GIST Update: NIH

Andrew M. Blakely, MD
Assistant Research Physician
Surgical Oncology Program
National Cancer Institute, NIH

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.

NIH NATIONAL CANCER INSTITUTE

At the NIH: Research Initiatives

SDHC Epimutant Biology

Paul Meltzer, M.D., Ph.D.

R. Taylor Sundby, M.D.

In vitro GIST models

John Glod, M.D., PhD.

Andrew Blakely, M.D.

Jonathan Hernandez, M.D.

Circulating Biomarkers of SDH-Deficient GIST

Jack Shern, M.D.

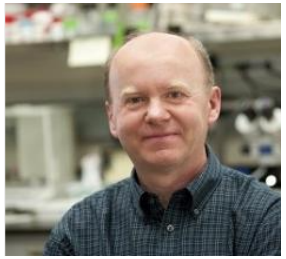
SDH-Deficient GIST Metabolism

Naomi Taylor, M.D., Ph.D.

Mouse Models of SDH-Deficient GIST

Karlyne M. Reilly, Ph.D.

Francesco Tomassoni, Ph.D.



Lino Tessarollo, Ph.D.

NIH NATIONAL CANCER INSTITUTE

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

NIH NATIONAL CANCER INSTITUTE

At the NIH: Clinical Trials

- Pediatric Oncology Branch: NCT03739827
- Developmental Therapeutics Clinic: NCT04595747
- Surgical Oncology Program: NCT04557969

NIH NATIONAL CANCER INSTITUTE

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

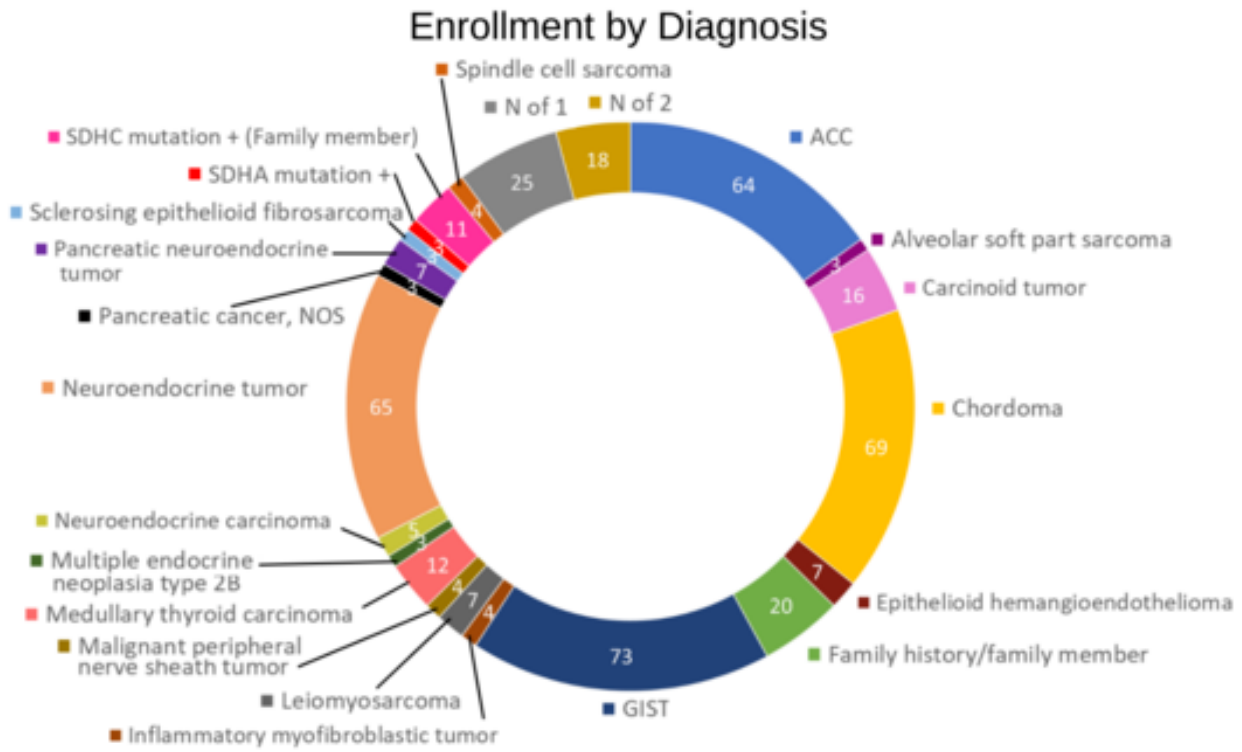
MyPART: My Pediatric and Adult Rare Tumor Network

- 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

NIH NATIONAL CANCER INSTITUTE

MyPART Update

- Thus far, 91 patients with GIST enrolled



NCICCRRareTumors@mail.nih.gov

NIH NATIONAL CANCER INSTITUTE

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)

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

Open, Recruiting

NIH

NATIONAL® CANCER INSTITUTE

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

NIH NATIONAL CANCER INSTITUTE

Clinical Trial NCT04557969:

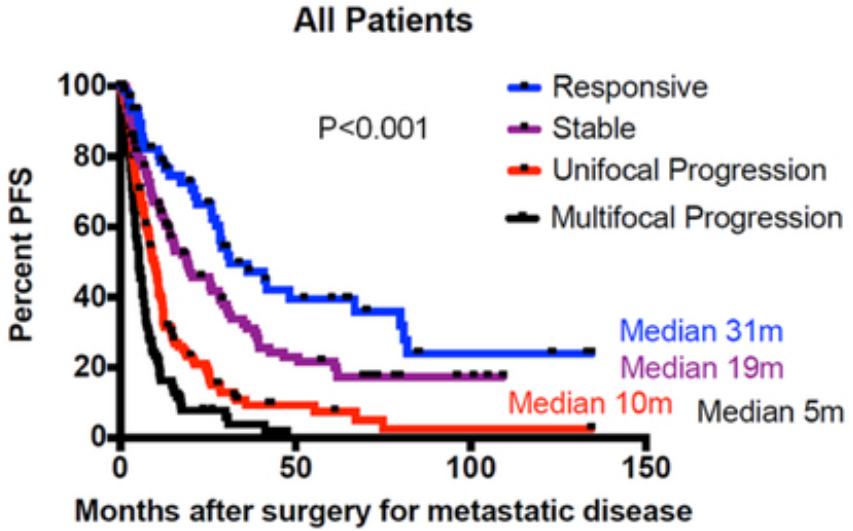
Prospective Study of Surgery in Gastrointestinal Stromal Tumors (GISTs) for Treatment, Tumor Modeling, and Genomic Analysis

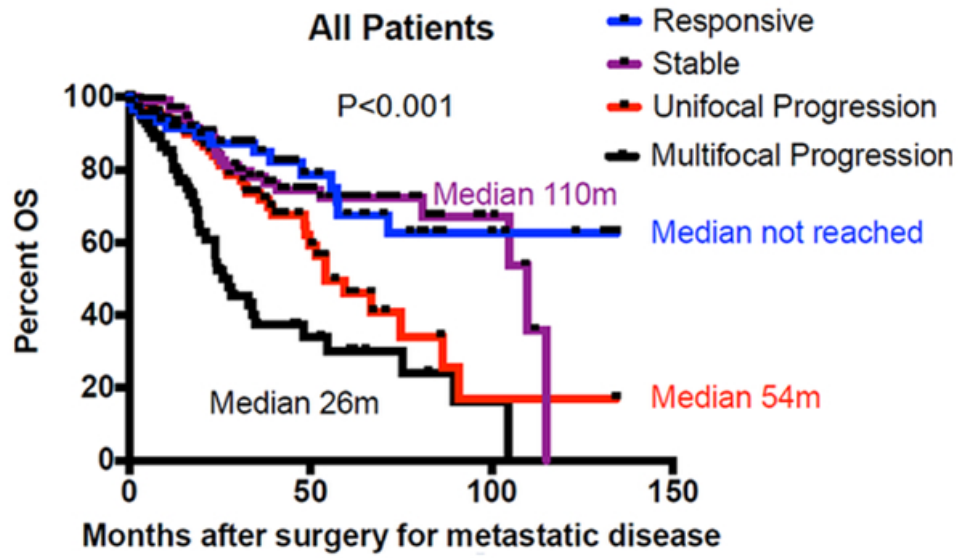
PI: Andrew M. Blakely, M.D.

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





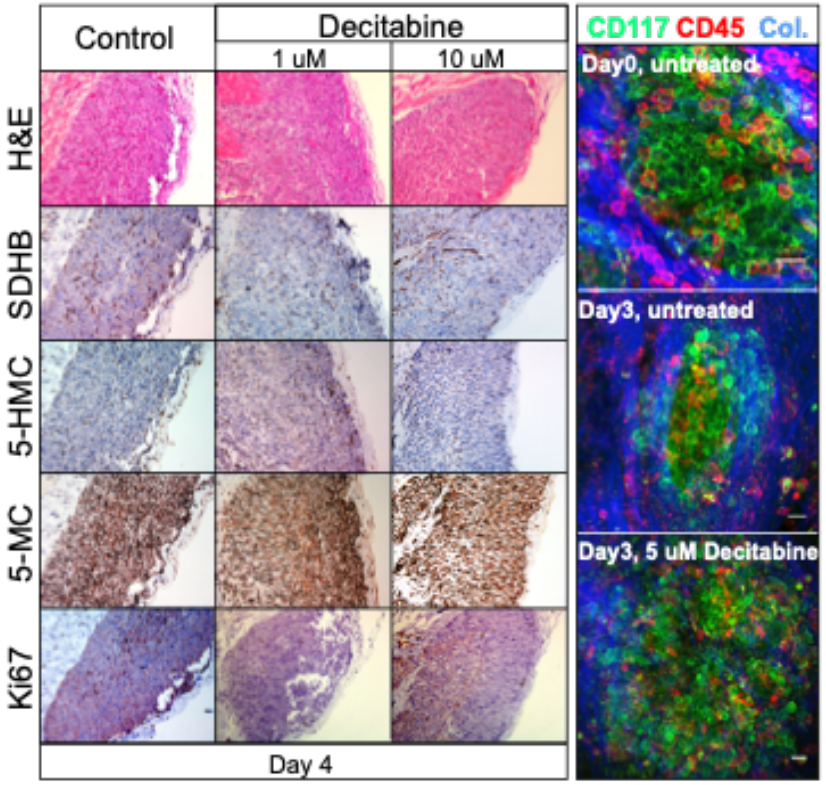
NIH NATIONAL CANCER INSTITUTE

Fairweather, et al. *Ann Surg* 2018.

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

NIH NATIONAL CANCER INSTITUTE

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

NIH NATIONAL CANCER INSTITUTE

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

NIH NATIONAL CANCER INSTITUTE

Contact Information

Referral Contact: Yvonne Mallory, RN

yvonne.mallory@nih.gov

Study Coordinator: Audra Satterwhite, RN

audra.satterwhite@nih.gov

NIH NATIONAL CANCER INSTITUTE