Creating the Future

"The best way to predict the future is to create it." – Peter Drucker

By Brandi Felser, CEO

This quote recently set the tone for a global meeting of the Pushing Ultra Rare Sarcoma beyond Hope (PUSH) Consortium, which unites the international research community to advance new and better treatment options for people diagnosed with ultra-rare sarcoma subtypes.

PUSH has many moving parts. It requires thinking differently across borders, across systems, and across traditional research structures to advance multiple working groups, data collection efforts, and collaborative studies. Yet at its core, the consortium represents a new way of imagining what research can look like: a model that benefits patients while working with regulatory bodies, academic investigators, and pharmaceutical partners. It is, in every sense, a way to create the future we want for getting new therapies to patients.

As Administrator of PUSH, SFA deeply understands that the only path forward is global collaboration. With a limited patient population and limited research funding, we must work together across countries and disciplines, finding innovative ways to accelerate progress.

I was reminded of this recently by an email from a candidate applying for an open role at SFA. The message said: "Sorry for going outside the formal process, but since this role is focused on research and engagement, sometimes that demands a direct, proactive approach—so I hope you will forgive me!"

My response was simple: in our work—especially when supporting patients—we often need to be creative and resourceful. Sometimes that means going through the window instead of the door.

Both the quote and that exchange reflect what defines SFA. The spirit of "making things happen" and "creating the future" is woven into everything we do. Since our inception, our founders, early board members, and supporters have envisioned a future for sarcoma patients

that did not yet exist—one where research would advance, treatments would improve, a pipeline of investigators would be cultivated, funding for sarcoma would grow, and patients and families would receive meaningful support. For 25 years, SFA has continued to build on that vision.

Through research funding, administering collaborative efforts like PUSH, supporting patients as they navigate their sarcoma journey—such as through our discussion guide—and advancing advocacy, public policy, and strategic partnerships, we are driving progress in sarcoma.

We must continue thinking differently. We may still find ourselves climbing through windows when doors aren't open. But at SFA, we remain guided by a bold and unwavering vision: **a world where no one dies from sarcoma.** Everything we do is grounded in that belief—and in the future we are committed to creating.

Nominate a Sarcoma Patient Navigator for 2026 Compassionate Care Award

Do you know a sarcoma patient navigator whose compassion and dedication deserve recognition? Nominate them for the 2026 Compassionate Care Award! This award honors nurses, community health workers, and social workers for outstanding contributions to patient care, support, and education.

The selected honoree will be recognized at the 2026 Stand Up to Sarcoma Gala in New York City on October 6, 2026. **Please submit nominations by March 11, 2026.**

Learn more about the award and submit a nomination to recognize a navigator who has made a meaningful difference in the sarcoma community.

SFA's 2025 Compassionate Care Award Honoree

SFA's 2025 Compassionate Care Award recipient Christina Kim, NP (left), is a nurse practitioner at the Mass General Cancer Center. Nominated by her patient, Jonathan Gardner, she is pictured here with him at the 2025 Stand Up to Sarcoma Gala.

RESEARCH ROUNDUP

Highlighted Research

By Dean Frohlich

The prognosis for sarcoma patients with unresectable or metastatic disease is poor and in the first study this month, "Cabozantinib and temozolomide in patients with unresectable or metastatic leiomyosarcoma and other soft tissue sarcomas: a multicentre, single-arm, lead-in phase 2 trial," the researchers conducted a multicenter, single-arm, lead-in phase 2 trial that used a combination of a drug that stops signaling of cancer cells to divide and the growth of new blood vessels into the tumor (cabozantinib) along with another drug that causes damage to tumor DNA leading to tumor cell death (temozolomide) in patients with unresectable or metastatic leiomyosarcoma and other soft tissue sarcomas.

