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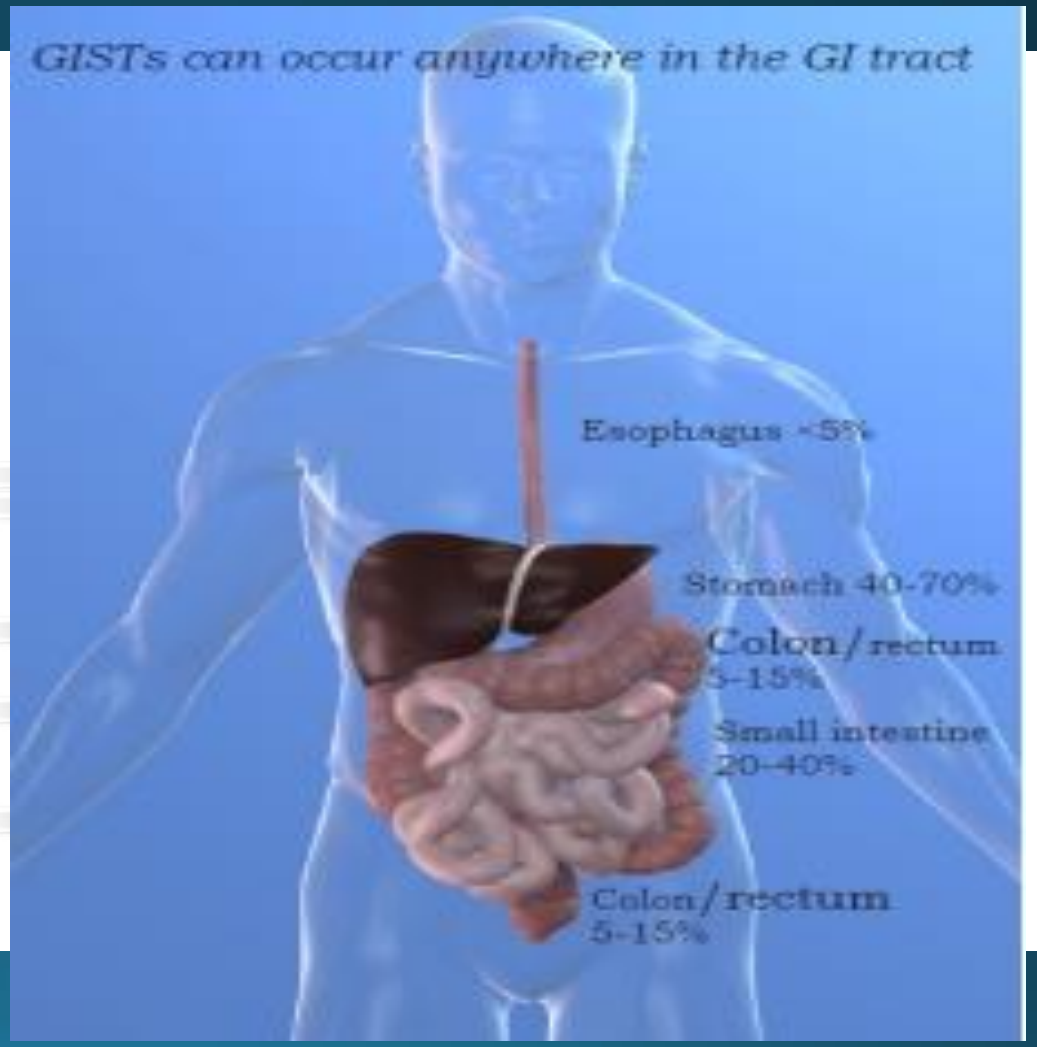
Research • Patient Support & Education • Advocacy

Understanding GIST

- Rare cancer: 4000-6000 US cases per year
- Occur anywhere in the GI Tract
- 80% mutations are in the KIT gene
  - Remaining 20% consists of PDGFRa and Wildtype
- Surgery and approved targeted drug therapy



