THE BARE BONES

A quarterly publication from Soft Bones - The U.S. Hypophosphatasia Foundation

SUMMER 2026

ISSUE 27

What a stronger community makes visible.

GATHERING CREATES SHARED EXPERIENCES.

EXPRESSION DEEPENS UNDERSTANDING.

STAYING CONNECTED STRENGTHENS WHAT COMES NEXT.

ALSO INSIDE:
WORLD HPP DAY • PATIENT VOICES
• RARE ART • HPP NEWS & MORE

Soft Bones
Finding the Key to HPP

NATIONAL PATIENT MEETING

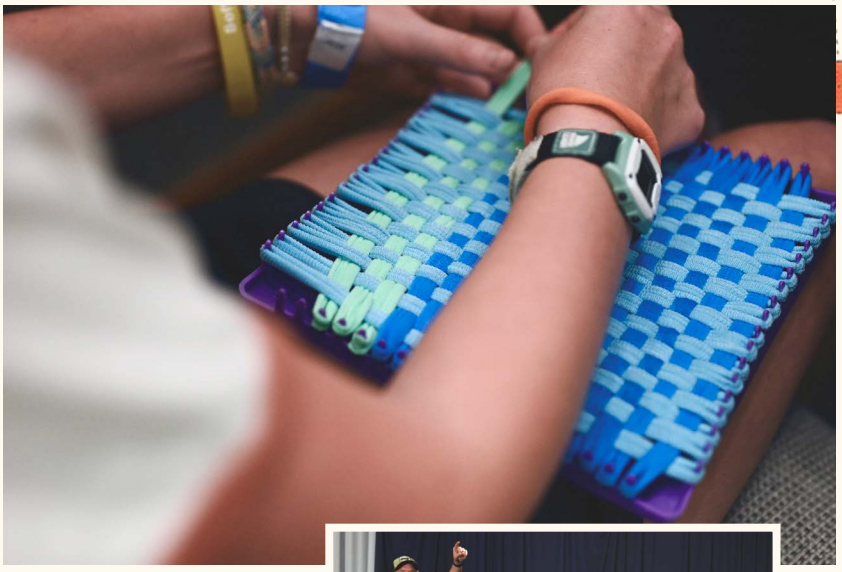
What comes into view when a community gathers.

Staying close keeps every voice within reach of the work ahead.

This summer, the HPP community came together to learn, create, listen, and share. At the National Patient Meeting, lived experience moved through conversation, visual art, scientific discussion, and music – offering new ways to understand what it means to live with and alongside HPP. These moments also underscored why staying connected matters: each voice adds to what Soft Bones can see, represent, and carry forward.

These moments would not have been possible without the support of Alexion, Alesta, PANTHERx Rare, and PuREC. We are deeply grateful for their continued support.

Three Ways Connection Took Shape.



01

GATHERING

The National Patient Meeting brought lived experience, clinical knowledge and community into the same room.

The morning session gathered doctors, dentists, advocates, caregivers and patients together to discuss topics surrounding HPP.

02

EXPRESSION

Dr. Dennis Deruelle, a Nashville-based country music songwriter, led a musical expression session in which participants worked with musicians to create a song about living with HPP. The completed song was performed at the end of the session. The collaborative music session gave words and feeling through song to experiences that are not always easy to describe.

In the teen and children rooms, the groups were guided in art projects.

03

STAYING CONNECTED

Every person who remains close to Soft Bones strengthens the community the organization can represent.

NATIONAL PATIENT MEETING

What We Carry Forward

"I can honestly say this was one of the best meetings I've attended. It was wonderful to reconnect with old friends, make new ones, and spend time with people who truly understand life with HPP."

— National Patient Meeting attendee

For our Soft Bones community, gathering is not simply about being in the same room. It is about recognizing something familiar in another person's story and realizing that an experience once carried alone is understood by someone else. That is why our National Patient Meeting is so special for us here at Soft Bones. We want to keep our community connected.

