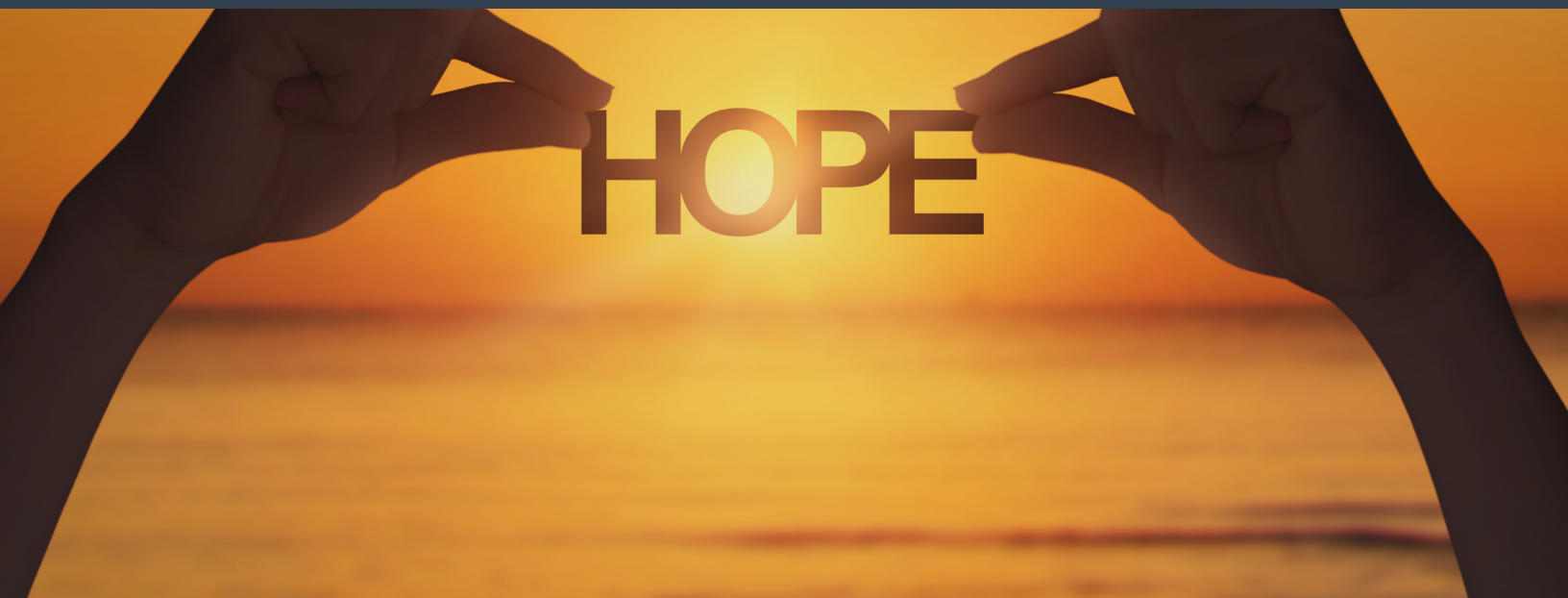


THE BARE BONES

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



IN THIS ISSUE:

- COVID-19 Updates
- Book launch information
- Meeting updates
- Regional roundups
- Save the Dates

COVID-19 Updates

Unprecedented, Crazy, Uncertain. These are words to describe the current environment we live in with the coronavirus. As a non-profit rare disease patient advocacy organization, Soft Bones has been greatly impacted financially by these difficult times. Staff have been working to help support patients through these trying times, while trying to continue to deliver on our mission and programs.

According to the Chair of the Soft Bones Scientific Advisory Board, hypophosphatasia patients are not believed to be at an increased risk of getting a COVID-19 infection because of low alkaline phosphate. However, increased risk of pneumonia that comes with COVID-19 could put some patients at a risk if there is a chest muscle weakness or deformity.

It is recommended that everyone follow the CDC guidelines, and we will do our very best to keep you informed with updates related to the rare disease community. If you have questions or need support during this time, please contact us at (866) 827-9937 or info@softbones.org. We are here to help.