Eligible patients had confirmed unresectable or metastatic uterine and non-uterine leiomyosarcoma (group 1) and other soft tissue sarcomas (group 2), and up to five previous chemotherapy regimens. Patients were given the combination therapy if their white blood cell and platelet counts were satisfactory until disease progression or unacceptable drug-related adverse events. The primary endpoint for group 1 was progression-free survival at 12 weeks. A total of 72 patients were enrolled between Jan 17, 2020, and Feb 6, 2023. There were 42 patients in group 1 and 30 patients in group 2, with a median follow-up of 18 months. In group 1, progression-free survival at 12 weeks was reached by 31 (74%) of 42 patients. Additionally, the combination therapy was tolerable and did not reveal any new safety signals. 20 (48%) patients in group 1 and 23 (77%) in group 2 had died due to disease progression at the end of the study. There were no treatment-related deaths.

These results indicate that, although additional studies need to be done, Cabozantinib with temozolomide may be a treatment option for patients with unresectable or metastatic leiomyosarcoma.

Many subtypes of sarcoma occur so infrequently that researchers have not been able to determine the course of these diseases from beginning to end, called the natural history. In the second study, "International multicenter retrospective study on pleomorphic rhabdomyosarcoma (P-RMS), a PUSH platform study: outcome of primary localized disease," researchers have done an analysis of the outcomes of patients with primary localized pleomorphic rhabdomyosarcoma (P-RMS). In total, 93 P-RMS patients who were over 40 years old and treated with surgery from January 2013 to December 2023 from 25 institutions across 12 countries were enrolled. The primary endpoint was overall survival (OS). Secondary endpoints were disease-free survival

(DFS), crude cumulative incidence (CCI) of local recurrence (LR), CCI of distant metastases (DM), post-relapse OS, and post-metastasis OS.

At a median follow-up of 39.8 months, 32 of 93 (34%) patients died and in 53 of 93 (57%) patients the cancer had recurred. The 5-year OS and DFS were 57.7% and 35.8%, respectively. Positive prognostic factors for disease free survival included a younger age at diagnosis, surgical edges free of tumor cells, and administration of radiotherapy. Local metastases and distant metastases occurred in 14/93 (15%) and 39/93 (42%) patients. The 3-year CCI-LR and DM were 16% and 44%. Post-metastasis median OS was 14.3 months.

While additional research needs to be done, and a prospective observational trial is underway, this study sets the groundwork for understanding the natural history of P-RMS. The results here indicate that radiation therapy may improve local control and chemotherapy may be beneficial. It also highlights the importance of clear surgical margins in the initial treatment of the disease.

Lastly, since sarcomas are rare and there are many subtypes, they are difficult to diagnose. New technologies are being developed to aid pathologists in the identification of sarcomas. In "Multicenter Histology Image Integration and Multiscale Deep Learning Support Machine Learning-Enabled Pediatric Sarcoma Classification," investigators used a set of 867 whole slide images (WSIs) from three medical centers and the Children's Oncology Group (COG) of Pediatric sarcomas along with deep learning methods to develop computational pathway to accurately classify pediatric sarcoma subtypes from digitized histology slides.

The newly developed models were able to distinguish rhabdomyosarcoma (RMS) from non-rhabdomyosarcoma (NRSTS) and differentiating rhabdomyosarcoma subtypes (alveolar vs. embryonal). Additionally, a two-stage pipeline was able to distinguish Ewing sarcoma images from other NRSTS. These technologies require further development, but this study highlights that progress is being made on the ability to properly identify subtypes more efficiently and get patients on the proper treatment earlier.

Clinical Trials Corner

The ATLAS trial is a Phase 1/2 clinical study evaluating a novel therapeutic approach for patients with relapsed or treatment-resistant soft-tissue sarcoma. The investigational treatment, [Ac-225]RTX-2358, is a targeted radiopharmaceutical designed to bind to fibroblast activation protein-alpha (FAP), a protein commonly found on sarcoma cells. Once bound, the therapy delivers radiation directly to tumor tissue using the radioactive isotope Actinium-225. Before enrolling, participants undergo a PET scan with the diagnostic imaging agent [Cu-64]LNTH-1363S to confirm that their tumor expresses FAP and is suitable for this targeted

therapy. The primary goals of the trial are to evaluate the safety, tolerability, and optimal dosing of [Ac-225]RTX-2358, as well as to assess its potential effectiveness in treating advanced sarcoma.