This year in Chattanooga, over two hundred patients and caregivers came together with different histories, questions, and perspectives. Some arrived with years of experience navigating HPP. Others were newly diagnosed and beginning to make the connections between their past health experiences and HPP. Around lunch tables, during sessions, and in the coffee conversations between them, people exchanged the kind of knowledge that is difficult to find in a handbook: how HPP enters daily decisions, changes over time, and shapes the lives of entire families. What felt especially wonderful was the connections that extended well beyond the scheduled session hours. Laughter could be heard through the lobby and hotel hallways long after the meeting rooms had emptied. Conversations start within the Soft Bones spaces, but connection carries on after hours, and we couldn't be more pleased.

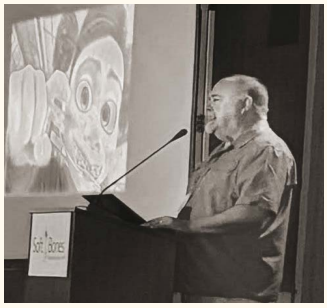
Soft Bones may create the event, but the relationships formed there belong to the community. We hope that what begins in one room travels home with every person who was there, eventually reaching those who were not.

"The experience of having HPP can be very alienating, so bringing us all together fostered a sense of collective validation and empowerment."

-National Patient Meeting attendee

Listening in Both Directions

The National Patient Meeting brings patients and caregivers together with clinicians, researchers, and other experts who are helping to deepen what is known about HPP. But the exchange is not simply a matter of professionals speaking and patients listening. Again and again, presenters acknowledged that they also learn from the people living with the condition every day.



From upper left clockwise: Dr. J. Carl Pallais, Dr. Donna Griffin, Dr. Sun Peck, and Dr. Timothy Wright.

What We Heard. A Panel of Experts. A Shared Commitment to patients.

NPM Panel Discussion

Dr. J. Carl Pallais, MD, MPH, spoke about his HPP research, including the importance of understanding ALPL variants and their associated phenotypes. He acknowledged how much he continues to learn from patients and discussed the value of tools such as the Soft Bones apps for tracking symptoms and contributing patient-reported information.

Dr. Timothy Wright, MS, DDS and Soft Bones Scientific Advisory Board Member discussed hypophosphatasia and the developmental defects it can cause in teeth. He emphasized the importance of establishing care with a dentist by age one and spoke about preventive measures such as fluoride, varnishes, and sealants. He also shared information about a clinical trial exploring the use of clear aligners to create gentler, more controlled tooth movement.

Dr. Donna Griffin, a Board-Certified Clinical Specialist in Pediatric Physical Therapy, spoke about caring for people with complex orthopedic, neurological, and metabolic bone conditions. She shared practical ideas for supporting safe movement and play, including the use of soft items such as pool noodles. She also spoke about the importance of consistency, from helping children wear orthotic leg braces regularly to keeping a journal that tracks symptoms and concerns without becoming overwhelming. "*Journaling without going overboard is helpful to us*," she explained.

Later, while discussing the importance of finding the right medical team, Dr. Griffin reminded attendees, "*You need someone who listens.*" The clinicians caring for people with HPP must also be willing to learn from the patients and families who understand how the condition affects their lives each day.

Sun Peck, PhD, Assistant Professor at Vanderbilt University, spoke in the afternoon session about the extracellular matrix and the role of collagen in bone. She explained her research into the molecular mechanisms of musculoskeletal disease and its goal of helping to inform innovative therapies for rare and degenerative bone conditions.

Additional to the doctors and researchers, we were so pleased to have Katerina Steele from Alexion and Elizabeth Reynolds, PhD, from Alesta. They spoke to their companies' commitments to the patients and the research towards HPP. Each presentation offered a different window into HPP. Together, they demonstrated why progress depends upon bringing scientific knowledge and lived experience into the same room.

Patients and families leave with information they can carry into their own care. The professionals leave with a deeper understanding of the lives behind the research. What each group brings helps move the conversation about HPP forward.

To view full sessions presented at the 2026 National Patient Meeting, visit our on-demand video library here.

