

A cluster of white medical icons inside hexagonal shapes, including a pill, DNA, atom, brain, heart, cross, syringe, microscope, and test tubes, arranged in a grid-like pattern in the top left corner.

Pediatric and SDH-Deficient GIST Consortium

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New Horizons GIST 2019



The Life Raft
GROUP



History

- NIH Pediatric and Wildtype GIST Clinic
- PAWS GIST UK

- Researchers and clinicians work independently on this rare sub-type of GIST

- Better collaboration is needed to bridge the gap among researchers to learn more about SDH-deficient GIST

Pediatric & SDH-Deficient GIST Research Symposium

- Meeting of leading experts in July 2018 in Miami
GOAL: To gather key clinicians/researchers to identify KIT-independent therapies for targeting SDH-Deficient tumors in order to improve patient outcomes.



Pediatric & SDH-Deficient GIST Research Symposium at Life Fest 2018



Pediatric and SDH-Deficient GIST Consortium



Consortium was officially launched at the Biden Cancer Initiative in September 2018

- The aim of the consortium is to extend and sustain the work of the NIH Clinic and other research efforts, with the ultimate goal of finding a cure through global collaboration.
- Experts and Advocates collaborate and share data and tissue, with the goal of finding successful targeted therapies that can help keep these patients alive.

“We are at an inflection point in the understanding and treatment of cancer and are starting to break down barriers and change the culture in ways that are needed to deliver what patients deserve – a cancer research and care system that puts saving lives above all else. The commitments we have received, including the Life Raft Group’s Pediatric and SDH-Deficient Consortium, bring us closer to developing the right systems, the right culture to get us there.”

- Vice President, Joe Biden



Accomplishments to Date

- Consortium members have met monthly since its inception and discussed global research on SDH-related tumors.
- Members agreed to share tissue and have worked on tissue sharing with several patients to date in the USA and even more in the UK.
- Information from our Patient Registry provoked the creation of an algorithm, which has been fine-tuned by leading pathologists, and will soon be shared (and hopefully) adopted by pathology societies. Once adopted, this can help influence diagnostic decision-making for this population around the world, which we hope will then also influence clinical decision making.
- The Consortium worked on a grant to fund future activities of the group.
- Two webcasts were presented for patients to educate them about SDH.
- Three Virtual GIST Tumor Boards were held to present challenging cases.
- Patient Advocacy groups are collaborating with the sharing of information and supporting each other's efforts.
- One clinical trial has been announced.
- Created Project Surveillance interactive system for physicians

Specific Researcher Highlights

- Dr. Maria Pantaleo, University of Bologna, Italy, presented to the Consortium in October, on three research SDH-related projects she is currently conducting. She will be working with LRG on a data project.
- Dr. Jonathan Trent has opened a HIF2a trial at Sylvester Comprehensive Cancer Center, sharing details with the Consortium in order to evaluate the possibility of SDH-deficient GIST patients as trial candidates.
- Dr. Joshua Schiffman, Huntsman Cancer Institute, is focusing on a project for patients with PGL/PCC tumors (plus any SDH-driven tumors). With the Paraganglioma Preclinical Project, his team is working to test therapies and combinations of drugs to identify what can effectively stop these tumors from growing in mice.
- Dr. Jason Sicklick, UCSD, is focusing on laboratory research with SDH-deficient GIST tissue.
- Drs. Schiffman and Sicklick are actively searching for tissue from recent surgeries and have appealed to GIST patients through the Consortium members and the resources of the LRG.