

THE BARE BONES

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

Research Study Results Are In! Impact Of Hypophosphatasia On Behavioral Health And Quality Of Life

Many of you participated in this research project that recruited participants from the International Hypophosphatasia Patient Registry. **Dr. Rene Pierpont** and her research team at the University of Minnesota recently conducted a survey-based study in which they assessed 30 children with a diagnosis of HPP between the ages of 3 to 16, and the effects that pediatric HPP has on neurodevelopmental functioning, mood, behavior, and quality of life.

Half of the children presented severe cases of HPP and previously received enzyme replacement therapy; the other 15 children presented milder symptoms of HPP and received no treatment. After reviewing each participant's medical history, including behavioral questionnaires completed by parents and/or caregivers, it was apparent that 2 out of 3 children with HPP are likely to experience difficulty sleeping, pain hindrance, mood or anxiety interference, or behavioral challenges.



Dr. Rene Pierpont

50% of study participants also met the ADHD criteria based on parent ratings; a rate that is 5 times greater than children in the general population. Sleep disturbances, as well as symptoms of irritability and depression, were also noted to be highly more present in children with HPP than healthy children.

The entire scientific publication of Dr. Pierpont's study in *Orphanet Journal of Rare Diseases* can be accessed via this [link](#).

IN THIS ISSUE:

- Rare Disease Day Recap
- 2021 Virtual Patient Meeting Details
- Fundraising Success Stories
- Teen Advisory Council Launch Update

Delta Dental And Soft Bones Host CE-Accredited HPP Dental Program

Soft Bones and Delta Dental of New Jersey and Connecticut recently offered an accredited online continuing education (CE) course, "Oral Manifestations of Hypophosphatasia (HPP)," to dental professionals in the region. **Amr M. Moursi, DDS, Ph.D.**, Professor and Chair, Department of Pediatric Dentistry, New York University, College of Dentistry, and current President of the American Academy of Pediatric Dentistry, led the course for participating Delta Dental members. This presentation was made possible by Delta Dental's S.M.I.L.E. mini-grant program, of which Soft Bones is a recipient.



This session educated participants on the pathogenesis of HPP, focusing on the dental components, such as early tooth loss. Many times dentists find themselves on the front lines of an HPP diagnosis and can refer their patients to life-changing therapies.

The presentation will be made available online in the near future.

To view additional oral health webinars for patients and caregivers, visit the [Soft Bones website](#).

Recording Now Available: The Impact of Hypophosphatasia on the Kidneys

Watch our recent webinar led by HPP and nephrology experts Dr. John D. Mahon and Dr. Rose Mary Ayooob. This informative session covered the effects of HPP on the kidneys and brought up some important points that can be addressed with your doctor. It also included questions from the attendees on a variety of topics. [Watch now](#) via the Soft Bones YouTube channel.



PRESENTERS:

**John D. Mahan, MD and Rose Mary Ayooob, MD
Pediatric Nephrology Specialists**

Dr. Mahan is professor of pediatrics in the Division of Nephrology and Hypertension at Nationwide Children's Hospital and The Ohio State University College of Medicine, as well as a Founding Member of the Pediatric Nephrology Research Consortium. He is also a General Board and Medical Advisory Board Member of the National Kidney Foundation of OH, KY and TN.



Dr. Ayooob is a physician at the West Virginia University Department of Pediatrics. She was a former fellow at Nationwide Children's Hospital, working alongside Dr. Mahan to treat patients with various diagnoses, including HPP.



Soft Bones Launches HPP TeleECHO Series

Project ECHO (Extension for Community Healthcare Outcomes) is a revolutionary guided-practice model that reduces health disparities in under-served and remote areas of the state, nation, and world. Through innovative telementoring, the ECHO model uses a hub-and-spoke knowledge-sharing approach where expert teams lead virtual clinics, amplifying the capacity for providers to deliver best-in-practice care in their own communities.



Soft Bones is launching its own TeleECHO program, hosting monthly educational sessions to medical professionals via Zoom, offering CME credit to attendees. Attendees will learn about hypophosphatasia directly from the experts. Physicians will also present and study individual cases to assess treatment approaches. Recordings will be posted on the Soft Bones YouTube channel after each of the sessions.