NATIONAL PATIENT MEETING

A Different Way to be Heard

From Conversation to Song

"I wish I could take your pain away."

The words came from a husband sitting with his group at the National Patient Meeting. He was talking about the person he loves and the part of HPP he cannot fix for her. Before long, that sentence became a lyric.

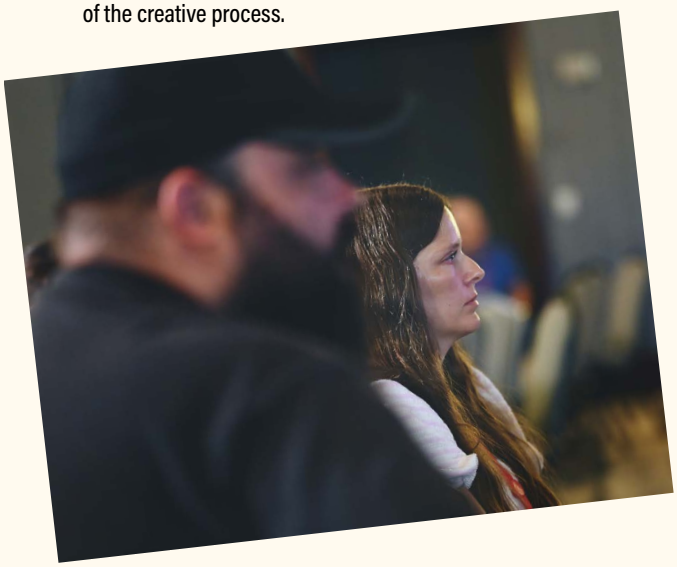
That was the idea behind an afternoon music-expression session led by Dennis Deruelle, MD, FHM. Patients and caregivers gathered in small groups with musicians and began not by trying to write songs, but by talking. A worksheet helped start the conversation, but the groups quickly found their own way through memories, frustrations, humor, fear, and hope. In one group, every line of the final song came directly from something a participant had said.

Suno, an AI-assisted songwriting and music-production platform, helped bridge the distance between those conversations and the technical work of composing a song. Dr. Deruelle and five professional music therapists and musicians from the Nashville area helped each group shape the participants' words into melody, instrumentation, and form, using AI as part of the creative process.

We are not alone.

"What surprised me most was my own reaction. I found myself tearing up—not just from sadness, but from happiness too. That's not something I do very often. Those songs brought everyone's experiences to the surface in a way that words alone never could."