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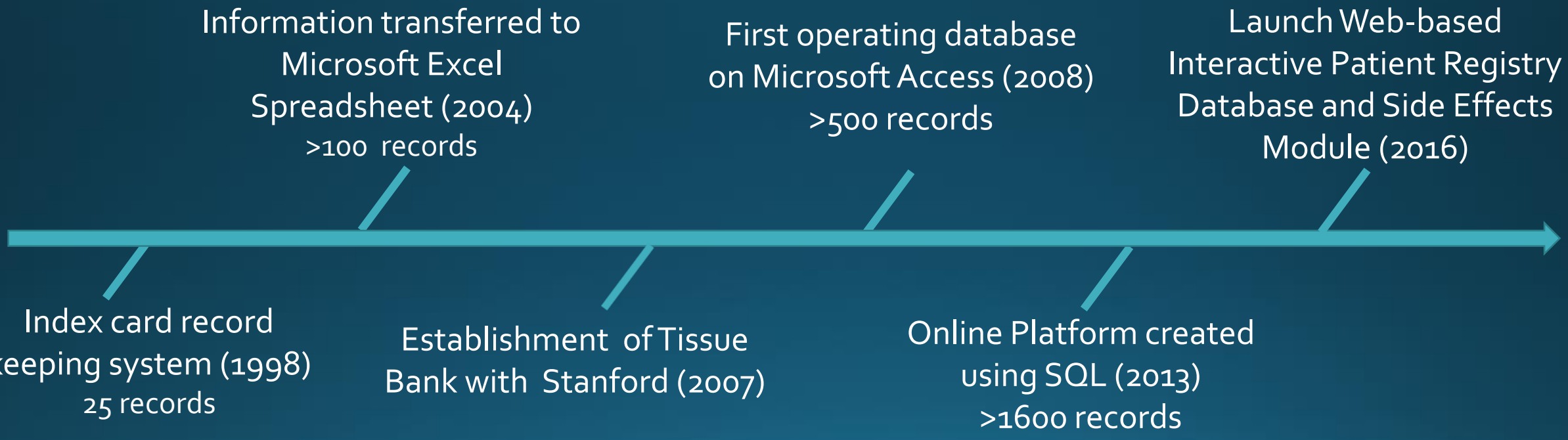
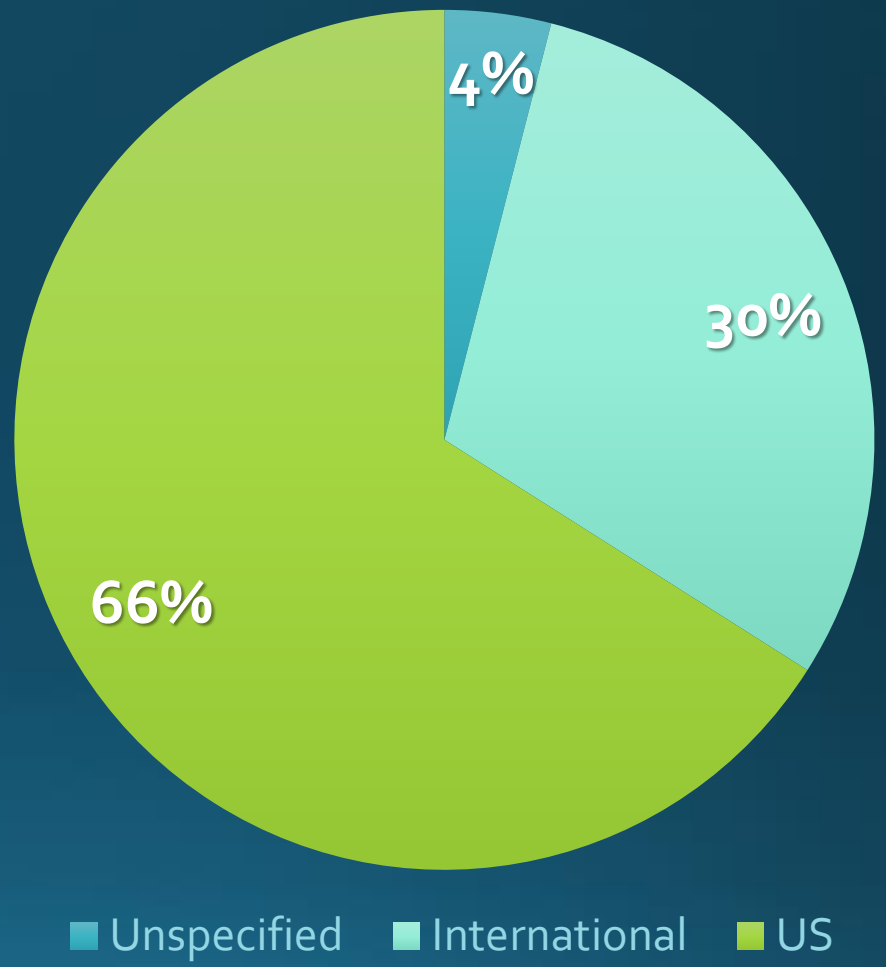
LRG Patient Registry

