Paediatric Adolescent Wild type & Syndromic (PAWS) GIST Clinic
United Kingdom

Ramesh Bulusu
Consultant Oncologist
Lead Clinician for PAWS GIST Clinic
PAWS-GIST

- Paediatric
- Adolescent
- Wild-type
- Syndromic
  - **SDH GISTs**
  - Neurofibromatosis 1 GISTs

- PAWS-GIST – idea born in a pub in
- Oxford in 2011
- Patient & Carer led initiative
Register for the Next Clinic

Title: ___________________ First Name: ___________________ Surname: ___________________

Address: ___________________ City: ___________________ Postcode: ___________________

Telephone: ___________________ Mobile: ___________________

Email: ___________________

NHS No: ___________________ DOB: ___________________

Age at Diagnosis: _______ Year diagnosed: _______

Primary Tumour location: ___________________ GIST tumour type: Wildtype Unknown

Hospital: ___________________

Oncologist Name: ___________________ Email address: ___________________ Tel No: ___________________

Can we contact oncologist?: ☐ Yes ☐ No

Surgeon Name: ___________________ Email address: ___________________ Tel No: ___________________

Can we contact surgeon?: ☐ Yes ☐ No

I am happy for my details to be shared with the Patient Director of the PAWS-GIST Initiative: ☐ Yes ☐ No

Submit details
PAWS GIST-Clinical data

• 12 Clinics so far
• 70 pts
• Male:Female  23:47
• Median age 38 yrs Range 14-76 yrs
• Heterogeneity is the hallmark
  – SDH Deficient
  – Quadruple Negative
  – NF1 GISTs
  – KIT mutations !
Research Focus in PAWS GIST

• Collaboration
• Innovation
  – Think differently
  – Think outside the box
  – Tailor the treatment to the patient
• GIST Tumour bank established for researchers
• Active engagement with pharma
• Two trials at NIH—Aim to bring in to UK
• Regional, National, International cooperation
SDH Deficient GIST patients

• A Phase II Trial of the DNA Methyl Transferase Inhibitor, Guadecitabine (SGI-110), in Children and Adults with Wild Type GIST, Renal, Pheochromocytoma and Paraganglioma associated With Succinate Dehydrogenase Deficiency
• ClinicalTrials.gov Identifier: NCT03165721
• Principal Investigator: John W Glod, M.D. (NCI)
• Estimated Enrolment: 70 participants
• Allocation: Non-Randomized
• Masking: None (Open Label)
• Subcutaneous inj 45mg/m2/day x 5 days on a 28-day cycle.
NF-1 GISTS

- Oral MEK 1&2 Inhibitor Selumetinib in Neurofibromatosis Type 1 (NF1) Mutated Gastrointestinal Stromal Tumors (GIST)
- ClinicalTrials.gov Identifier: NCT03109301
- Principal Investigator: Brigitte C Widemann, M.D. (NCI)
- Estimated Enrollment: 20 participants
- Allocation: Non-Randomized
- Masking: None (Open Label)
- Oral tablets 50 mg twice daily continuous daily dosing