Important Things To Remember During This Time

During this temporary yet new "normal" state of affairs where packages and deliveries may be arriving at your home more frequently than usual, please remember, your health comes first. A sign like the one pictured here should be placed on your front door to alert all visitors about the importance of social distancing for your household. If you are expecting a package that requires a signature, you can also leave a signed note on your door to grant permission for them to leave your delivery without your physical consent. If you would like a printer-friendly copy of the sign pictured here, please contact bonnie@softbones.org.

Reminder:

For the safety of all parties involved, HPP baby teeth are not being accepted for research purposes until further notice. Our foundation receives the latest updates and information on topics such as this, and will inform everyone immediately if there is a change to this protocol.

A sign template with a red border. At the top left is the logo for Soft Bones, featuring a red heart and the text "Soft Bones Finding the Key to HPP". In the center is a red octagonal stop sign with the word "STOP" in white. Below the sign is the text "A PERSON IN THIS HOUSE HAS COMPLEX HEALTH NEEDS". Underneath that is a paragraph: "People with complex health needs are more likely to catch the coronavirus (COVID-19) and suffer life-threatening complications if they become ill." Below that is another paragraph: "Because of this, we are self-isolating and/or practicing social distancing as a preventative measure." At the bottom is a red button with the text "PLEASE LEAVE DELIVERIES".

Soft Bones
Finding the Key to HPP

STOP

A PERSON IN THIS HOUSE HAS COMPLEX HEALTH NEEDS

People with complex health needs are more likely to catch the coronavirus (COVID-19) and suffer life-threatening complications if they become ill.

Because of this, we are self-isolating and/or practicing social distancing as a preventative measure.

If you feel at all unwell or we don't answer the door,

PLEASE LEAVE DELIVERIES

Rare disease organizations such as the National Organization for Rare Disorders (NORD) are offering valuable programs and resources to support patients, families and caregivers:

- **NORD's COVID-19 Resource Center** provides a wealth of information for those living with a rare disorder such as HPP; it can be accessed online at [this link](#)
- **NORD launched its COVID-19 Critical Relief Program** to provide financial relief for members of the rare community affected by the pandemic. Funds may be utilized to support critical, non-medical needs to eligible patients, covering up to \$1,000 annually for essential expenses including, but not limited to unexpected utility expenses; cellular or internet service; emergency repairs to car, home or major appliances; and rent or mortgage payment assistance. For more information call 203.242.0497 or visit their website (listed above).
- Stay informed! **To receive the latest COVID-19 updates and information from Soft Bones, Inc.**, including news from partner organizations and pharmaceutical companies such as Alexion and Panther, please connect with us via Facebook and join our email list. Please contact bonnie@softbones.org to sign up or update your contact information if it has changed and you are not currently receiving updates.



NORD[®]
National Organization
for Rare Disorders

Our Letter to Congress: Potential Aid for Patient and Community -Based Organizations Impacted COVID-19

Written by Natalia Castelan

Amidst these unprecedented times, Soft Bones, as well as 212 other organizations, wrote a letter to congress regarding prospective federal intervention to support nonprofit patient advocacy community-based organizations within any federal aid packages or supplemental appropriations measures, as many have been and will be harmed by reduced charitable giving, travel restrictions, fund-raising event cancellations and reduced meeting attendance.



Nonprofit advocacy community-based organizations are vulnerable during these trying times, but we hope to continue to provide the needed support to our Soft Bones community members despite the given situation. We will continue to be your advocates and be rest assured we are going to get through this pandemic together.

Soft Bones' HPP Children's Book, *Journaling My Hypophosphatasia*, is Finally Here!