Along with Soft Bones staff, a carefully selected faculty of experts plan and oversee the program and curriculum. Faculty members are Dr. Michael Whyte, Shriners Hospitals for Children, St. Louis, Dr. Deborah Wenkert, Rheumatology Consultant, Dr. Matthew Drake, Mayo Clinic, Rochester, MN, and Dr. Kathryn Dahir, Vanderbilt University Medical Center, Nashville, TN, who each bring a unique specialty to the program. Stay tuned for more information on how your physicians can register for this no-cost program.

Hippo Squad Projects Are a Hit With Members



Not part of the squad?
[Sign up today!](#)

For our February 2021 Hippo Squad activity box, each child received a fun and educational package based on 3 themes: 1. celebrating Rare Disease Day 2021; 2. understanding the "lanyard" concept both emotionally and scientifically; and 3. completing Valentine's Day hippo artwork. Members were able to make their own boondoggle keyrings to visually represent how individuals are "stronger together" when they come together for a common purpose (like their squad!). The project also resembled a strand of DNA, introducing this concept to children and parents through a fact sheet. Members also shared HPP factoid postcards with their peers for Rare Disease Day and completed Valentine's Day hippo coloring sheets. The next box will be delivered to registrants in time for the Soft Bones 2021 Virtual Meeting.

"Express Feedback For Good" Program Surpasses Fundraising Goal!

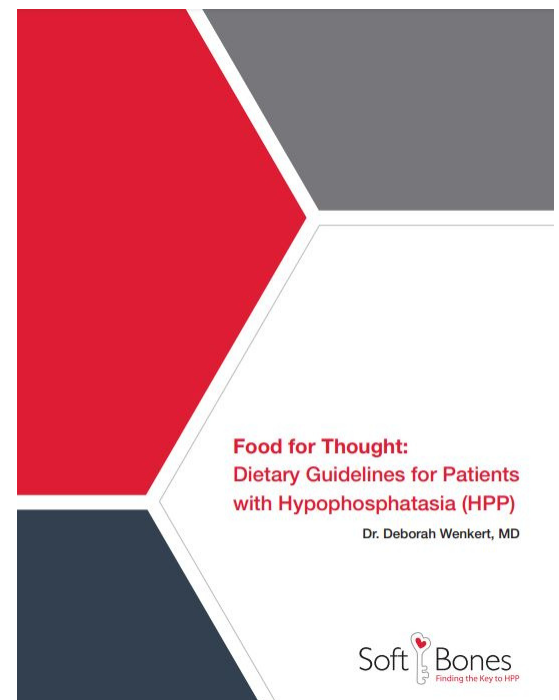


In recognition of Rare Disease Day 2021, Soft Bones was able to participate in the **Express Feedback For Good Program** through fundraising partner Hundred X, Inc. 250 community supporters completed 30-second consumer feedback forms about popular national retailers, online services, and corporate chains, earning \$2 per form for our organization. Nearly 20% of participants submitted 75 forms, the program maximum, and received a Soft Bones swag item of their choice. The program ran from February 8 to March 9. THANK YOU from all of us here at Soft Bones for helping us make our goal.

Newly Released Soft Bones Publication

After years of requests and through collaboration between multiple institutions and HPP experts, we are excited to provide you with this new resource, **Food for Thought: Dietary Guidelines for Patients with Hypophosphatasia (HPP)**. This interactive document provides sources for dietary guidelines and provides references for why certain recommendations are made.

It is important to note that this is a living document and will likely change as we learn more about HPP. There are many theories and other approaches that are untested, so it is important to differentiate what is scientifically validated vs. unsubstantiated dietary recommendations. Of course, always talk to your doctor about the best approach for you in managing your HPP symptoms. [Read the publication here.](#)



Join Us For The 2021 Virtual Patient Meeting



2021 **VIRTUAL** PATIENT MEETING



Dr. Eric Rush



Dr. Chris Sobey



Dr. Maike Dohrn



Dr. Rene Pierpont

Join Soft Bones and the entire patient community for our largest meeting of the year! Hear from leading HPP experts **Dr. Eric Rush** of Children's Mercy, Kansas City, MO, **Dr. Christopher Sobey** of Vanderbilt University Medical Center, Nashville, TN, **Dr. Maike Dohrn** of RWTH Aachen University and University of Miami, FL, and **Dr. Rene Pierpont** of the University of Minnesota Medical School, Minneapolis, MN while learning about genetics, pain management, neuropathy, and more. There will be time for Q&A, networking, and chatting amongst members of the community- old and new, as well as sponsors.