— National Patient Meeting attendee



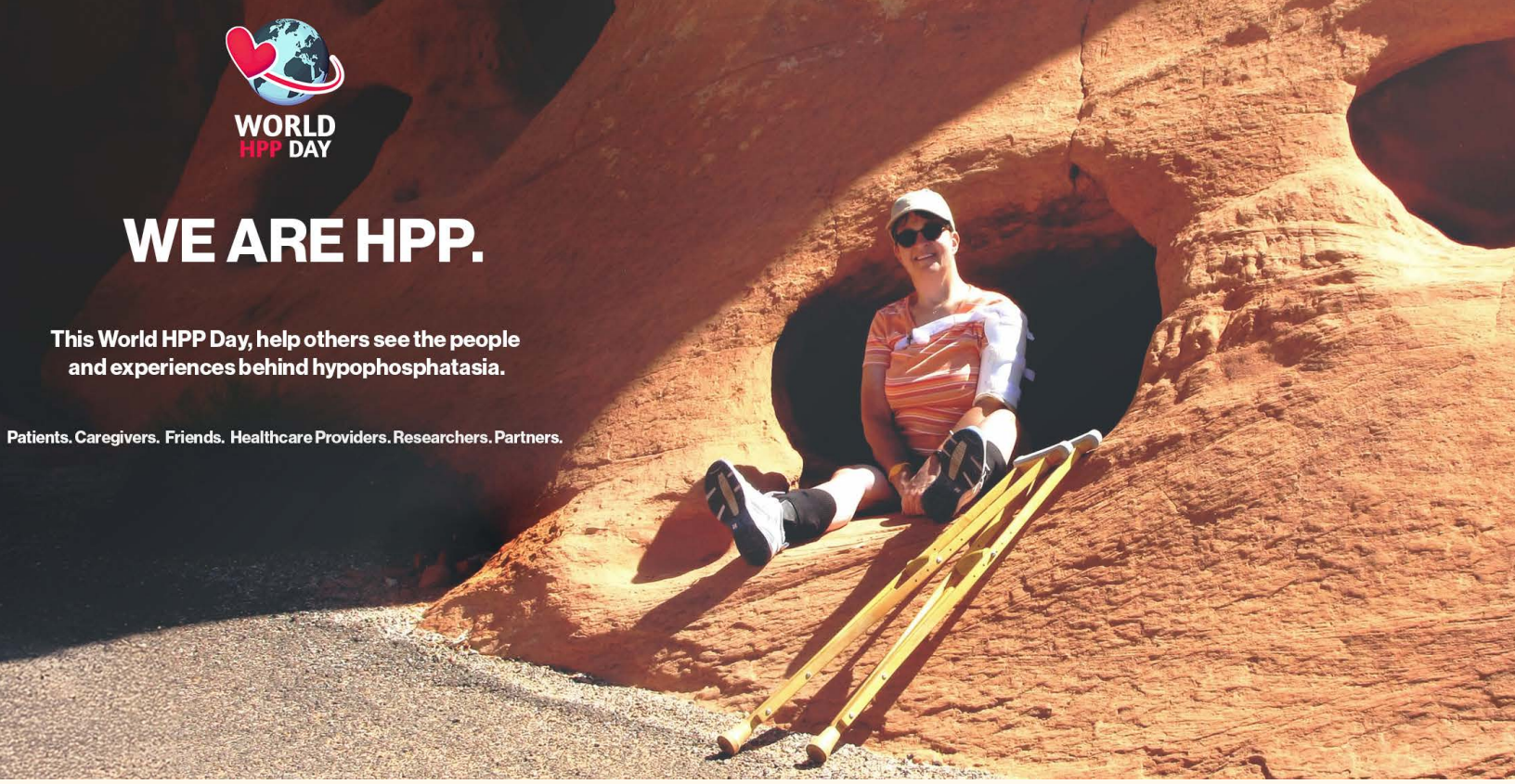
What emerged was vastly different from table to table. Some songs were tender; others were defiant or carried humor alongside pain. They spoke of being dismissed, searching for answers, supporting someone you love, living with symptoms others cannot see, and finding people who understand.

One song asked, "*Can we be who we want to be?*" Another returned again and again to the words "*We are not alone.*" Others explored the feeling of having HPP choose a path you never would have chosen, or of navigating different waves while moving through the same ocean.

At the end of the session, everyone listened to what each group had created. There was laughter, quiet reflection, and, during one song, the glow of cell phones swaying in the air.

For a community accustomed to explaining an uncommon disease, music offered another language, one that did not require every feeling to be translated into clinical terms. Patients, caregivers, and family members heard their experiences reflected back and recognized parts of themselves in one another's songs.

Listen to the songs from the 2026 National Patient Meeting HERE.