Written by Natalia Castelan

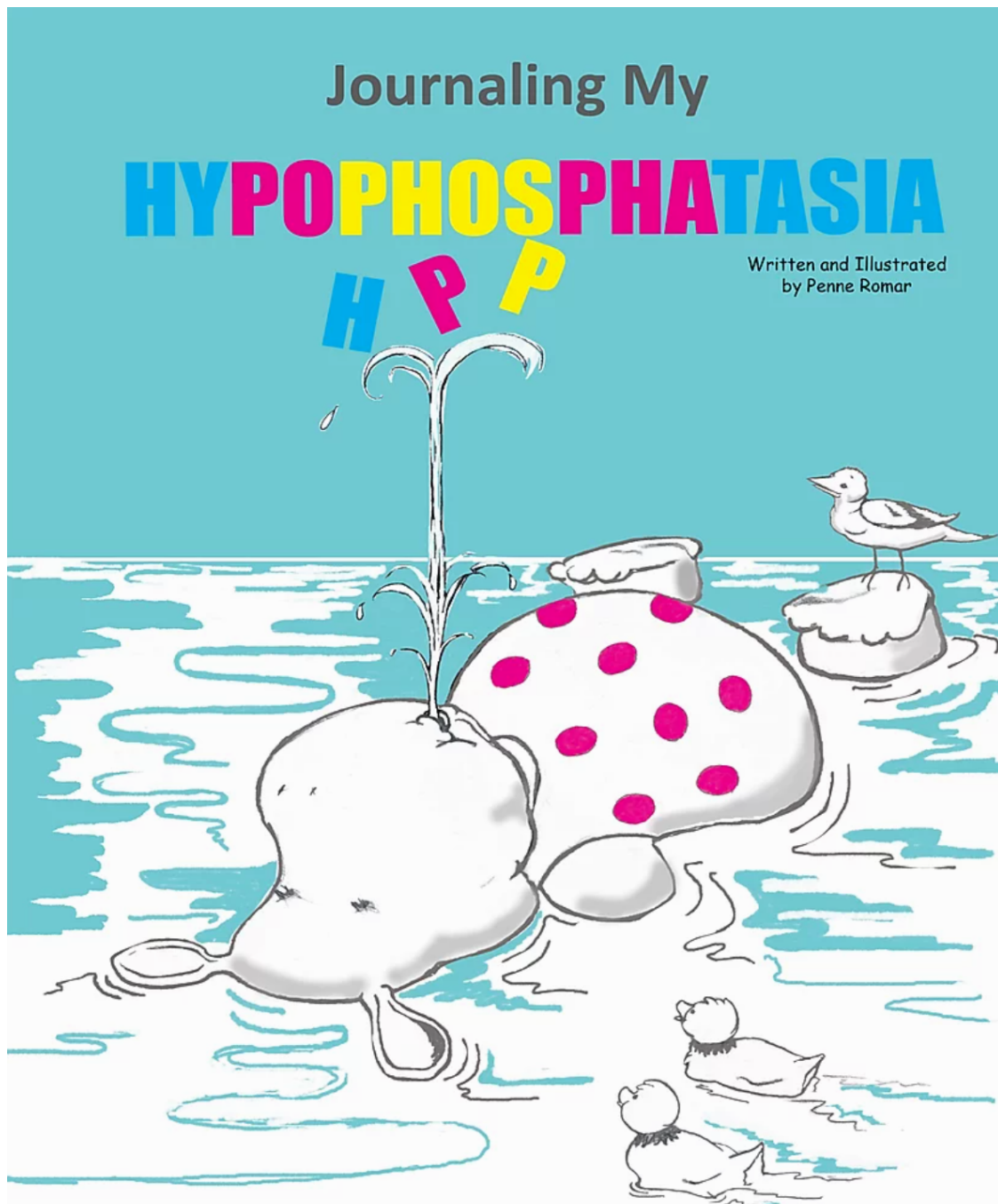
We are excited to announce that Soft Bones new children's book, *Journaling My Hypophosphatasia* has recently launched! Soft Bones teamed up with author Penne Wittner to develop *Journaling My Hypophosphatasia*, which provides an inclusive and celebratory outlet for children that draws connections to their own HPP journey. The launch featured Facebook Live readings by Soft Bones community members David Talkington and Kirsten Mitchell, which can be viewed on our [Facebook page](#).

