

Challenges and questions in, Wild-type / Paediatric GIST



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Challenges and questions in, Wild-type / Paediatric GIST



- **Why have I got this?** *(Nobody knows)*
- **What caused it?** *(Nobody knows)*
- **What will treat / cure it?** *(Nobody knows)*
- **Who specialises in treating it?** *(NIH & PAWS-GIST Clinic)*
- **Who is doing research?** *(NIH, PAWS-GIST, members of SDH consortium &?).*

NEW HORIZONS GIST

SEPTEMBER 5 – 7, 2018 – VIENNA, AUSTRIA

The challenges:

- **Ultra-rare cancer** - *not well known*
- **Small patient numbers** – *no big pharma attention*
- **Lack of infrastructure** *to support research*
- **Lack of funding** *to support research*
- **Lack of dedicated researchers**
- **Lack of awareness** *amongst medical community*
- **Lack of awareness** *amongst general public.*
- **Doctors focusing on this disease do it as one small element of their work rather than full time.** *Everything takes longer than it would do if it had a dedicated team.*
- **Not all GIST patients have mutational testing**
- **Mutational testing rarely tests beyond KIT/PDGFRA**
e.g. SDH deficiency testing.



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To-date paediatric/wild-type GIST research has relied upon a relatively small number of compassionate & dedicated medical professionals & volunteer patient advocates who are directly impacted by the disease.

the NIH Pediatric & Wildtype GIST Clinic



PAWS - GIST

Improving treatment & finding a cure for rare

GIST cancer
in young people

gust
PAWS-GIST™ Supported by: GIST Support UK Charity NO. 1129219

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e.g. From a standing start in
2010 PAWS-GIST now has:



- **X3 clinics per annum** (*likely to increase to x4 p.a.*)
- **P/WT GIST Patient Data**
- **A small collection of P/WT tissue in the National GIST Tissue Bank**
- **X 4 research projects underway**

This is what has been achieved in 8 years.

(During this time 11% of the patients who have attended the clinic have died, we need effective treatments urgently).

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We do not yet have:

- **A full time medical, research or administrative team who focus only on PAWS-GIST**
- **Sufficient funds**
- **Clinical trials or effective treatments**
- **Answers to the above questions**

Very recent work (July 2018) by LRG & PAWS-GIST has brought together clinicians who are interested in working as a consortium focusing on SDH deficient GIST.



NEW HORIZONS GIST

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So how can you help?

- i. Ensure that **ALL P/WT operations result in fresh tissue being donated to a relevant tissue bank** and to researchers who are able to grow the cells. (*we need to ID where they are and who to contact*).
- ii. **Reach out and attract researchers**
- iii. Find **pharma companies willing to allow use of their drugs in P/WT GIST patients clinical trials.**
- iv. Find significant **sources of funding** to sustain the clinics and the teams needed to make them work.
- v. Work to ensure **all GIST patients have mutational testing** and that the **testing goes beyond KIT/PDGFRA e.g. SDH testing.**
- vi. **Other...**

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Are these things possible in your country?

What other suggestions do you have?