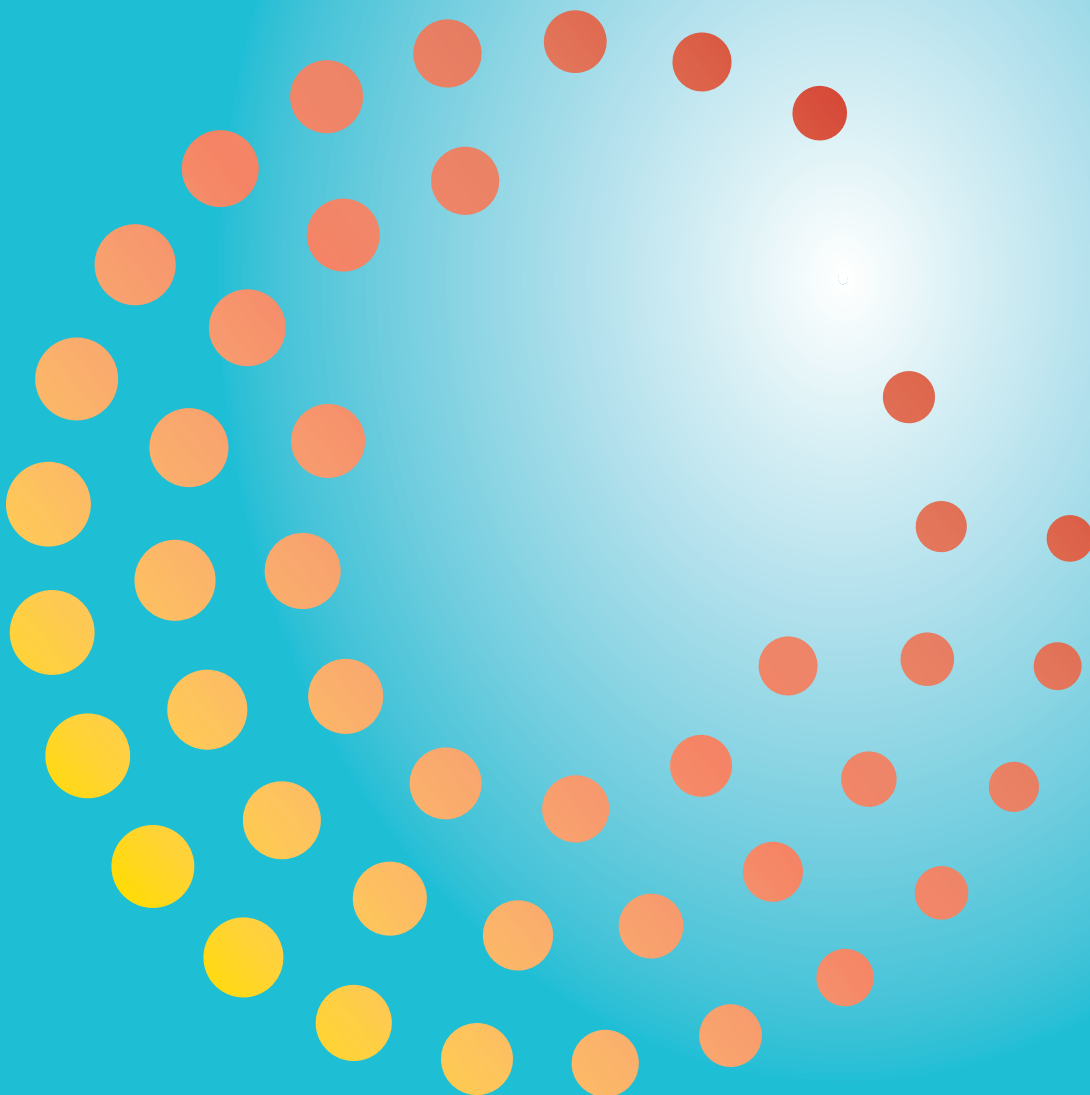




COLLABORATION
ACTION
EVOLUTION

2020 PROGRESS REPORT



Collaborating to cure sarcoma

SARC was founded in 2003 by sarcoma researchers who recognize the opportunity to improve outcomes for sarcoma patients by working together. Today we have a dedicated membership of researchers from across the country working collaboratively to achieve breakthroughs in sarcoma – to ultimately find a cure.