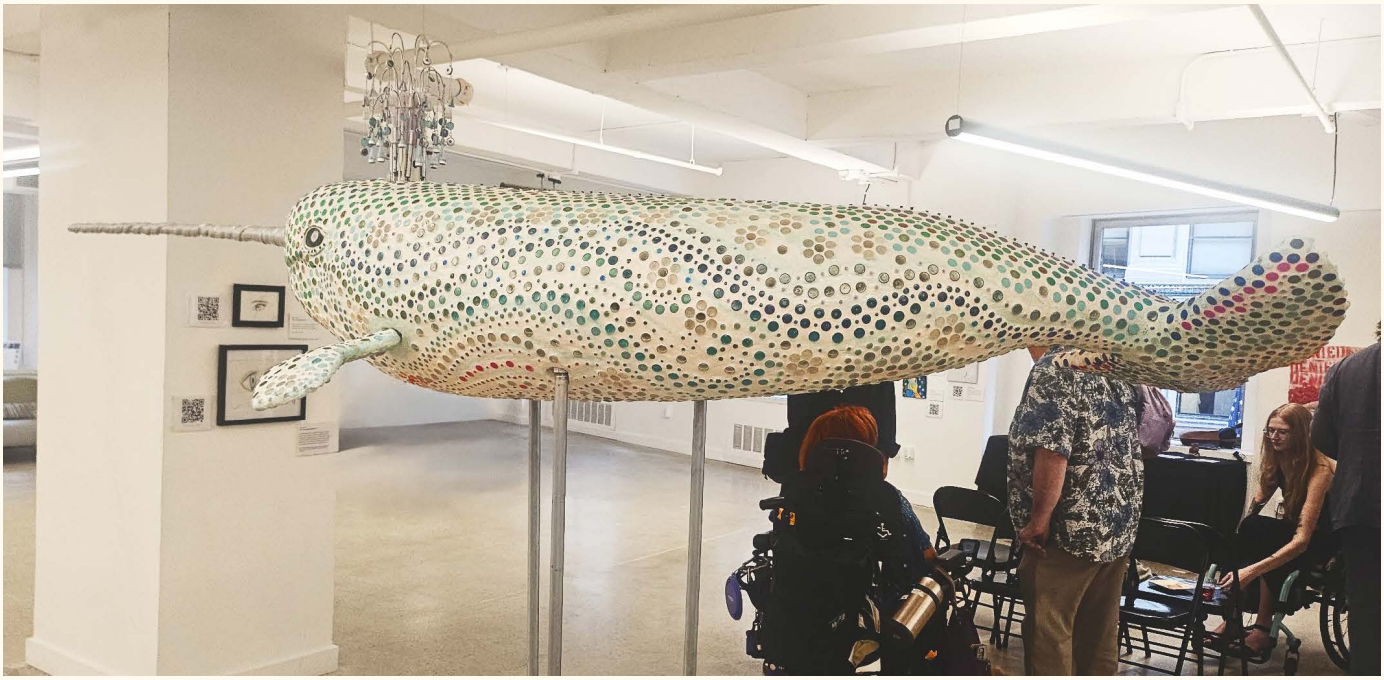
World HPP Day

World HPP Day | October 30

As World HPP Day approaches, you may recognize some familiar faces. This year, we are celebrating our Soft Bones community and inviting you to bring the people who are part of your own HPP experience into view.

That is the idea behind this year's theme, **We Are HPP**. Surrounding every person living with HPP is a wider community working to understand it, support one another, and move knowledge forward. Patients. Caregivers. Families. Friends. Doctors. Researchers. Advocates. Partners. The list goes on and on.

Throughout October, we invite you to recognize someone who has made a difference in your life with HPP. So many people are connected by hypophosphatasia, and we want to recognize as many of them as we can. Visit our World HPP Day page for resources, printable signs, and fact cards.



A Place for Rare to be Seen

On a summer evening in downtown Manhattan, the walls and gallery floor at Positive Exposure gallery were given over to Rare Square, an exhibition celebrating art from across the rare disease community.

Rare Square brought together portraits and artwork created from within rare communities—work that invited people to experience something about rare disease rather than simply read about it. Among the pieces on view was something familiar to Soft Bones: hundreds of medication caps covering the body of a nearly eight-and-a-half-foot narwhal named Baby.

Many in the Soft Bones community already know Suzanne D., and Bare Bones readers have followed Baby's progress along the way. This summer, we were able to see the project in its completed form and in a setting that gave it an entirely new context.

The idea had been developing for years. After Suzanne and her twin sister, Amy, who also lives with HPP, began treatment, both started saving the small caps and other materials that would ordinarily be discarded. The sisters had drawn a connection between HPP and the narwhal, an animal so unusual that people sometimes assume it is almost mythical. HPP can be similarly unfamiliar to those who do not know what to look for.

