

SCIENTIFIC ROUNDTABLE FOCUSES EFFORTS ON LEIOMYOSARCOMA RESEARCH

Research clinicians from the US and abroad participated in a scientific research roundtable this past September to establish the most important issues facing leiomyosarcoma (LMS) research and clinical trials. The workshop, expected to be an annual event, is a joint effort of the National Leiomyosarcoma Foundation (NLMSF) and Sarcoma Patients EuroNet (SPAEN).

The roundtable's mission is to bring together sarcoma experts for a meeting dedicated to LMS, where participants discuss the present state of LMS and the continued challenges of diagnosis and treatment. Its goal is to develop working plans to close the gaps in LMS patient care and improve LMS patient-care protocols. They also advise the NLMSF on worthy and important research projects that deserve the foundation's future funding efforts.

Plans for roundtable meetings in 2020 and 2021 are already underway. Between the annual meetings, workgroups continue to take steps toward addressing the issues identified by the roundtable. For more information on the roundtable and NLMSF, visit: <https://nlmsf.org>.

FDA AWARDS GRANT TO STUDY TEMOZOLOMIDE IN GIST

A phase 2 study of temozolomide in gastrointestinal stromal tumors (GIST) received one of the 12 grants awarded in October by the US Food and Drug Administration (FDA) to enhance the development of medical products for patients with rare diseases. Jason Sicklick, MD, and the University of California San Diego in La Jolla will receive \$1.5 million over 3 years to conduct the phase 2 study (NCT03556384). The objective of the study is to determine the efficacy at 6 months of temozolomide therapy in patients with the *SDH*-mutant/deficient subtype. Temozolomide is approved by the FDA to treat newly diagnosed glioblastoma multiforme and refractory anaplastic astrocytomas. It is not approved for the treatment of *SDH*-mutant/deficient GIST.

The FDA awarded the grants through the Orphan Products Clinical Trials Grants Program. Other orphan diseases receiving grants included glomerulopathy, gliomas, Fanconi anemia, sickle cell respiratory complications, Duchenne muscular dystrophy, HPV-associated respiratory papillomatosis, refractory viral infections and T-cell immunodeficiency, oral cancer, retinoblastoma, cerebellar brain tumors, and acute myeloid leukemia.