Why we are here

Sarcoma is an uncommon type of cancer, accounting for 1% of adult cancers and 15% of pediatric cancers. Although sarcoma is a rare disease, its impact is significant for patients and families who face it.

SARC and our collaborators are working to advance the science and knowledge of sarcomas to improve outcomes for patients with sarcoma.

About sarcomas

Sarcomas are cancers of the bones, muscles, and other soft tissues attached to, supporting, or joining the bones. Sarcomas can occur in people of all ages – infants, young children, teenagers, and adults. There are many different types of sarcomas – experts have identified more than 70 subtypes – but collectively it is a rare disease. Additionally, there are a limited number of sarcoma experts and it can be challenging to accurately diagnosis sarcoma.

In order to help people facing this uncommon cancer it is critical to share sarcoma knowledge and resources so that progress can be made.

OUR MISSION

SARC provides the collaborative infrastructure to support progress in the prevention, diagnosis, and treatment of sarcoma.



DENISE REINKE, MS, NP, MBA
PAST PRESIDENT & CEO

What started as an idea nearly two decades ago

to bring together a clinical trials group has evolved beyond what any of us could have imagined in the beginning. Today, the SARC community has expanded to include not only a greater number of sarcoma specialists – from the laboratory to the clinic – but also many collaborative partnerships including pharma and biotech, as well as several incredibly passionate patient advocacy groups.

As we reflect upon SARC’s past 10 years in this progress report, I trust you’ll be inspired by the impact of this collaborative effort.

WE’VE EXPANDED OUR SCIENTIFIC WORK. WE’VE EXPANDED OUR RESOURCES. WE’VE EXPANDED OUR PARTNERS AND ADVOCATES. AND WE’VE EXPANDED OUR UNDERSTANDING OF SARCOMA.

Perhaps one of our most notable achievements in the past 10 years was being awarded a Sarcoma SPORE grant – peer-reviewed government funding to support translational research and translational research teams. Our investigators’ unique skills and the infrastructure that SARC has built make us uniquely positioned for this type of collaborative grant research.

The opportunity to explore the potential benefit of immunotherapy in sarcoma through the SARCO28 trial represents another milestone for our SARC community. This investigator-initiated trial was the first multi-center, phase 2 study of immunotherapy in patients with advanced sarcoma, including soft tissue and bone sarcoma. The entire study is a testament to the collaborative efforts of SARC investigators, industry, and philanthropic organizations, which enabled access to the drug, rapid accrual of patients, and the collection of high-quality data and biospecimens to examine the potential benefit of immunotherapy in sarcoma.

Our progress includes initiating the SARC Career Development program in 2009. With it, we’ve expanded and bolstered our scientific community through Career Development Awards, which provide early career support to help ensure the continual renewal of top-notch scientific researchers. We have watched young investigators initiate research ideas, establish careers, and dedicate their life’s work to sarcoma translational research.

Our semiannual SARC meetings have long provided the forum for sarcoma collaborators to gather, present and discuss clinical research, share insights and discoveries, spark ideas, and inspire new directions for researchers to pursue. We have conducted more than 30 SARC meetings at ASCO and CTOS, joined by hundreds of sarcoma colleagues from across the country and around the world – all gathered with the common goal to better understand sarcoma and ultimately improve outcomes.

Ultimately, our work is dedicated to helping the patients who face sarcoma, and their families. Increasingly, we have recognized the importance of patient engagement – ensuring that patients and their families have a voice in the research arena, understand the importance of clinical research in improving outcomes, are informed about the research process, and importantly know how to access centers with sarcoma experts.