Eventually, Amy handed her growing collection to Suzanne, and Suzanne began to build. What emerged is part sculpture and part archive of life with HPP. Medication-shipping coolers form the body. Vial caps create its brightly patterned surface. An ACE bandage and patient-education materials became part of the tusk. A calcium bottle is concealed within the sculpture. Suzanne even left a broken section of the eye unrepaired after it cracked on the same day Amy was undergoing her third eye surgery.

The heart is made from a different set of red caps, materials connected to a potential treatment being studied in clinical trials. When Suzanne received them from another member of the HPP community, she saw them differently from the materials already covering Baby: as a glimpse toward what might come next.

That is what makes the sculpture particularly interesting in a gallery setting. The materials are not symbolic substitutes for living with a rare disease. They come directly from it. Objects associated with injections and medication shipments have been rearranged into something people want to circle and examine. Collected over years, they form a timeline of treatment and life with HPP. Details that might otherwise disappear into the routines of managing a rare disease become the beginning of a conversation.

For one evening in New York, Baby stood alongside the work of other rare artists and communities. HPP occupied physical space in the gallery—not through a brochure or educational panel, but through something made by a person who knows the disease from the inside.

Baby will continue traveling with Soft Bones, including an appearance at the 2026 ASBMR Annual Meeting in Boston.

Explore Baby up close and learn the stories behind the materials here.

Elissa S. (far left) with her teammates.

Community *in Motion*

FROM THE LOCAL COURT TO THE WORLD STAGE

> "I heard the crack of metal on metal."
> -Elissa S.

This summer, our Soft Bones Community member, Elissa S. earned a place on the U.S. women's pétanque team after winning the women's triples world qualifier, putting her on track to represent the United States at the 2027 World Championships. Pétanque is the french version of bocce.

For Elissa, the path to that moment has been anything but straightforward. Long before she had a name for HPP, she had spent years managing pain, unexplained injuries and appointments that often left her with more questions than answers. In 2024, a new rheumatologist noticed persistently low alkaline phosphatase in her lab work and raised the possibility of hypophosphatasia. Genetic testing later confirmed the diagnosis.

Pétanque entered her life almost by accident. After a year of severe back pain, Elissa went to a local park for some fresh air. "*I heard the crack of metal on metal,*" and stopped to watch a group of pétanque players throwing steel balls near their clubhouse. When one of them invited her to try, she said yes.

"*Honestly, I now have NO IDEA where the last two years of my life have gone!*" she later described in the Bone Zone's Patient of the Month, how she became hooked on the sport. Because players throw from a small circle without moving their feet, it also offered her a way to keep competing during a period when pain and mobility had become increasingly difficult.

As her health picture became clearer, so did the role of community. During the months when Elissa stepped back from pétanque to rest and work with a new medical team, she leaned on Soft Bones. "*I felt so supported and buoyed by their support and community during this time,*" she wrote. "*I had lots of questions, and they never made me feel silly for asking any of them.*"

And gave a special shout-out to Region Lead and Patient Liaison, Sue Krug. "*Sue, you're the best! We are SO lucky to have this organization to lean on.*"

Elissa eventually returned to pétanque and began competing more seriously. She won national gold in women's triples and a bronze medal in women's singles before earning her place on the U.S. women's team.

Now, a sport she first encountered during one of the hardest periods of her life is taking her onto an international stage. For those of us at Soft Bones, it has been especially meaningful to watch Elissa's journey unfold, and we're so happy to witness what comes next.

What are you up to? We want to hear about your pursuits and accomplishments happening across the HPP community. Share yours at Info@SoftBones.org.

HPP News

A New Home for an Investigational HPP Therapy

BioMarin has completed its acquisition of Alesta Therapeutics, bringing ALE1 into its clinical pipeline. ALE1 is an investigational oral small molecule being studied as a potential treatment for hypophosphatasia.

The treatment candidate is currently being evaluated in an ongoing Phase 1/2a clinical trial involving healthy volunteers and adults with HPP. The study is assessing its safety, tolerability and how it behaves and acts within the body.