Congratulations



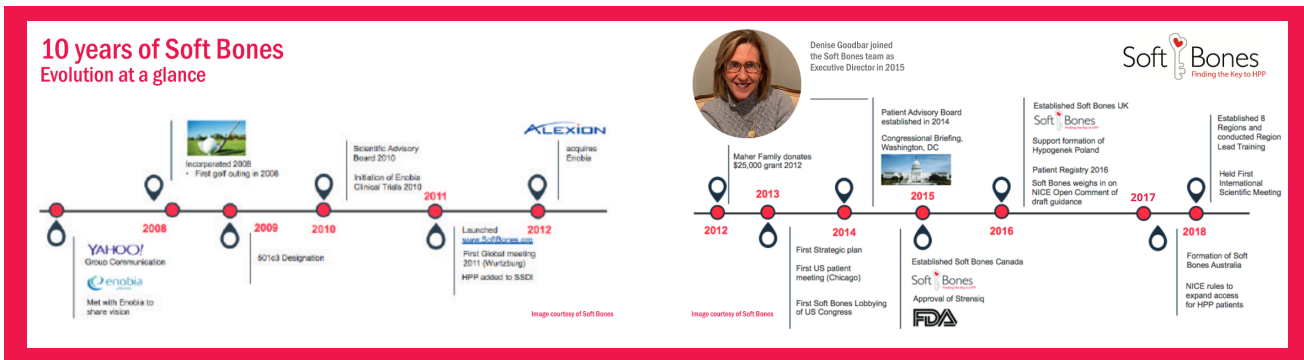
Remember to sign up to receive your free copy if you have a child with HPP - 1 free book per household. Upon signing up for your free copy, there will be an option for you to sign your child up to be part of the Soft Bones Hippo Squad, a free support and educational program for children with HPP!

Pictured below: Soft Bones' community members Kirsten Mitchell and David Talkington



Soft Bones in the News

Soft Bones was featured in the latest edition of Rare Revolution Magazine. This article commemorates 10+ years of service to the HPP community. We are grateful for the coverage and appreciate the time and effort that went into documenting our achievements over the past decade.



Soft Bones Joins Institute of Gene Therapies (IGT)

Written by Natalia Castelan

Earlier this year the newly established Institute of Gene Therapies (IGT) launched with an aim for the modernization of the U.S. regulatory and reimbursement framework, to ensure gene therapies can be accessed by the patients that need them. The main objective of IGT is to educate stakeholders across the healthcare community about gene therapies and support policies that help patients in need can benefit from treatment. Soft Bones, Inc., has been invited to sit on the IGT's Advisory Board and will support IGT's mission to incorporate gene therapy as a means to improve the lives of patients.

Joining the IGT will enhance the work that Soft Bones is conducting on the front lines of gene therapy research. Members of the IGT Advisory Board will collaborate to

share their successes and challenges in the research and development of these therapies and will work together to share specific disease-related issues to fortify the Institute's approach. Gene therapy has great promise and is predicted to make a positive impact in the health community by managing symptoms at the root, not just managing them over time. It works by altering non-functioning genes or replacing absent ones. These therapies can reshape the way many diseases are treated. Gene therapy targets the cause of the disease at the DNA level, creating a shift in the body. Some of these gene therapies are even capable of being one-time treatments that can have life-long benefits. The U.S. Food and Drug Administration (FDA) has approved the first transformative therapies and hundreds more are presently being studied on many types of diseases, both common and rare, as well as cancer, and more.

As part of IGT's effort, experts from across the healthcare system will work together to ensure health policies reflect the latest medical advances, remove barriers that hinder patient access to gene therapies and advocate for sustainable, long-term solutions. IGT will work to ensure a greater understanding about the value gene therapies bring to patients, families, the healthcare system and our society so that gene therapies can achieve their full potential.

For the full press release, visit this [link](#).



Recent Dental Webinar Keeps Our Community Informed

Written by Bonnie Starr

On April 12, leading dental expert Dr. Timothy Wright of UNC Chapel Hill School of Dentistry (pictured below) led a webinar entitled “Hypophosphatasia: Oral Manifestations and Management.” This webinar provided a well-rounded overview of care and considerations for children and adults with HPP, including tips for preventing oral complications and questions to ask one’s own dental provider. The audience drew a record number of 170 attendees, consisting of both patients and dental professionals.

To view the webinar in its entirety, visit our YouTube channel [SoftBonesHPP](#). You can also browse our library of online resources about oral care and many other topics of relevance to those living with HPP and a dental fact sheet at our [web-page](#). Dr. Wright will offer another opportunity to learn about this topic during his presentation at the 2020 Virtual Patient Meeting on July 11. Be sure to [sign up](#) for the meeting today if you haven't done so already!



"Ask the Expert"



Written by Emily Quinones

Question: “What treatment option should be expected when trying to compensate for or correct a skeletal irregularity?”