Looking ahead, and under the new leadership of Steven Young, I’m confident the components are in place to enable this organization to continue providing the platform to bring together those committed to achieving sarcoma breakthroughs and ultimately making a difference in the lives of patients and their families. Because progress toward curing sarcoma will be best achieved through collaboration.



DENISE REINKE

Denise has been with the Sarcoma Alliance for Research Through Collaboration since its inception in 2003. She took on the role of President following the departure of SARC co-founder Dr. Laurence Baker, who served as the Executive Director and Chairman through 2011. Denise served as President since 2007 and was named President & CEO of SARC in 2014.

**SARC
COLLABORATION
ACTION
EVOLUTION**

SARC CLINICAL TRIALS
26 TRIALS CONDUCTED
SINCE 2003

**SUPPORTING SCIENCE
CAREERS**

CAREER DEVELOPMENT AWARDS
LAUNCHED 2009
26 RECIPIENTS AWARDED

5-YEAR NIH SPORE GRANT
AWARDED 2012

**MILESTONES IN
TRANSLATIONAL
RESEARCH**

**COLLABORATION
WITH PARTNERS**

2014 PHASE 3 STUDY CONDUCTED AT
99 CENTERS ACROSS THE WORLD,
ACCRUING 641 PATIENTS IN
COLLABORATION WITH THRESHOLD
PHARMACEUTICALS

2017 DEMONSTRATION PROJECT RFP
SUPPORTING USE OF BANKED
BIOSPECIMENS, DATA, AND IMAGES

**RESOURCES
FOR RESEARCHERS**

**SARCOMA COMMUNITY
ADVOCATES**

SARCOMA COALITION
RESEARCH ADVOCACY COUNCIL
ORGANIZED IN 2019

2018-2022 FUNDING
FOR THE "LMSARC" PROGRAM

**PROGRAM
SUPPORT**

Founded 2003

2009

2011

2013

2015

2017

2019

The core of our work is clinical trials – facilitating the development and support of studies, with a focus on continually achieving better outcomes for patients. Our sarcoma research community embraces every opportunity to capture data and findings at the bedside, return to the bench with new insights, and take another step forward.

SARC024

Phase 2 study of regorafenib in osteosarcoma, Ewing, liposarcoma and rhabdomyosarcoma

OVERALL STUDY PRINCIPAL INVESTIGATOR

ROBERT MAKI, MD, PHD, FACP, FASCO

Clinical Director, Sarcoma Program, University of Pennsylvania

COHORT STUDY PRINCIPAL INVESTIGATORS

Liposarcoma: Richard Riedel, MD, Duke University

Osteosarcoma: Chris Ryan, MD and Lara Davis, MD, Oregon Health & Science University

Ewing Sarcoma: Steven Attia, DO, Mayo Clinic, Florida

Rhabdomyosarcoma: Leo Mascarenhas, MD, MS, Children’s Hospital Los Angeles

Study Highlights

- Multiple-arm trial of several specific subtypes in a single study
- Senior PI with cohort specific PIs engaged more investigators in research leadership
- In osteosarcoma, median PFS was significantly improved with regorafenib versus placebo
- With a trial from France, provided possible FDA registration-enabling data
- These data represent the first active systemic therapy in metastatic osteosarcoma in 30 years