The acquisition reflects continued scientific and commercial interest in developing additional approaches to HPP treatment. ALE1 remains investigational, however, and has not been approved or established as safe or effective.

Read BioMarin's announcement here.

Standing with HPP Patients in Korea

On July 27, Soft Bones submitted a formal letter to the Office of the President of the Republic of Korea and the Speaker of the National Assembly in support of improved treatment access for Korean patients living with HPP. Under Korea's current reimbursement criteria, patients must meet biochemical requirements, show characteristic bone manifestations through imaging and begin treatment before age 19. These restrictions can leave people diagnosed later in life, or those whose symptoms are not fully captured by radiographic findings, without access to treatment despite significant disease burden.

Soft Bones joined the Korean Hypophosphatasia Patient Association in calling for criteria that better reflect HPP as a lifelong disease and consider each patient's symptoms and clinical needs. A proposal to expand the country's reimbursement criteria, submitted to Korean authorities in April, remains under review. "*Korean patients with HPP and their families are not alone,*" said Deborah Fowler, Founder and Chairman of the Board of Soft Bones. "*The global HPP community supports their efforts to improve treatment access.*"

Read the full announcement here.

When Patient Participation Becomes Published Research

A University of Manitoba research team that included HPP expert Dr. Cheryl Rockman-Greenberg surveyed people with lived experience of HPP about their perspectives on in-utero enzyme replacement therapy. The survey, made available in part through the myHPP app has contributed to a newly published study in Molecular Genetics and Metabolism. The research examined how people with lived experience of HPP view in-utero enzyme replacement therapy for life-threatening forms of the disease. Ninety-two individuals completed the survey, with most supporting continued research and the availability of the treatment following prenatal detection. The study does not establish whether the investigational approach is safe or effective. Instead, it brings patient and family perspectives into the conversation while the research is still developing.

Its publication also demonstrates one of the purposes of tools such as myHPP: helping researchers reach people with direct experience of a rare disease and understand what matters to them. By taking part, members of the HPP community helped ensure that decisions surrounding future research are informed not only by clinical possibilities, but by the people and families those advances may affect.

Read the published study here.

Patient Voices

Amanda G.

For more than a decade, Amanda knew something did not add up. Pain, fatigue, neurological symptoms, and other concerns sent her from specialist to specialist, yet answers remained elusive. Eventually, she opened her own patient portal and noticed something that had appeared repeatedly for 14 years: low alkaline phosphatase. That clue ultimately led to an HPP diagnosis.

Amanda writes candidly about the work of self-advocacy, the loneliness of medical uncertainty and the relief of finding others whose experiences felt familiar. Soft Bones became part of that shift. *"That is what Soft Bones gave me,"* she writes of finding a community that understood without requiring explanation.

Her experience also changed the way her own physicians viewed chronically low ALP, knowledge she hopes may help another patient reach answers sooner.

Read Amanda's full story here.

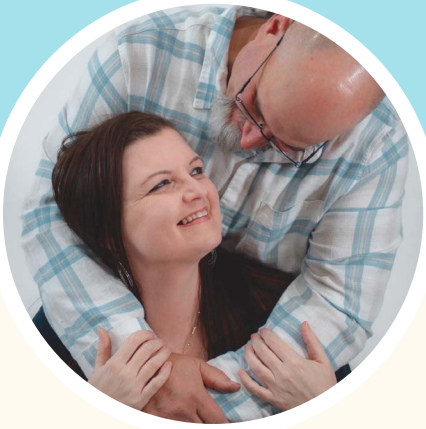
Michelle S.

Michelle spent more than 10 years searching for an explanation for persistent pain and symptoms that had often been attributed to weight, smoking, or other causes. Then a routine change in plans led her to her nurse practitioner, who reviewed Michelle's records and immediately noticed something others had overlooked: her alkaline phosphatase had been chronically low.

Genetic testing eventually confirmed HPP. For Michelle, finally having an answer brought both relief and a new set of questions, particularly after learning that two of her daughters also carry the variant.