Initial Development of the Patient Registry



Registry Population

- More than 1600 participants globally
- Over 14 years of self-reported data collection encompassing 35 years of patient history
- Ages 5 - 82 representing 12 mutational types



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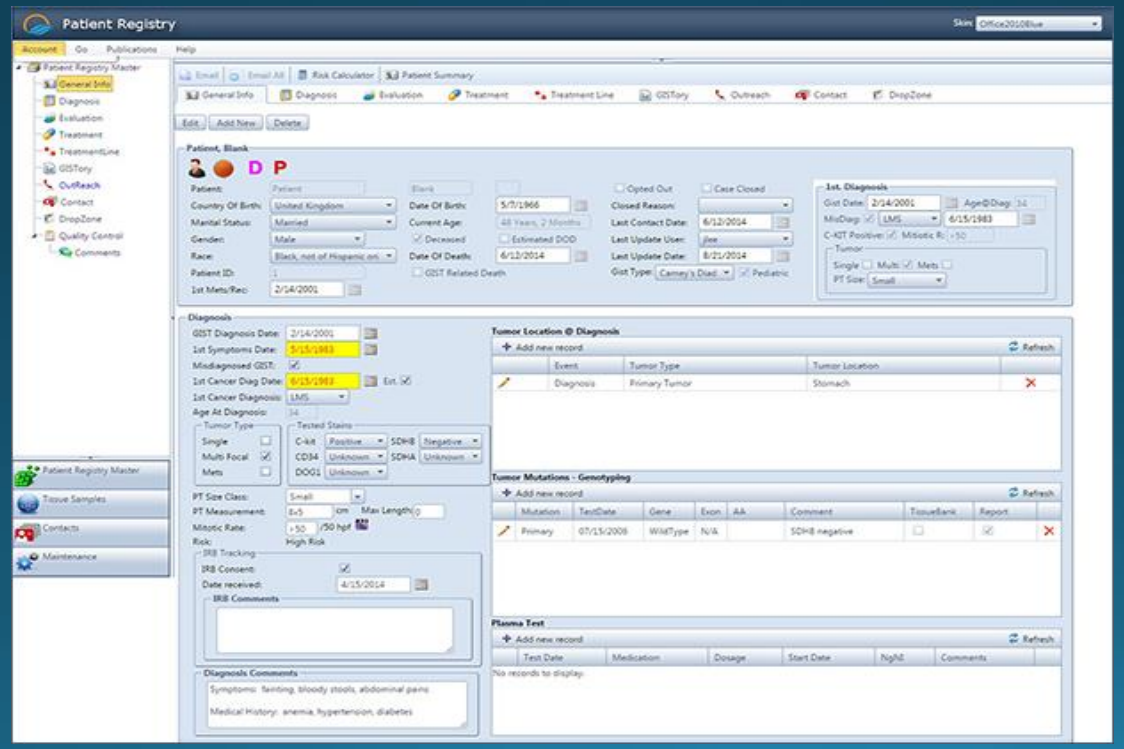
Data Collected