SARC028

Phase 2 study of pembrolizumab in advanced sarcomas

OVERALL STUDY PRINCIPAL INVESTIGATOR

HUSSEIN TAWBI, MD, PHD

Associate Professor, MD Anderson Cancer Center, The University of Texas

Study Highlights

- First published study to test immune checkpoint inhibitors to patients with advanced sarcomas
- Enrolled patients from 12 sites with 7 histologic subtypes to methodically examine the most common bone and soft tissue sarcomas
- Identified a potential signal of activity in only 2 soft tissue sarcoma subtypes (liposarcoma and undifferentiated pleomorphic sarcoma)
- Confirmed meaningful clinical activity in an expansion cohort (response rate 23%) in the most common soft tissue sarcoma
- Identified markers of a better response (such as PD-L1 status) from pre- and post-treatment biopsy specimens
- Validated a novel marker of useful immune responses in sarcomas, the Sarcoma Immune Classifier (SIC)

SARC033

Phase 2 study of trametinib in epithelioid hemangioendothelioma

OVERALL STUDY PRINCIPAL INVESTIGATOR

SCOTT SCHUETZE, MD, PHD

Professor, Medical Oncology, and Internal Medicine, University of Michigan

Study Highlights

- Study design underscored the need for disease-specific trials; first EHE-specific trial conducted and completed in the U.S.
- Demonstrated accrual to scientifically-based clinical trials could be conducted in an ultra-rare sarcoma subtype (< 1 per million per year incidence)
- Demonstrated that patients with rare sarcoma subtypes are eager to participate in cutting-edge clinical research that contributes to improvements in therapeutic options
- Model example of the benefit of collaboration between academia, NCI, and a patient-supported sarcoma foundation



ROBERT MAKI, MD, PHD
SARC024 Principal Investigator
SARC SCIENTIFIC STEERING COMMITTEE MEMBER



HUSSEIN TAWBI, MD, PHD
SARC028 Principal Investigator



SCOTT SCHUETZE, MD, PHD
SARC033 Principal Investigator

A MILESTONE IN TRANSLATIONAL RESEARCH

The collaborative infrastructure SARC has built positions us for truly collaborative grant writing and research. Successful grant funding awards offer proof of the SARC concept: bringing research teams together – from the laboratory to the clinic – to advance translational research.



Awarded a Sarcoma SPORE Grant

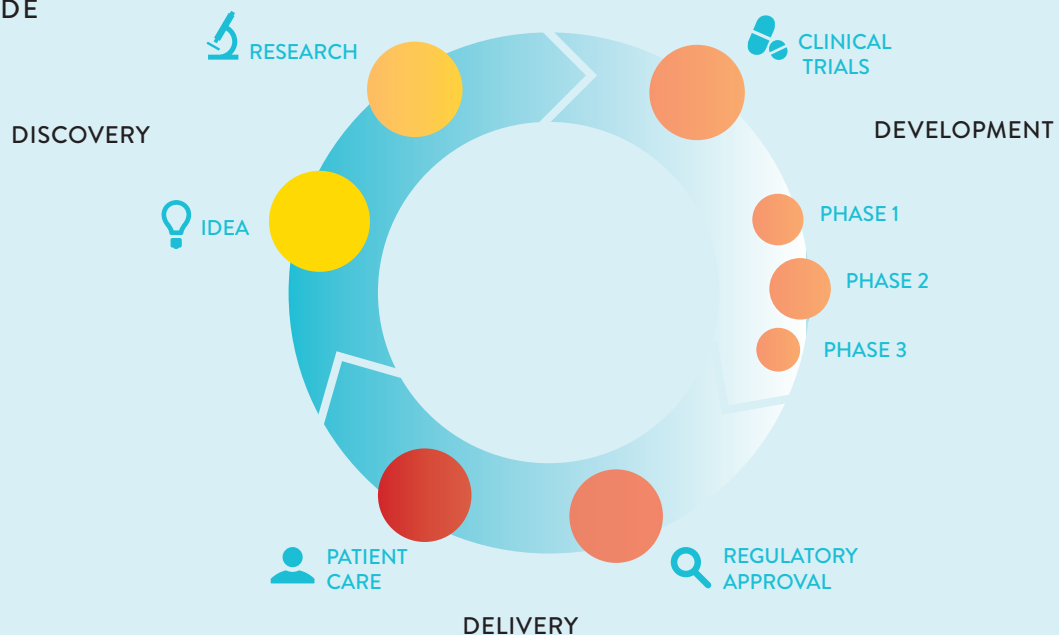
Recognizing the importance of understanding the biology of sarcoma when designing translational sarcoma research, SARC convened a team of sarcoma laboratory and clinical scientists, under the leadership of Dr. Larry Baker, to explore the possibility of pursuing NIH funding of SARC-led translational research. Through the collaborative spirit and strengths of the team members, SARC was awarded a Sarcoma SPORE (Specialized Programs of Research Excellence) grant by the National Cancer Institute in 2012. This is the first SPORE to be awarded to a nonprofit consortium of academic researchers, and only the second SPORE to be awarded in sarcoma research.