She initially doubted whether connecting with Soft Bones would help. That changed as she met others living with HPP and attended the 2026 National Patient Meeting on her own. *"I didn't feel alone for very long,"* she recalls. *"Even though we are not biologically related, we have a connection. And you could feel it."* Sitting with people who truly understood what she was dealing with, she says, was *"the best feeling in the world."*

Read Michelle's full story here.



Francis S.

Written by his mom, Liviera
TW: Infant/baby loss

Francis's story began before he was born. His parents had already lost their first son, John, to HPP before they knew they both were carriers. That knowledge changed the course of Liviera's next pregnancy: when Francis's long bones began measuring behind in utero, the family knew what questions to ask and sought out specialists who could prepare for his arrival.

Francis received his first treatment only two hours after birth and spent three months in the NICU, including two months on a ventilator. Today, his life still includes injections, therapies, specialist appointments and feeding support, but it also includes walks, swimming, his little blue car and an enthusiastic love of buses.

Liviera credits Soft Bones with connecting the family to other parents during an extraordinarily uncertain time. For their family, community meant having people who understood the fear while still making room for hope.

Read Francis's full story here.

CONNECTED ACROSS THE COMMUNITY

TEEN ADVISORY COUNCIL

The Teen Advisory Council (TAC) took a short break over the summer, but several members were able to reconnect in person at the National Patient Meeting. Familiar faces that usually appear in Zoom squares had time to talk, learn and spend time together before the group returns for a new school year of activities.

Learn more about the TAC here.

HIPPO SQUAD

For younger members of the community, Hippo Squad continues to offer its own way to stay connected. The next Hippo Squad box arrives this October, centered around an activity to celebrate World HPP Day.

Want to join?

Register for Hippo Squad here.

SEP 28

GOLF CLASSIC

Somerset Hills Country Club, Bernardsville, NJ.
Registration is open.

OCT 3

MIDWEST 2 REGION MEETING

Midwest registration HERE

OCT 10

NORTHEAST REGION MEETING

Northeast registration HERE

OCT 15

UK ZOOM CHAT

UK Zoom registration HERE

OCT 17

SOUTHEAST REGION MEETING

Southeast registration HERE

OCT 17

SOUTHWEST REGION MEETING

Southwest registration HERE

FIND YOUR REGION

Central / Northeast / South Central / Southeast / Midwest 1 / Midwest 2 / Southwest / Northwest

Find your local Soft Bones community HERE.

CONNECTION CONTINUES...

HUNDREDX FUNDRAISER

This year, Soft Bones is asking for a little time from a lot of people. We know everyone is busy, so the ask is simple: find five minutes when you can.

Five thoughtful responses can generate about $8 for Soft Bones. On its own, that may not seem like much, but across our community, those small moments add up.

If you can find five, Sign up here.

A FOND FAREWELL TO MARGARET

After two years with Soft Bones, Margaret Robb, our Communications Manager and Patient/Caregiver Navigator, is headed into a medical lab career. She helped the Soft Bones team with her pleasant 'calm in the storm' demeanor but was laser focused with files and management.

She always found a solution to a problem, and we are certain that she will bring that same attention to detail with her upcoming endeavor.

We wish her every success in what comes next.

OCT 6

HUNDREDX FUNDRAISER ENDS

Find 5, give 5.

We appreciate your efforts during this fundraiser.

OCT 9-12

ASBMR ANNUAL MEETING

Boston, Massachussetts.

Soft Bones will have a booth featuring Suzanne D.'s Baby, the narwhal sculpture.

OCT 30

WORLD HPP DAY

This year's theme, We Are HPP, celebrates everyone who helps make the HPP community visible.

Patients. Caregivers. Families. Friends. Healthcare Providers. Researchers. Advocates. Partners.

Find ways to spread awareness here.

NOV 6-8

THIRD SOFT BONES INTERNATIONAL SCIENTIFIC MEETING

New York, New York.

NATIONAL PATIENT MEETING 2027

We have officially landed on our next location for next year's National Patient Meeting....Dallas , Texas!

More details coming soon.