The study includes two parts: Phase 1 focuses on identifying the safest and most tolerable dose. Participants receive an intravenous treatment once every eight weeks, for up to six cycles over approximately one year. Phase 2 evaluates how well the selected dose works against the cancer in a larger group of patients. Participants receive [Ac-225]RTX-2358 once every eight weeks for a total of 4–6 treatment cycles. In the first cycle, patients attend multiple clinic visits for safety assessments and laboratory testing, followed by bi-weekly visits in subsequent cycles. After completing treatment, patients enter a four-year long-term follow-up period to monitor ongoing safety and outcomes.

To learn more about this study, patients and/or care partners can talk to their doctor or reach out to the study contact. If you think you may be eligible or interested in participating and are in need of travel or financial support to do so, you may apply for assistance from SFA.

Researcher Spotlight

Karen E. Pollok, PhD, is the Caroline Symmes Professor of Pediatric Cancer Research and a Professor of Pediatrics at the Indiana University School of Medicine. She discusses her research, *Dual targeting of CDK4/6 and PI3K/mTOR in high-risk osteosarcoma*, and the critical role SFA funding had in supporting this work. Dr. Pollock and her colleagues recently published a manuscript from this research in the journal *Neoplasia*.

What question were you trying to answer through this research?

We wanted to know whether using two drugs together—one that stops cancer cells from dividing (a CDK4/6 inhibitor called palbociclib) and another that blocks a key growth pathway (a PI3K/mTOR inhibitor called voxtalisib)—could better control osteosarcoma, a very aggressive bone cancer that affects children, adolescents, and young adults. Each drug alone had shown promise, but we believed that combining them could be more effective and longer-lasting while remaining safe.

What was your biggest takeaway or most exciting finding from this research?

Our most exciting finding is that the combination of palbociclib and voxtalisib produced durable, well-tolerated tumor growth inhibition in both newly diagnosed and previously treated metastatic osteosarcoma models. Even more encouraging, in a lung colonization (metastasis) model, the CDK4/6 inhibitor palbociclib—used alone or together with voxtalisib—markedly reduced the number of metastatic tumors in the lungs, where osteosarcoma most often spreads. We are now conducting long-term studies to see whether the combination therapy can go even further—preventing new metastatic tumors from forming and sustaining long-term control.

How could your findings make a difference for patients and families in the sarcoma community?

For families facing osteosarcoma—especially when the cancer has spread to the lungs and standard treatments no longer work—options are limited. Our findings bring real hope that these targeted drugs, already used safely in other cancers, could help control both the primary tumor and metastatic disease in osteosarcoma. This approach could lead to new, less toxic therapies that improve survival and quality of life for children and young adults battling this devastating cancer.

Why is funding from organizations like SFA so important for rare cancer research?

Support from organizations like SFA makes early-stage, high-risk research possible. For rare cancers such as osteosarcoma, large federal grants are difficult to secure without solid preliminary data. SFA's investment helped us take those first critical steps—turning promising scientific ideas into discoveries that now underpin a federally funded program aimed at changing how we treat osteosarcoma.

SFA Global

Drug Repurposing – Changing the Rules

By Pan Pantziarka

Drug repurposing is the use of an existing approved drug to treat a new disease - for example, aspirin is a pain killer that's now used worldwide in cardiovascular disease. It's a complementary strategy to developing new drugs from scratch. For rare diseases, including sarcomas, repurposing is an attractive proposition when commercial organizations don't see a big market for developing a new drug. Most drugs used in sarcoma were originally developed for other cancers and the sarcoma use came later - this is called 'soft repurposing' as it's reusing a cancer drug to treat a different cancer. Another form of repurposing is called 'hard repurposing' - this is where a non-cancer drug is used to treat cancer. For example, The Bacillus Calmette–Guérin (BCG) vaccine is used to prevent tuberculosis, but it's also used now to treat bladder cancer. It's no surprise then that repurposing - both hard and soft - are big topics in the sarcoma world.

