



Pediatric & SDH-Deficient GIST Consortium Executive Summary

Pediatric & SDH-Deficient GIST Consortium Background

Historically, most researchers with an interest in Pediatric & SDH-Deficient GIST worked independently, with each seeing very few of these rare disease patients. The National Institutes of Health (NIH) Pediatric and Wild-Type GIST clinic was established in 2008 with the support of advocacy organizations, to bring together clinicians and patients to begin to better understand this rare disease. After a successful ten years of this clinic with over 10 peer-reviewed publications describing Pediatric & SDH-deficient GIST, this program is at a crossroads, with no sustainable funding just at a time when no standard of care for this rare disease has been defined.

Because of this need, the Life Raft Group created and convened the inaugural Pediatric & SDH-Deficient GIST Research Symposium on July 14-15, 2018. The goal of the Symposium was to gather key clinicians/researchers to identify KIT-independent therapies for targeting SDH-Deficient tumors in order to improve patient outcomes. Because Pediatric & SDH-Deficient GIST is a rare, small sub-specialty, the number of clinicians and researchers in attendance was specific and targeted. However, it is this small group of accomplished researchers who can make significant advances in the field by sharing their research progress with one another and agreeing to work together to strengthen and advance research on Pediatric & SDH-Deficient GIST.

An outgrowth of the symposium was the formation of the Pediatric & SDH-Deficient GIST Consortium. 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. As patients' survival and quality of life are dramatically impacted by this disease, it is critically urgent that the leading Pediatric & SDH-Deficient GIST experts collaborate and share data and tissue, with the goal of finding successful targeted therapies that can help keep these patients alive.

Commitments

1. Build a Consortium

- Assemble a multi-stakeholder team to form a consortium for the purpose of increasing collaboration to accelerate research and clinical advancement of Pediatric & SDH-deficient GIST tumors by summer of 2018.
- Apply for a U01 NIH research cooperative consortium grant by March 2019.
- Create and utilize a platform where each can share best practices, case reports, data and tissue to answer critical questions by September 2018.
- Create ground rules for consortium membership by September 2018.
- Set up next in-person meeting to discuss accomplishments and next steps by June 2019.
- Develop at least one publication of the consortium by end of 2019 as an outcome of data and tissue sharing.



Commitments (continued)

2. Accelerate Research
 - Create template MTA, CTAs, and universal consents for all institutions to approve for data and tissue transfer by December 2018.
 - Create a tissue bank (paraffin, flash frozen, viably frozen tissues and other potential tissue sources) by October 2018.
 - Create the first Pediatric GIST Global Tissue Directory by end of 2018.
 - Create the first Pediatric GIST cell line Directory by end of 2018.
 - Explore methylation of ctDNA by January 2019.
3. Create Guidelines
 - Create guidelines for the following groups by end of 2019: medical oncologists, pediatric oncologists, surgical societies, pathologists.
 - Create screening guidelines by end of 2019 for:
 1. SDH-deficient patients who are NED
 2. Affected individuals with no tumors yet
 - Ensure appropriate access to these guidelines throughout key professional and scientific organizations: ASCO, ASPHO, AACR, ACP, NCI and others.
4. Create and Expand Clinical Trials
 - Throughout 2018-2019, develop natural history benchmark to be used as an endpoint.
 - Expand trial sites for TMZ trial for an R01 proposal by Aug 2018.
 - Expand sites for guadecitabine trial in early 2019.
 - Create a PARP inhibitor study for SDH-deficient tumors in 2019.
5. Involve Patient Advocacy Groups
 - Create SDH Deficient GIST Expert Database by end of 2018.
 - Create white paper on NIH Pediatric and Wildtype GIST Clinic by end of 2018.
 - Conduct Virtual GIST Tumor Boards to also serve as training for community oncologists (1 in 2018 and 2 in 2019).
 - Conduct educational webinars for families (2 in 2018 and 3 in 2019).

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