Answer: “Conservative options including physiotherapy and braces should be considered prior to surgery. Particularly with younger patients, medical treatment to compensate for metabolic deficits may also help to correct skeletal irregularities during growth. Furthermore, despite these treatment considerations, the risk of fracture progression is high. Therefore, in many cases, surgery is advisable to regain mobility and prevent complete fractures or potential deformities.” - **Lothar Seefried, M.D., Head of the Clinical Trial Unit, The Orthopaedic Institute University of Wuerzburg, Germany and Soft Bones, Inc. Scientific Advisory Board Member. Dr Seefried's full bio can be found [here](#).**

For more information on orthopedic treatment for patients with hypophosphatasia, check out the newly created Orthopedic Overview Fact Sheet authored by Dr. Lothar Seefried that can be on the SoftBones.org website. For the full press release, visit this [link](#).

Region Meeting Roundup

Written by Alex Brito

SOUTHWEST

On February 29, 2020, Southwest Region Leads Sue Krug and Amy Britt held an HPP Patient Meeting in Phoenix at Phoenix Children’s Hospital for Rare Disease Day. There were 25 attendees, including Dr. David F. Carpentieri, Section Chief of Clinical Chemistry, Therapeutic Drug Monitoring, Point of Care Testing and Urinalysis.

Many presentations were made to educate the attendees, including an overview by Dr. Smith on the types and severity of HPP. Dr. Wasden, DDS explained why children with HPP often lose their teeth and offered information about how to protect the teeth of HPP patients. Soft Bones’ Northwest Region Lead, Ann Haak, also attended to teach newly diagnosed families about HPP.

At the end of the meeting, attendees were given copies of the State Proclamations which were, HPP Awareness Day and Rare Disease Day 2020.

SOUTHEAST

The Soft Bones Southeast Region held a meeting on February 22, 2020, in Nashville Tennessee near Vanderbilt University. The gathering, hosted by Region Leads Blynda Kellner and Kirsten Mitchell, offered the opportunity for members to meet, connect, socialize, and hear the latest information and research on hypophosphatasia (HPP) from highly respected experts in the field.

The meeting began with introductions and a discussion of the purpose and mission of Soft Bones, Inc. OneSource Case Manager, Sarah Hanson, provided a presentation on the role of Alexion and how nurse case managers are able to support patients with HPP. Renowned experts Dr. Kathryn Dahir and Dr. Jill Simmons provided an in-depth look at how hypophosphatasia presents itself, how it is diagnosed, the genetic role, and how it is presently being treated, including a look at some newer treatments being studied.

Deb and Denise's Trip to Washington, D.C. for Rare Disease Day

Written by Alex Brito

On February 28, Soft Bones Founder Deborah Fowler and Executive Director Denise Goodbar participated in the National Institutes of Health (NIH) Rare Disease Day activities in Washington, D.C. Rare Disease Day typically takes place in late February to raise awareness among the public and policymakers about rare diseases and their impact on patients' lives. The NIH's goals are to commit and support those with rare diseases through research, developing diagnostics and treatments and sharing stories of patients, families, and communities on Rare Disease Day.



Deb and Denise met with fellow members of the Rare Bone Alliance, as well as other partners whom they worked with from NORD, CoRDS, Global Genes, and RDLA. The team also spoke with NORD and RDLA to discuss ways to partner on policy issues and SB's involvement in the ELSA Legislation (Ensuring Lasting Smiles Act). They also spoke with CoRDS, who is the keeper of the patient registry, on how to continue to help patients understand the value of the patient registry.

Since the meeting occurred during the early stages of COVID-19, the team had to adjust to uncommon gestures, such as bumping elbows instead of greeting with handshakes. Deb also mentions that the beginning topic of discussion was about COVID-19 and how it impacts the rare disease community. Deb reports that, “this meeting is always valuable as we rekindle relationships and also spark new ways to collaborate by working with partners.”

To learn more about Rare Disease Day and how you can participate, visit the website www.rarediseaseday.org.