- Demographics
- Diagnosis events
- Evaluations/Scan reports
- Treatments and Side effects

Treatment Patterns
Treatment Outcomes
Better Disease Management



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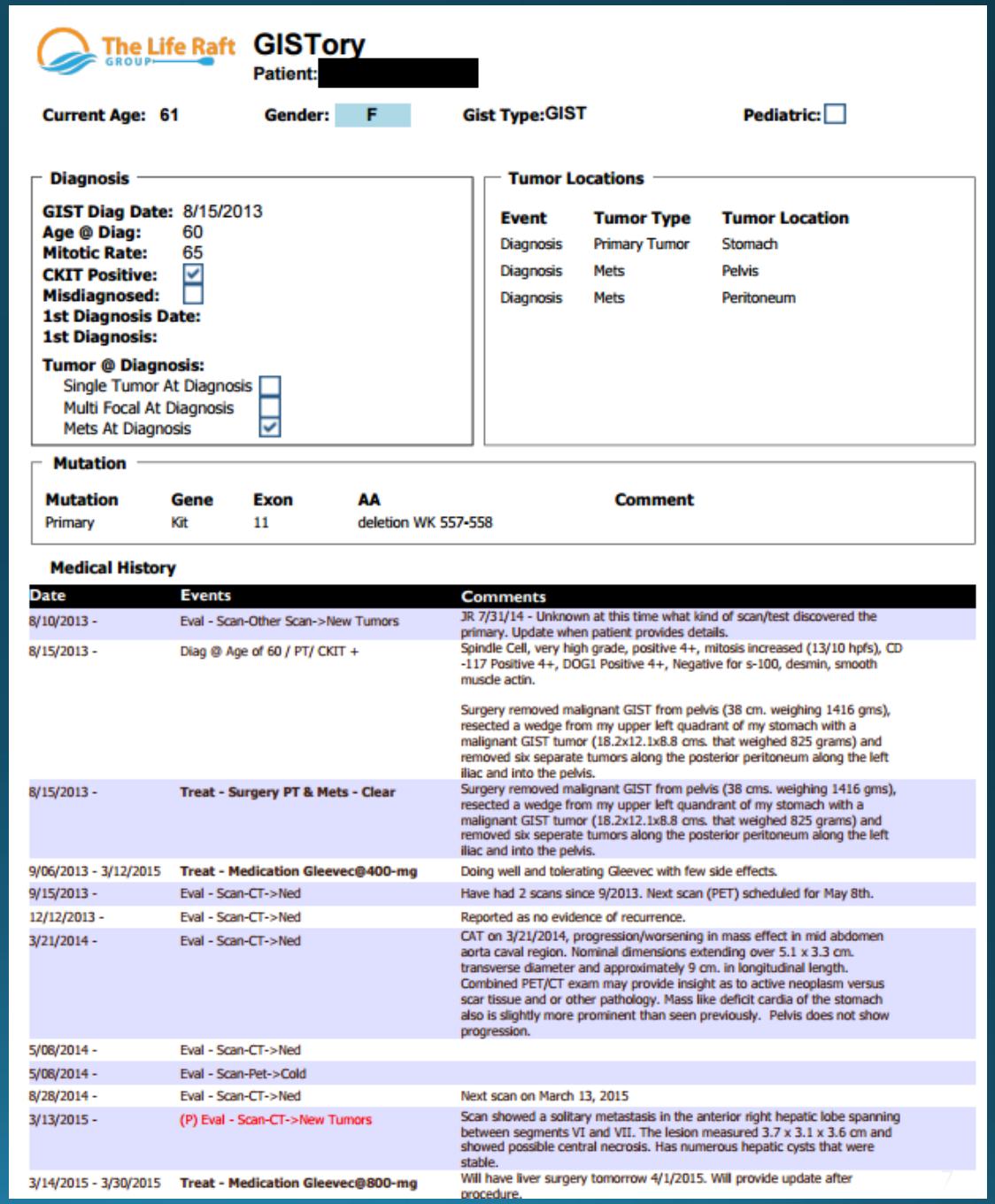
Benefits of Membership