LAURENCE H. BAKER, DO
Professor, Michigan Medicine
Sarcoma Clinic, Rogel Cancer Center

**SARC BOARD MEMBER;
SARC CO-FOUNDER, SARC
EXECUTIVE DIRECTOR AND
CHAIRMAN 2003–2011**

“SARC has provided an infrastructure to support translational sarcoma research since its incorporation in 2003,” Dr. Baker said. “This SPORE grant leveraged the organizational structure of SARC to serve as the focal point for clinical trials and related clinical and laboratory research.”

BENCH TO BEDSIDE AND BACK AGAIN



We've established a vital collection of biospecimens, imaging, and data sets through clinical trials. These are available to help investigators and organizations in their scientific inquiry and translational discovery.

SARC Demonstration Project

Having completed several clinical trials and translational projects, SARC was able to establish a repository of annotated biospecimens linked to clinical data sets and imaging studies (anatomical and functional). These resources are inventoried and available to the sarcoma community. To inspire new ideas, in 2017 SARC issued a request for proposals to support and encourage sarcoma clinical and basic researchers to use these resources. It was expected that a successful demonstration project would yield the preliminary data needed for further grant-funded research.

Dr. Brian D. Crompton was the recipient of a 2017 SARC Demonstration Project Grant that supported his project entitled Development of a Circulating Tumor DNA Assay for Leiomyosarcoma.

“This is an exciting opportunity to apply emerging technology to an important but relatively rare disease. We hope the results of this project initiate a new era of risk-based treatment strategies for patients with soft-tissue sarcomas by utilizing non-invasive liquid biopsy assays for risk-adaptive personalized treatment approaches,” said Dr. Crompton. His work supported by the Demonstration Grant now serves as the basis for a project that is part of a SARC multi-center grant application that will be submitted to the NIH in Fall 2020.

SARC Unified Data System (UDS)

SARC Biospecimen Bank

SARC has banked human biospecimens from consenting sarcoma patients participating in our clinical trials into a single biorepository. The collection consists of hundreds of well-annotated specimens and is designed to help qualified sarcoma investigators or organizations access specimens to explore biomarkers, identify therapeutic targets, or make discoveries.

SARC Clinical Data Repository

SARC has established a repository of clinical data sets which includes essential clinical trial data acquired over the years from our clinical research projects. This data is now available to sarcoma researchers for further study.

SARC Imaging Resources

SARC has compiled a significant number of radiographic imaging studies from several SARC clinical trials into a data imaging repository. This bank of images is linked to clinical data and available to all sarcoma investigators. Investigators are invited to use these resources for further exploration and the generation of new ideas in sarcoma research and understanding.



“We hope the results of this project may initiate a new era of risk-based treatment strategies.”

BRIAN D. CROMPTON, MD
Assistant Professor of Pediatrics,
Harvard Medical School

**SARC DEMONSTRATION
PROJECT AWARD RECIPIENT**

SARC Unified Data System (UDS) houses SARC resources which are available to the sarcoma community for further research. SARC UDS was developed with grant support from the QuadW Foundation.