2020 National Patient Meeting UPDATE



2020 **VIRTUAL** PATIENT MEETING

**JULY 11,
2020**

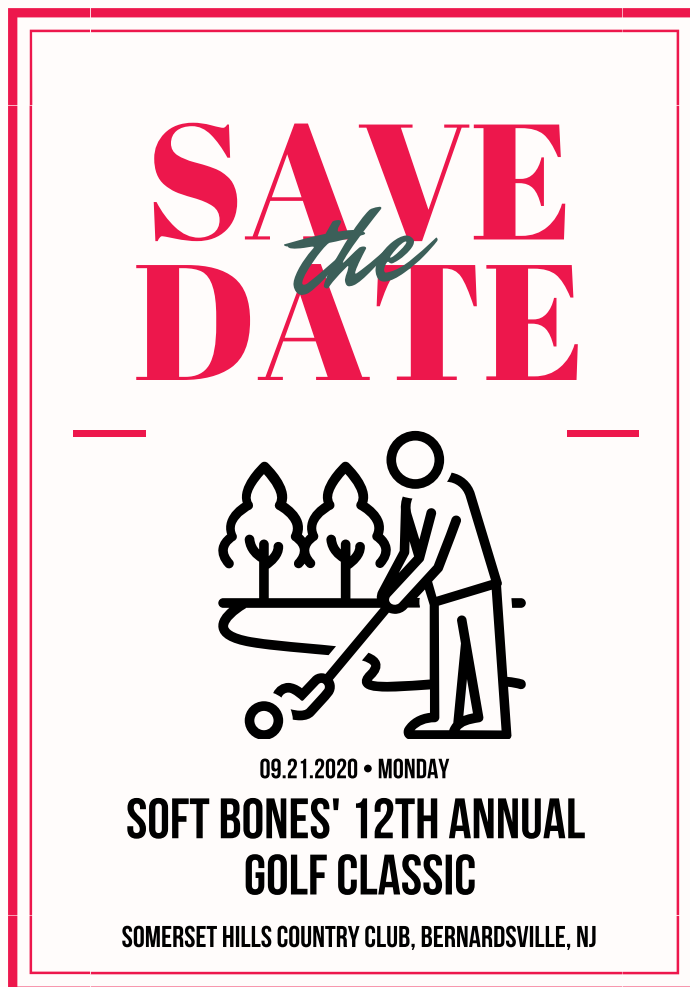
**REGISTER
TODAY!**

Due to the continued uncertainty around the period of change and the health and safety of the Soft Bones community, our in-person meeting at Duke University (originally scheduled for the same day) has been cancelled and will be rescheduled for next summer. We are disappointed as we all look forward to getting together, but after discussions with other advocacy groups and Duke staff, it was the consensus that this was the right decision as to not inconvenience families who are planning for travel. The Soft Bones team is focused on delivering creative educational programming that is sure to capture everyone's attention through their computer or mobile device. You can register via this [link](#). A detailed agenda will be available via Facebook and the Soft Bones' website as they become available.

Show your Support Using Amazon Smile

Are you making a lot of online purchases these days through Amazon? If you have, we've got some exciting news. **AmazonSmile**, Amazon's free community giving program, is now available on both your computer AND your iOS or Android mobile device! By linking your Amazon account to AmazonSmile, 0.5% of sales from your eligible purchases will be donated back to Soft Bones. Registration is fast, and totally free! No extra donations required, and no extra effort involved aside from shopping using Amazon's linked Smile website. This is an ongoing program, so as long as you keep shopping using the Smile link, all of your current and future purchases will count. To participate via the Amazon Shopping app, select the 'Settings' option once the app is open and follow the on-screen instructions to complete the process. We thank you all for your contributions!

SOFT BONES' 12TH ANNUAL GOLFING OUTING



Join us once again for Soft Bones' beloved fundraising event supporting programming and research efforts for hypophosphatasia!

Join us September 21, 2020 at the Somerset Hills Country Club in Bernardville, NJ for a day full of fun, love, and laughter.

We welcome all support for the event, including donations and sponsorships, auction items, and volunteer assistance during the event. Contact Bonnie Starr at bonnie@softbones.org for more information.



TEEN ADVISORY COUNCIL

JOIN OUR EMPOWERING COMMUNITY

A support and advocacy group for teen patients (or teenage siblings of patients) who have been diagnosed with Hypophosphatasia. Apply now to be one of 12 members selected 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 can directly reach out to local politicians to garner federal support, or 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 **win a scholarship!**



Council Management

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

SUBMIT YOUR APPLICATION

We look forward to reading your applications! Apply now at www.softbonestac.wixsite.com/website/apply.