A pre-event Meet and Greet Vision Board Workshop will also be held, and families with children ages 3-12 can reserve a Soft Bones Hippo Squad Family Activity Box to keep the kids entertained on meeting day. Whether you are newly diagnosed, seeking a diagnosis, or highly educated in HPP, this meeting is for you!

REGISTER TODAY

Headline Sponsor:



Bronze Sponsor:



Exhibitor Sponsor:



Rare Disease Day 2021 Roundup

Soft Bones is proud to support NORD and EURORDIS in this annual international campaign to raise awareness amongst the general public and decision-makers about rare diseases and their impact on patients' lives and the healthcare system. Here is a look at the activities and events sponsored by Soft Bones this past February, culminating on February 28th, Rare Disease Day.

Soft Bones Unveils New HPP Awareness Ribbon



Traditionally, HPP has been associated with the bone disease ribbon, which is plain white. However, there was a patient who said she wished there was a ribbon that reflected the true nature of HPP, which goes beyond bone. In reality, HPP impacts many different parts of the body and can range from mild to severe. Soft Bones hired a graphic designer who was up for the challenge and looked to develop a ribbon that showcased the nature of HPP, varied, complex, and different. The ribbon was launched to the HPP community in February, just in time for Rare Disease Day this year. Each color or pattern of the ribbon represents a symptom or complication of the disease. The keys below show how different colors and patterns correlate with various aspects of HPP.

Join the conversation on the [HPP AND ME discussion board](#) and let us know what you think! Stay tuned for new apparel and swag featuring the new design at the Soft Bones store on our website.



Kidney

Skull
Neuropathy

Bone



Trach



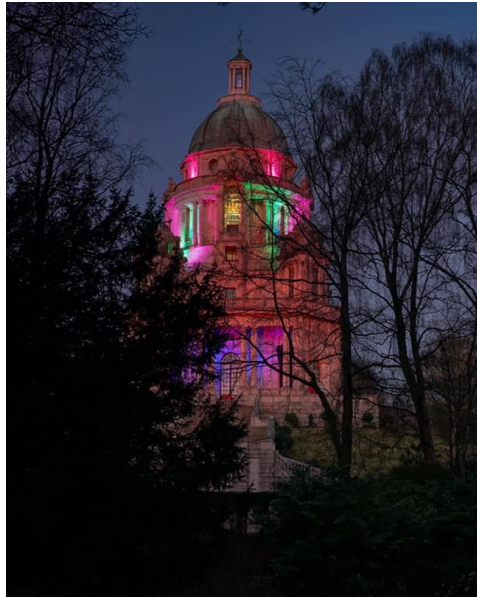
Dental Disease



Rare Disease

Rare Disease Day 2021 (continued)

Towers Lit Throughout The World



Ashton Memorial, Williamson Park, Lancaster, UK lit for Rare Disease Day in Lancaster, UK. Photo Credit: Ian Green

Storyvine Video Creation

More than 30 video submissions were recorded using the Storyvine app, allowing patients and caregivers to tell their stories and easily share them via social media to spread awareness. To view each patient and caregiver journey, visit our [YouTube channel](#). Pictured below- Blynda K.



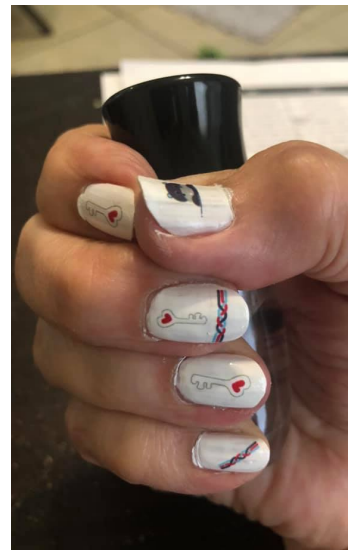
Showing Those Zebra Stripes

Lovely Natalie H., age 2, showing her Zebra stripes this February.