A core value of SARC is to foster the careers of young and early career investigators through support and mentorship, assisting them in dedicating their careers to sarcoma, and ensuring a strong community of sarcoma researchers in the future.



“In supporting the ideas and career paths of individuals dedicated to sarcoma research and investigation, SARC fosters the development of new and innovative research.”

JONATHAN FLETCHER, MD
Associate Professor of Pathology and Pediatrics, Harvard Medical School

SARC BOARD MEMBER

SARC Career Development Program

The SARC Career Development program was established in 2009 to help prepare the next generation of investigators. Career Development Awards are awarded in the amount of \$100,000 for one year. A second year of funding is dependent upon progress. The goal of this level of funding is to ensure protected time for junior investigators to establish their sarcoma-focused research careers. Since the award’s inception, we have provided funding support to 22 investigators who have gone on to become leaders in the field of sarcoma research. Three of this outstanding group of awardees are pictured below. To see all of the recipients please visit the SARC Career Development web page.

Dr. Fletcher (left) has been a member of the Career Development Award Committee since its inception. He has served in a mentoring role to several SARC career development awardees, inspiring young scientists who are committed to sarcoma research. Dr. Patrick Grohar (below), a 2010 Career Development award recipient, acknowledges that the award provides financial support, encouragement, and a vote of confidence. “The award provided me an opportunity to establish an independent lab focused on sarcoma and to work toward new therapies,” Dr. Grohar said.



PATRICK J. GROHAR, MD, PHD
Associate Professor of Pediatrics, University of Pennsylvania

2010 AWARD RECIPIENT

Developing small molecule therapies and clinically translating novel EWS-FLI1 targeted therapies.



LARA E. DAVIS, MD
Assistant Professor, Oregon Health & Science University

2016 AWARD RECIPIENT

Studying CDK4 inhibition in a molecularly-selected population of sarcomas.



INGA-MARIE SCHAEFER, MD
Instructor, Department of Pathology, Brigham and Women’s Hospital, Harvard Medical School

2018 AWARD RECIPIENT

Clinical Relevance of Cell Cycle Aberrations in GIST Progression.

We're an organization that is known for welcoming opportunities to explore working partnerships and collaboration with researchers, clinical researchers, and teams interested in making progress together to better understand sarcoma and help patients.

Providing Infrastructure and Expertise

SARC is unique in its ability to bring together the network of participants and components necessary to translate and test preclinical findings and bring clinical trials to reality, through an ongoing process of discovery, development, and delivery of better options.

We have worked collaboratively with several partners in academia and industry to explore the potential benefit of new agents. We have designed and conducted trials to assist in developing new treatments. We have conducted phase 1 trials with selected centers, and we have partnered on pilot phase 2 studies as well as registration phase 3 studies for FDA approval.

Additionally, we are able to collaborate with contract cooperative networks, bringing to the table our unique insights, experience, and access to the SARC network of expertise, data, and models.

SARC maintains a connection to the National Cancer Institute. Dr. Widemann (right) serves as the National Cancer Institute's liaison to the SARC Board of Directors and is an instrumental partner in leading collaborations with the Neurofibromatosis Consortium of the U.S. Department of Defense. Collaboratively, we have successfully been awarded four Department of Defense grants to study sarcomas, including malignant peripheral nerve sheath tumors that can occur in people with neurofibromatosis.

Recently SARC joined the NCI MyPART (My Pediatric and Adult Rare Tumor) Network as an advocacy partner helping advance the MyPART mission to engage patients, families, physicians, researchers, and advocates in cancer research.



“Every step forward, every success, is the result of our sarcoma community working together.”

BRIGITTE C. WIDEMANN, MD
Chief, Pediatric Oncology Branch;
Acting Director, Immune Deficiency Cellular Therapy Program; Head, Pharmacology & Experimental Therapeutics Section; Senior Investigator; Deputy Director; Center for Cancer Research, National Cancer Institute

NCI LIAISON TO THE SARC BOARD

Sarcoma advocacy is integral to the mission of SARC and to our community. Together, we're working to help build awareness of sarcoma and the resources that are available to help patients and their families.



