

Advocacy Priorities

It is estimated that over 17,000 people will be diagnosed and over 7,000 people will die from sarcoma in 2024. Sarcoma accounts for fifteen to twenty percent of cancer diagnoses in people below the age of 18, making sarcoma among the top five causes of cancer deaths in children under 18 years old in the U.S.¹

Because it is a rare cancer, representing approximately 1% of adult cancers, sarcoma is often left behind. While advancements continue to be made in other cancer types, most of the over 70 subtypes of sarcoma do not have an FDA approved therapy and patients are being treated with therapies that are thirty-years old and not indicated for their diagnosis. This leads to poor outcomes and a sixteen percent survival rate for patients with metastatic disease. In addition, the rarity of the disease poses challenges for diagnosis. Due to these challenges, it can take many months to years for patients to receive a sarcoma diagnosis, causing many patients to be diagnosed at the metastatic stage.

People diagnosed and living with sarcoma face unique challenges.

SFA and the sarcoma community address these challenges with the following priorities.

Priorities:

1. Research-related issues: increased funding, updated drug approval process, regulations, and guidance
2. Access to existing treatments and quality patient care; sarcoma patient financial toxicity

Research-related issues: increased funding, updated drug approval process, regulations, and guidance

There is tremendous unmet medical need for sarcoma. Sarcoma is sometimes curable by surgery (about 20 percent of cases) or by surgery with chemotherapy and/or radiation (cure rate of 50-55 percent), but about half the time they are totally resistant to all of these approaches—thus the extreme need for new therapeutic approaches.

The low patient population is a deterrent to pharmaceutical investment because it is difficult to collect enough patient data to meet FDA regulations and criteria. While legislation has been enacted to address this issue, it does not go far enough. We must think differently about data necessary for drug approvals. Integrating patient experience data, patient reported outcomes and real-world data with clinical data can accelerate advancements in a rare cancer like sarcoma. Otherwise for sarcoma patients, it will be decades before they see any change.

Research funding lags significantly behind compared to other cancers. An example from the Department of Defense CDMRP Program provides a robust example. A recent report from the American Cancer

¹ <https://cancerstatisticscenter.cancer.org/>; <https://www.cancer.org/cancer/types/soft-tissue-sarcoma/about/key-statistics.html>; <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC6497437/>

Research-related issues cont.

Society (ACS) found that one in five cancers diagnosed in the United States (U.S.) is a rare cancer, which is defined as an incidence of fewer than six cases per 100,000 people per year. By this definition, around 200 forms of rare cancer compose around 20%-25% of all U.S. cancer diagnoses, which affect more than 400,000 Americans per year.² The number of patients affected by rare cancers is similar to the number affected by breast cancer, yet people diagnosed with a rare cancer like sarcoma do not receive the same level of support. Currently the DOD CDMRP program provides research funding of \$140 million for breast cancer compared to just \$17.5 million in funding for rare cancers.

Ask: Increase research investments in sarcoma so we can save lives.

Access to existing treatments and quality patient care

Because most community clinicians see very few sarcoma patients and may never encounter a sarcoma patient at any time throughout their career, these clinicians will not have expertise or even knowledge of treating sarcoma with the latest standard of care or latest research. Nor will they have the most up-to-date information about clinical trials.

Receiving care at a designated sarcoma specialty center increases patient outcomes and improves quality of life. Patients have access to the latest standard of care, research, and clinical trials. Since many subtypes of sarcoma do not have an FDA-approved treatment, clinical trials become an important treatment option for patients. Yet approximately 80 percent of the people diagnosed with sarcoma are diagnosed and treated in the community setting. There are many reasons patients do not seek treatment at a sarcoma specialty center. Primary among them are proximity to a specialized center, socioeconomic reasons including the inability to take time off from work, and patients lack of or have limited insurance.

Expanding telehealth options for people with sarcoma and other rare cancers can go a long way in changing outcomes and providing access to treatment otherwise unavailable to most. Rather than encountering barriers to care due to an inability to seek treatment at a sarcoma specialty center, patients can be treated in the community with oversight from a sarcoma specialist.

It can be difficult for sarcoma patients to access the latest treatments. Notwithstanding the high cost of therapies, insurance companies often consider treatments experimental and limit patient access. Rare diseases must be covered by insurance as we work to realize new treatment options, especially when treatment options are limited or ineffective. Legislation has included coverage for clinical trials through Medicare and Medicaid, but sarcoma patients using private health insurance continue to suffer. In a rare cancer like sarcoma, with limited treatment options, clinical trials become a patient's only hope.

Moreover, the future is precision oncology. Cancers will no longer be treated by their specific type but by their tumor makeup. This will involve many off-label treatment options. Drug repurposing can be very effective in accelerating treatment options for patients diagnosed with a rare cancer like sarcoma. Currently, patients cannot get coverage for off-label treatments even if data shows that treatment can be effective.

Patients continue to choose between a life-saving treatments or living life. Not unique to sarcoma, but sarcoma patients suffer incongruently due to the barriers they face simply because they have been diagnosed with a rare cancer.

Ask: Advance telehealth options and access to treatments to remove barriers for improved outcomes.

¹ <https://cdmrp.health.mil/rcrp/default>