GIST Update: NIH

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At the NIH: Clinical Care

Pathology Review
Markku Miettinen, M.D., Ph.D.

GIST Medical Oncology
John Glod, M.D., Ph.D.

Paraganglioma, Pheochromocytoma Medical Care
Karel Pacak, M.D., Ph.D.

GIST, Paraganglioma, Pheochromocytoma Surgical Care
Andrew Blakely, M.D.
Jonathan Hernandez, M.D.
Naris Nilubol, M.D.
At the NIH: Research Initiatives

- **SDHC Epimutant Biology**
  - Paul Meltzer, M.D., Ph.D.
  - R. Taylor Sundby, M.D.

- **In vitro GIST models**
  - Jonathan Hernandez, M.D.
  - Andrew Blakely, M.D.

- **Circulating Biomarkers of SDH-Deficient GIST**
  - Jack Shern, M.D.
  - Naomi Taylor, M.D., Ph.D.

- **SDH-Deficient GIST Metabolism**
  - Karlyne M. Reilly, Ph.D.
  - Francesco Tomassoni, Ph.D.

- **Mouse Models of SDH-Deficient GIST**
  - Lino Tessarollo, Ph.D.
At the NIH: Pediatric & Wild-Type GIST Clinic

• Connects patients, caregivers, and experts to:
  • Review medical records and imaging
  • Provide recommendations regarding management and clinical trials

• To be held this year, September 14-16

BJ Thomas, RN
ncipediatricgist@mail.nih.gov
At the NIH: Clinical Trials

- Pediatric Oncology Branch: NCT03739827
- Developmental Therapeutics Clinic: NCT04595747
- Surgical Oncology Program: NCT04557969
Clinical Trial NCT03739827:
Natural History and Biospecimen Acquisition for Children and Adults with Rare Solid Tumors

PI: Mary Frances Wedekind Malone, D.O.

Open, Recruiting
MyPART: My Pediatric and Adult Rare Tumor Network

- Focusing on rare solid tumors affecting children, teens, and young adults (<39 yo), while studying all ages in tumors that occur across age groups
- Engaging patients, family members, advocates, clinicians, scientists, healthcare providers as partners in research on rare tumors
- Collecting longitudinal molecular, clinical, and patient reported outcome data through the Natural History Study of Rare Solid Tumors (NCT03739827)
- Holding workshops and symposia on rare tumors to develop expert consensus on research priorities
- Hosting multi-day clinics for rare tumors to bring patients and nationwide experts together
- Building a multi-institutional network of sites to collaborate on data collection
MyPART Update

• Thus far, 91 patients with GIST enrolled
Clinical Trial NCT04595747:
Testing the Anti-cancer Drug, Rogaratinib (BAY 1163877), for Treatment of Advanced Sarcoma with Alteration in Fibroblast Growth Factor Receptor (FGFR 1-4), and in Patients with SDH-Deficient Gastrointestinal Stromal Tumor (GIST)

Pl: Suzanne George, M.D.; Alice Chen, M.D.

Open, Recruiting
Developmental Therapeutics Clinic

Geraldine O’Sullivan Coyne, M.D., Ph.D.

Alice Chen, M.D.

CCR referral coordinator at 1-888-NCI-1937 (1-888-624-1937) or the Patient Recruitment and Liaison Office at 1-800-411-1222
Clinical Trial NCT04557969: 
Prospective Study of Surgery in Gastrointestinal Stromal Tumors (GISTs) for Treatment, Tumor Modeling, and Genomic Analysis

PI: Andrew M. Blakely, M.D.

Open, Recruiting
Clinical Problem

• Surgery is only curative for localized disease
  • Selected patients benefit from cytoreduction

• Systemic therapy options are limited
  • Poor response rates and/or high toxicity

• Novel therapeutic options are needed
  • WT and treatment-refractory non-WT GIST

At the NIH

- SMART system: Sustained Microenvironment Analysis of Resected Tissue
  - Keeps tumor tissue alive for prolonged analysis
  - Preserves tumor microenvironment
  - Uses patients’ own serum to perfuse
  - Allows introduction of potential therapies to evaluate response

- Cell line, PDX, and organoid development

SMART system analysis of WT GIST tissue exposed to decitabine
Study Objectives

• Obtain fresh GIST tumor tissue for translational research analyses and to contribute to ongoing tumor banking efforts

• Characterize genomic features of GISTs

• Assess disease-free intervals between surgical resections

• Develop primary cell cultures for drug screening assays

• Correlate drug screen results with ex vivo response in SMART system
Study Update

• Thus far, 27 patients accrued
  • 20 patients have undergone or will undergo surgery at NIH
  • 7 patients on active surveillance

• Upcoming collaboration with UCSD and LifeRaft
  • Expanding tumor banking efforts
  • Enhancing *ex vivo* tumor modeling
Contact Information

Referral Contact: Yvonne Mallory, RN
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Study Coordinator: Audra Satterwhite, RN
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