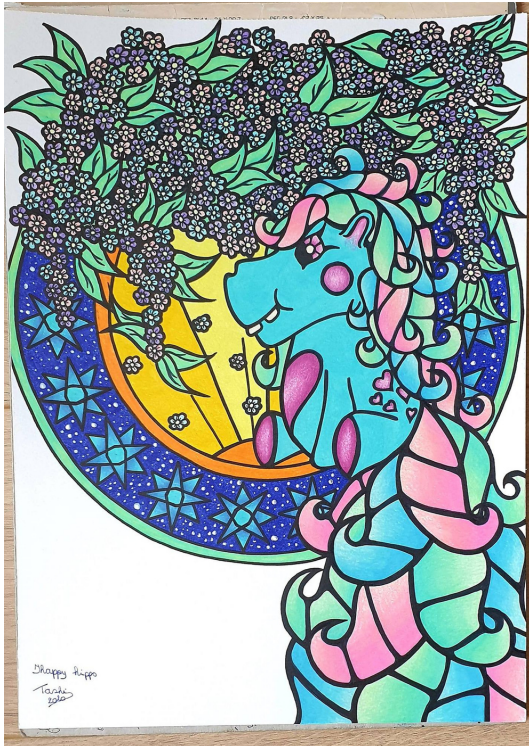


Members Get Creative To Spread Awareness

Community member Francene S. shows her Soft Bones pride by purchasing logo nail decals, pictured here.



Rare Disease Day 2021 (continued)



Soft Bones' very own hippo mascot depicting a current mood, by community member Nele M. Truly a work of art!



Social Media Brings Unique Opportunities To Spread Awareness

HPP community members posted and shared educational facts and awareness messages all month long using the hashtags #HPPTogether, #SoftBones, #RareDiseaseDay2021, #RareDiseaseDay, and #hypophosphatasia. Many shared photos of themselves, their artwork, or their own outreach activities to spread awareness about HPP and RDD (shown here).

Mandy J. and her family placed these beautiful hand-painted, rare disease-themed rocks around their community in Washington state to raise awareness about rare.



THANK YOU TO ALL WHO PARTICIPATED!

Record High: Photo Frames For Rare Disease Day

More than 200 people changed their Facebook profile picture to include the official Rare Disease Day frame, which is a record high for Soft Bones, topping our recent record set on World HPP Day 2020.



Rare Disease Day 2021
Hypophosphatasia Awareness

Applications Now Being Accepted For The Teen Advisory Council (TAC)



What is the TAC?

A support and advocacy group for teen patients (or teenage siblings of patients) who have been diagnosed with hypophosphatasia. Apply now to be part of the team!



Make It YOUR Platform

TAC provides a platform for teens to speak up about HPP to the broader community. Members will be trained to engage with local politicians to garner federal support, fundraise and spread awareness within their schools and communities.



Get Involved

From writing blog posts to participating in social media challenges, there are numerous ways for you to get involved. By joining the team, you can compete in challenges to **win prizes**, be selected for **awards**, or even **learn about scholarship opportunities!**



Council Management

As part of the team you will have the opportunity to run SB's TAC social media accounts, participate in content challenges on YouTube, TikTok, and Instagram, organize fundraising events such as bake-sales, walks, and raffles, and much more!

SUBMIT YOUR APPLICATION TODAY

Meet Our TAC Coordinators

We are excited to introduce our new Teen Advisory Council coordinators, Julia O'Leary and Alexandra Hearn! As TAC coordinators, they will engage with the teens to empower them to speak about HPP and work with the group to create fun ways to educate their friends and the general public. The participants will be encouraged to bring new ideas to the table to enrich the program and will have opportunities to develop their leadership skills.

Julia O'Leary



Julia has a passion for storytelling on a local, national, and international level. She fosters that love through her classes at Syracuse University as a writing and journalism student. In her free time, Julia is an avid traveler, skier, seafood connoisseur, and thrill seeker. She is a passionate advocate and has worked with the Juvenile Diabetes Research Foundation since her type 1 diabetes diagnosis in 2008.