One of the problems that drug repurposing faces in sarcomas is that the data showing a drug is effective is often produced by academic or not-for-profit organizations rather than by the companies that market the drug. When a clinical trial is positive, and it shows a repurposed drug is effective, the question is paramount about what happens with the data. A drug can only be approved for a new disease if a company applies to the regulator (FDA in the US, EMA in Europe etc.). If the company, for whatever reason, doesn't want to apply, then those positive results end up in a published paper or conference presentation only. In the US the drug might be added to the NCCN guidelines, which means that it will be made available to many patients, even if it's an 'off label' prescription. In Europe, it means the drug will remain unavailable to most patients, even if the doctors know that it might be a good treatment for their patients.

All of this is about to change, however. The European Union (EU) has just agreed to a complete overhaul of its pharmaceutical legislation - the first in 20 years. There are a host of changes in the revised regulations, covering everything from drug supply issues to patent protection to drug repurposing. For the first time ever, academics will be able to present data showing a drug is effective in a new disease, even if the company that markets the drug is not involved. The EMA will assess the data and if it is judged to be effective, the EMA will instruct the company to add the new disease to the label for the drug. This will ultimately make the drug available to patients - at a stroke by-passing a major obstacle to drug repurposing. For patients with rare and ultra rare diseases, including sarcomas, academic clinical trial results will now have a clearer path to implementation and patient access.

The details of how this change in regulation will be implemented are still to be released, so for now we must wait to see how much of an impact it will have. In Europe, sarcoma doctors are already looking at the backlog of trial data they've had where they've not been able to progress because there was no company involvement - that data may soon be informing drug approval decisions by the EMA. At the same time, plans for new clinical trials are also being prepared - no longer constrained by company involvement. These are positive changes - and perhaps regulators outside of the EU should also be taking notes.

New rEECur Treatment Group to Open in Europe for Relapsed Ewing Sarcoma

The rEECur trial will open a new treatment group using trabectedin for patients with relapsed Ewing sarcoma in the UK and other countries later in 2026. This development came from collaboration between patient advocates, researchers, trial funders, and PharmaMar.

Trabectedin is the first drug in the trial that directly targets the gene fusion that drives Ewing sarcoma. The treatment is given in an outpatient clinic and is supported by clinical data from ASCO 2024.

The trial is run by the Cancer Research Clinical Trials Unit at the University of Birmingham and the Euro Ewing Consortium. Learn more.

ADVOCACY AND ENGAGEMENT

Listen to the Latest Episodes of Our Sarcoma Stories Podcast

In recent episodes of Sarcoma Stories, we sit down with patients, survivors, caregivers, and advocates whose experiences inspire and inform the sarcoma community. From navigating diagnosis and recovery to celebrating 25 years of progress, these conversations remind us that no one faces sarcoma alone.

Julie Harp: Care Partner and Advocate

In this episode, we speak with Julie Harp, who shares her experience as a care partner to her son, Don, during his sarcoma journey and as he approached the end of life. Julie offers a unique and powerful perspective on caring for an adult child through terminal illness.

Julie reflects on Don's path to diagnosis, including misdiagnosis and the feeling of being lost within the medical system. Julie emphasizes the importance of self-advocacy and the need for better systems to help patients navigate the healthcare system and achieve timely care. She also shares how she continues to honor Don's legacy through her advocacy work, fighting for better awareness, research, and outcomes for sarcoma patients.

With courage and compassion, Julie not only tells Don's story and her family's experience with sarcoma but also reminds us of the importance of being an audible voice for inaudible voices, as we continue to push for better treatments and hope for all affected by sarcoma.

Listen to all episodes of the Sarcoma Stories podcast

Become a Mentor in Our New Sarcoma Match Program

SFA is now recruiting Mentor Angels for Sarcoma Match, our free peer-to-peer support program, in partnership with Imerman Angels.