“The people getting involved in advocacy have a real interest in science and have the motivation to make something meaningful out of their experience.”

JANE PERLMUTTER, PHD
Cancer Survivor and Active Advocate

SARC BOARD MEMBER

The Sarcoma Coalition Is Launched

In 2017, SARC played a pivotal role in helping form the Sarcoma Coalition – a coalition of sarcoma advocacy groups from around the country working toward a common purpose – to provide resources and support for those dealing with sarcoma, from patients and their families to our allies in the research community. Sarcoma Coalition member organizations recognize that collaboration and cooperation make their individual efforts stronger.

Research Advocacy Council

SARC established the Research Advocacy Council in October 2019. The purpose in forming this group is to engage the sarcoma advocacy community and ensure the inclusion of the perspectives of the patient and family as sarcoma research is planned. Using the model established by the National Cancer Institute, SARC has established a sarcoma-focused council to work side-by-side with researchers to provide the patient voice when appropriate and to advise, review, and disseminate research information. SARC is committed to engaging the council in our research planning and to providing training to the members.

Advocates Working Together

Pediatric sarcoma survivor Peter Fantozzi (lower right) learned of the SARC Research Advocacy Council through his involvement with the Northwest Sarcoma Foundation, a member of the Sarcoma Coalition. He attended the Council's inaugural 2019 meeting in Detroit. There, long-term cancer survivor and research advocate Dr. Perlmutter (upper left) presented a workshop to guide sarcoma advocates on how to advocate for research. Her goal is to ensure that advocates have a good understanding of the research process and their potential role in it. “The council provides a platform to share our perspectives and learn from the people who know this field best,” Fantozzi said.



ANNIE ACHEE
Sarcoma Coalition Steering Committee Member



LISA DEYOUNG
Sarcoma Coalition Steering Committee Member



PETER FANTOZZI
SARC Research Advocacy Council Member

SARC fosters collaboration and unity among the sarcoma research community, bringing together individuals and organizations committed to the discovery, development and delivery of new treatments for sarcoma.

Together we can improve outcomes and change the future.

JOIN US

The mission of SARC is advanced through a broad range of supporters – friends, family and sarcoma patients, as well as healthcare professionals and organizations sharing our vision to find a cure for sarcoma. **All are welcome.**

Membership

Open to all interested in working together to make progress to improve outcomes for patients with sarcoma. We welcome you to attend our semiannual meetings, submit ideas for research, and collaborate on research process.

sarctrials.org/join-sarc

Meetings

Our semiannual meetings present opportunities for our members to learn about the latest developments in sarcoma research and to network with colleagues. SARC investigators meet as a group twice per year. In spring we meet during the American Society of Clinical Oncology (ASCO) annual meeting. In fall we meet at the Connective Tissue Oncology Society (CTOS) annual meeting.

sarctrials.org/attend-an-event

Donate

We appreciate your support of this important cause and invite you to discuss making SARC a part of your gift-giving plans.

sarctrials.org/donate

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President and CEO
Effective October 1, 2020

DENISE REINKE*
Past President
Effective October 1, 2020

AMY ALBERT
Executive Assistant

ERIN PEREGRINE ANTALIS
Research Manager

CARRIE BERGMANS
Research Project Manager

KIMBERLY DOOLEY
Research Project Manager

CLAUDIA HILL
Operations and Grants Manager

ERIN KOZLOWSKI
Research Project Manager

GARY MUNCK
Finance Manager

LORI SMITH
Staff Accountant

**Beginning in 2021 Denise will retain a role with SARC as a research manager, continuing her work on NIH-funded grants, projects, and patient advocacy.*



SARC
collaborating to cure sarcoma

SARC

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