Julia has years of experience from attending the JDRF Children's Congress, founding the Central PA Teen Task Force, to living with a chronic illness for more than half of her life. She looks forward to connecting with teens through the TAC and using her skills to help Soft Bones succeed. Her future plans include traveling the world and completing her never-ending bucket list.

Alexandra is studying Public Health and International Relations at Syracuse University. She has a focus and passion for community health education.

Alexandra has experience working with youth as a peer tutor and a volleyball coach. In Summer, 2019, she studied in South Africa and led and facilitated a conflict resolution project partnering with high school students. Alexandra has many years of volunteer experience, and has worked with multiple nonprofits including, Girls on the Run, American Cancer Society, JDRF and Midway Shelter. She is thrilled to work with the TAC members and help pilot this important initiative.

Alexandra Hearn



Connect with Julia or Alexandra, or inquire about the program via email, at tac@softbones.org.

Looking for a P.A.L.?

Look No Further!
The Soft Bones HPP P.A.L. (Partner and Learn) Program has got you covered!

If You Are:

- Newly Diagnosed
- New To Injection Therapy
- An adult or teen living with HPP
- A parent or guardian of a child who has HPP
- A caregiver, spouse or sibling



Be matched with a person on a similar journey for peer support and encouragement through the daily management of hypophosphatasia.

Contact denise@softbones.org for more information and to get started.

Self-Care Corner

HPP is a journey of ups and downs. It's important that we should pay special attention to our bodies, especially when one may be dealing with a chronic disease such as HPP.



Self-care is vital during those rough days, but the following practices can help alleviate the pain and help you get in tune with your body:

- Practice gratitude for your body - through self-mantras, or journaling
- Keep it simple - making yourself a cup of tea or enjoying a bath can be just enough to ease you
- Allow yourself to actually feel your emotions - if you want to cry, cry it out; acknowledge the bad day and know that better days lie ahead

For more tips on how to practice self-care [click this link.](#)

Welcome To Our New Region Lead: Midwest 1

Haley Gast



In Haley's own words: "My name is Haley Gast. I'm originally from the Hoosier state and had the pleasure of growing up in the rural countryside near Lake Michigan. I eventually found myself in central Indiana (Indianapolis) where I now work, study, and live. I coordinate the Soft Bones Midwest 1 Region that includes Indiana, Illinois, Iowa, Minnesota, and Wisconsin.

HPP has been a major part of my life. I was diagnosed with the mild/severe childhood-onset of HPP at six months of age and have experienced/lived with many of the symptoms that come along with this rare condition. I experienced delayed milestones, early tooth loss, hypermobility, craniosynostosis, failure to thrive, delayed motor skills, short stature, and a broken collarbone at birth. Growing up with HPP also meant understanding my limitations, but did not keep me from doing hobbies or sports. I have seen many specialists over my lifetime, some who are no longer with us and some who are brand new to the world of HPP. Learning how to juggle work, school, and HPP disease management has been a challenge, but rewarding. And while there was no existence of formal treatment, I often had to rely on at-home remedies to ease my symptoms. It wasn't until my 30th birthday that I started formal treatment. Now I'm currently studying to complete my Master of Public Health, in hopes to help give back to the community and field in which has enriched my journey of living with a rare disease. HPP has shaped me to look at health and life in general from a different perspective and appreciate the growth of knowledge we discover as a disease community.

Having a connection with others in the HPP world has been so vital to my understanding of other experiences and journeys of fellow patients and caregivers. Personally, I had never formally met anyone else with HPP besides my sister, until I attended a Soft Bones meeting in Chicago at the age of 25. It was an experience of a lifetime, and I feel that every HPP patient should have that experience. As the Midwest 1 Region Lead, I hope to create a space for our HPP family to share ideas, have important discussions, and ask each other questions about HPP-related experiences. I also hope to partner with nearby regions and share patient education opportunities. I'm looking forward to connecting to more HPP patients and families in my region."

If you live in the Midwest 1 Region and have not yet reached out to Haley to welcome her, or learn more about activities and connections within the region, please contact her at haley@softbones.org.