting

The 2023 National Patient Meeting is approaching quickly! The event will be held February 17th-18th in Phoenix, Arizona. This important event will bring the HPP community together to learn valuable information about hypophosphatasia from healthcare experts and peers. There is no cost to attend this meeting, and a limited number of travel grants are still available, so be sure to sign up as soon as possible. Registration closes on January 10th to ensure that hotel rooms are available. Last year, nearly 150 attendees (patients, caregivers and exhibitors) participated in this event, making it the first in-person gathering since the pandemic. You won't want to miss out!

Click **HERE** to learn more and register for the event.



Countdown To Rare Disease Day 2023

Rare Disease Day (RDD) is coming up soon on February 28th! This year's theme is #ShareYourColors, and we urge Soft Bones members to participate by posting pictures, videos and experiences with hypophosphatasia in an effort to raise awareness.

What is RDD? It is an internationally coordinated effort for people living with rare diseases to achieve fairness in social opportunities, healthcare, and access to diagnosis and therapies. Every year on February 28 (or February 29 in leap years), AKA the rarest day of the year, RDD is honored. The purpose of this day is to raise awareness and provide a focal point for rare disease advocacy activities at the local, national and worldwide levels. Though RDD is patient-led, anyone, including individuals, families, caregivers, healthcare professionals, researchers, clinicians, policymakers, industry representatives, and the general public, can participate by sharing their stories. This year's goal is to impact and improve the lives of 300 million people globally by Sharing Your Colors through social media, events, illuminating buildings, monuments, and houses, sharing experiences online and with friends, calling on policymakers and casting a light on people suffering with a rare disease. To learn more about Rare Disease Day events near you, click **HERE**.

A Penny For Your Thoughts - Or $2 For Your Opinions!

Between February 6th and March 7th, anyone can give to Soft Bones without spending a dime. For the third year, we are partnering with HundredX Causes to raise money by taking surveys on products, companies and brands you use everyday. For every survey completed, a $2 donation will be made to Soft Bones. This year we are setting out to raise a total of $15,000! Help us spread the word and invite your friends and families to get involved, share their opinions, and join us in supporting the HPP community! More information will be available in the coming weeks.

A Word From Our Partners

National Organization for Rare Disorders (NORD)

NORD's Hypophosphatasia (HPP) Patient Assistance Program is open for 2023 enrollment and re-enrollment.

NORD has developed a suite of patient financial assistance programs to support the greatest breadth of patient assistance needs within the limitations of program funding.

Individuals who were enrolled in NORD's HPP Assistance Programs should have received via email link or mail, a re-enrollment application. Reapplication is required on an annual basis to determine continued eligibility for assistance. Please check your email and spam filters for the re-enrollment application and submit it to NORD as soon as possible. Applications are processed on a first come, first serve basis and funding is limited.

In order to facilitate re-enrollment, the following information and documentation is needed:

- Name and phone number of the healthcare provider treating the enrolled individual’s rare diagnosis.
- Insurance card(s) - a scanned copy or photo of insurance card(s)
- If assistance with health insurance premiums is requested, please have premium information available.
- If assistance with prescription costs is requested, please have a copay cost for specialty medications (your pharmacy can advise you as to this cost).
- Please give thought to financial assistance needs for the upcoming year so that we may provide the broadest assistance permitted in the assistance program.

Please do not hesitate to reach out to the program if you have any questions.

Phone: **866.828.8902**
Email: **HPP@rarediseases.org**

A Word From Our Partners

A Message From The Assistance Fund about the Hypophosphatasia Financial Assistance Program

My name is Stephanie Marshall, and I am the Director of Patient Advocacy at The Assistance Fund (TAF). TAF staff were glad to see so many warm faces at the 2022 Soft Bones National Patient Meeting in Orlando, Florida, in July 2022. It was a wonderful opportunity to connect with the hypophosphatasia community and share the types of financial assistance TAF offers.

TAF helps patients and families facing high medical out-of-pocket costs by providing financial assistance for copayments, coinsurance, deductibles, and other health-related expenses. Since its founding in 2009, TAF has helped more than 160,000 children and adults in all 50 states, Washington, DC, and Puerto Rico.

Among TAF's nearly 80 disease programs is the Hypophosphatasia Financial Assistance Program. The program provides financial assistance for out-of-pocket costs associated with prescription drug assistance (copays, deductibles, and coinsurance) on FDA-approved treatment, health insurance premiums, therapy administration costs, disease management (such as prescribing physician copayments), treatment-related travel costs, diagnostic laboratory tests, and genetic testing.

To be eligible for TAF assistance, you must be a U.S. citizen or permanent resident, meet certain income requirements, have a diagnosis of the disease named in the disease program, have government or private health insurance, and a prescription for an FDA-approved treatment for hypophosphatasia. Once a patient is enrolled in a disease program, their coverage lasts the entire calendar year and there is no cap on the amount of assistance in that calendar year.

I strongly encourage anyone struggling with out-of-pocket expenses due to hypophosphatasia to check the program status and apply on our website at **https://enroll.tafcares.org/**. Our team of Patient Advocates is also available Monday – Friday from 8 a.m. to 7 p.m. ET to answer questions at **(855) 308-9435.**

Jan Wigington, Director of Operations at The Assistance Fund, and Alexcia Williams, Contact Center Supervisor, exhibiting at the 2022 Soft Bones National Patient Meeting in Orlando, FL.

Stephanie Marshall, Director of Patient Advocacy at The Assistance Fund

A Word From Our Partners

PANTHERx Rare

PANTHERx Rare would like to encourage patients to report any upcoming 2023 insurance changes and that those receiving Foundation assistance will need to re-enroll.

As you place your reorders for the rest of the year, a PANTHERx representative will be asking if your insurance will be the same or changing as of January 1st, 2023.

However, to allow time to process your new insurance, if you do anticipate an insurance change for the New Year and have the new insurance information available, please contact PANTHERx Rare Pharmacy to advise them of this change if you have not done so already. You can reach the PANTHERx Rare Team at 855-726-8479 between the hours of 8 AM to 8 PM EST Monday - Friday or you can email your new insurance information through a secure link to the following email address: **insurancechanges@pantherxrare.com**.

Please be advised, that if you are receiving financial assistance through any of the programs listed below, that you will need to contact that program directly to start the re-enrollment process for the 2023 calendar year:

- NORD - Phone: **800-999-6673**
- The Assistance Fund (TAF) - Phone: **855-845-3663**

Also a reminder, if you have any questions about traveling with Strensiq to please call to speak with a Pharmacist for tips and to coordinate shipments. Our Strensiq number is **844-787-6747**.

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A Word From Our Partners

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