Through Sarcoma Match, individuals affected by sarcoma are connected one-on-one with someone who has shared experience. Mentors may be patients, survivors, caregivers, or siblings of patients who are willing to offer empathy, perspective, and encouragement to others navigating sarcoma. Learn more about becoming a mentor. We will open enrollment for mentees soon.

SFA NEWS

A Warm Welcome to Our New Development Manager

Caileen Coleman is a non-profit leader and advocate for health and disability equity. As an Osteosarcoma survivor with a cancer-related disability, Caileen is passionate about SFA's mission to improve the lives of sarcoma patients and their families.

Caileen is thrilled to join SFA's Development team and raise awareness and funds for lifesaving sarcoma research, patient support services, and public policy improvements.

Prior to SFA, Caileen was a Senior Development Manager at the American Cancer Society, a Special Education teacher and leader, and Fulbright Scholar. She holds a BA in Communications from UCLA and MA in Education from the University of New Orleans.

Outside of work, Caileen is on the board of directors for the Lafitte Greenway Partnership and is a member of the AdaptaBelles, a collective of women with disabilities. Caileen enjoys traveling and trying new foods, loves all things nature and water, and is certified to teach accessible yoga. On the weekends, you can usually find her enjoying music and food festivals in New Orleans.

Save the Date for the Sunflower Society Webinar

Join us online on March 4th at 12 PM ET to hear from CEO Brandi Felser and Dean Frohlich PhD, Director of Scientific Affairs, as we review 2025 accomplishments and discuss our plans for 2026.

SFA's Sunflower Society is comprised of individuals whose donations of $1,000 or more per calendar year collectively move us one step closer to accelerating progress and improving outcomes for sarcoma patients. Your commitment helps us continue to be the leading voice for the sarcoma community. We could not do this without you.

To learn more about joining the webinar and the SFA Sunflower Society, contact Caileen Coleman at ccoleman@curesarcoma.org.

Save the Date for the 2026 Stand Up to Sarcoma Gala

Stand Up to Sarcoma, now in its 24th year, is a night of celebration and community, bringing together the sarcoma community from across the country and around the world in solidarity to support sarcoma patients and survivors, honor our loved ones, and recognize progress.

Save the Date for Sarcoma Advocacy Weekend

Sarcoma Awareness Month is right around the corner and so is the [2026 Sarcoma Advocacy Weekend](). Mark your calendar to advocate on behalf of the sarcoma community. Together, we can improve outcomes for people diagnosed and living with sarcoma.

RACE TO CURE SARCOMA

Run with Team SFA at the 2026 Marine Corps Marathon

Registration is now open to join Team SFA at the Marine Corps Marathon on October 25, 2026, in Arlington, VA. Run in support of sarcoma patients and their families while taking part in one of the nation's most iconic races.

As a proud partner of the Marine Corps Marathon, SFA's Marathon Teams have raised nearly $100,000 for sarcoma research, and we look forward to continuing that impact in 2026.

To join the team, contact Annie Blake at ablake@curesarcoma.org Runners who already have a race entry are also welcome to join Team SFA and fundraise in support of SFA's mission. Learn more.

Sign up for SFA's 2026 Race to Cure Sarcoma Events!

More than just a race, Race to Cure Sarcoma events are a chance to connect with others in the sarcoma community, recognize people living with sarcoma, honor those we've lost, and fund vital sarcoma research. Whether you walk, run, or cheer, you'll be making a difference!

Find your city and sign up today!

- South Florida—2/7/2026
- Austin — 3/21/2026
- Atlanta — 4/4/2026
- Boston — 4/5/2026
- New York City — 4/25/2026
- San Francisco — 5/9/2026
- Cleveland — 6/20/2026
- Milwaukee — 7/11/2026
- Washington D.C. — 7/18/2026
  *location may change
- Louisville — 8/8/2026
- Philadelphia — 8/29/2026
- San Diego — 9/19/2026
- Chicago — 9/26/2026
- New Jersey — 10/11/2026
- Denver — 10/24/2026
- Los Angeles —11/15/2026