- GISTory
  - Portable medical history
  - Tracks patient history across institutional boundaries
- Patient Support
  - Local support groups
  - Email community
  - Virtual Tumor Board
- Education
  - Expert Patient Course
  - Advocacy Training
- Research



The Life Raft GROUP **GISTory**

Patient: [redacted]

Current Age: 61 **Gender:** F **Gist Type:** GIST **Pediatric:** ☐

Diagnosis

GIST Diag Date: 8/15/2013
Age @ Diag: 60
Mitotic Rate: 65
CKIT Positive: ☑
Misdiagnosed: ☐
1st Diagnosis Date:
1st Diagnosis:
Tumor @ Diagnosis:
Single Tumor At Diagnosis ☐
Multi Focal At Diagnosis ☐
Mets At Diagnosis ☑

Tumor Locations

Event	Tumor Type	Tumor Location
Diagnosis	Primary Tumor	Stomach
Diagnosis	Mets	Pelvis
Diagnosis	Mets	Peritoneum

Mutation

Mutation	Gene	Exon	AA	Comment
Primary	Kit	11	deletion WK 557-558	

Medical History

Date	Events	Comments
8/10/2013 -	Eval - Scan-Other Scan->New Tumors	JR 7/31/14 - Unknown at this time what kind of scan/test discovered the primary. Update when patient provides details.
8/15/2013 -	Diag @ Age of 60 / PT/ CKIT +	Spindle Cell, very high grade, positive 4+, mitosis increased (13/10 hpfs), CD -117 Positive 4+, DOG1 Positive 4+, Negative for s-100, desmin, smooth muscle actin. Surgery removed malignant GIST from pelvis (38 cm. weighing 1416 gms), resected a wedge from my upper left quadrant of my stomach with a malignant GIST tumor (18.2x12.1x8.8 cms. that weighed 825 grams) and removed six separate tumors along the posterior peritoneum along the left iliac and into the pelvis.
8/15/2013 -	**Treat - Surgery PT & Mets - Clear**	Surgery removed malignant GIST from pelvis (38 cms. weighing 1416 gms), resected a wedge from my upper left quandrant of my stomach with a malignant GIST tumor (18.2x12.1x8.8 cms. that weighed 825 grams) and removed six seperate tumors along the posterior peritoneum along the left iliac and into the pelvis.
9/06/2013 - 3/12/2015	**Treat - Medication Gleevec@400-mg**	Doing well and tolerating Gleevec with few side effects.
9/15/2013 -	Eval - Scan-CT->Ned	Have had 2 scans since 9/2013. Next scan (PET) scheduled for May 8th.
12/12/2013 -	Eval - Scan-CT->Ned	Reported as no evidence of recurrence.
3/21/2014 -	Eval - Scan-CT->Ned	CAT on 3/21/2014, progression/worsening in mass effect in mid abdomen aorta caval region. Nominal dimensions extending over 5.1 x 3.3 cm. transverse diameter and approximately 9 cm. in longitudinal length. Combined PET/CT exam may provide insight as to active neoplasm versus scar tissue and or other pathology. Mass like deficit cardia of the stomach also is slightly more prominent than seen previously. Pelvis does not show progression.
5/08/2014 -	Eval - Scan-CT->Ned	
5/08/2014 -	Eval - Scan-Pet->Cold	
8/28/2014 -	Eval - Scan-CT->Ned	Next scan on March 13, 2015
3/13/2015 -	(P) Eval - Scan-CT->New Tumors	Scan showed a solitary metastasis in the anterior right hepatic lobe spanning between segments VI and VII. The lesion measured 3.7 x 3.1 x 3.6 cm and showed possible central necrosis. Has numerous hepatic cysts that were stable.
3/14/2015 - 3/30/2015	**Treat - Medication Gleevec@800-mg**	Will have liver surgery tomorrow 4/1/2015. Will provide update after procedure.

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GIST Collaborative Tissue Bank

One tissue donation can reach the world's leading GIST research scientist

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6oo tissue donations linked to clinical histories in the Patient Registry

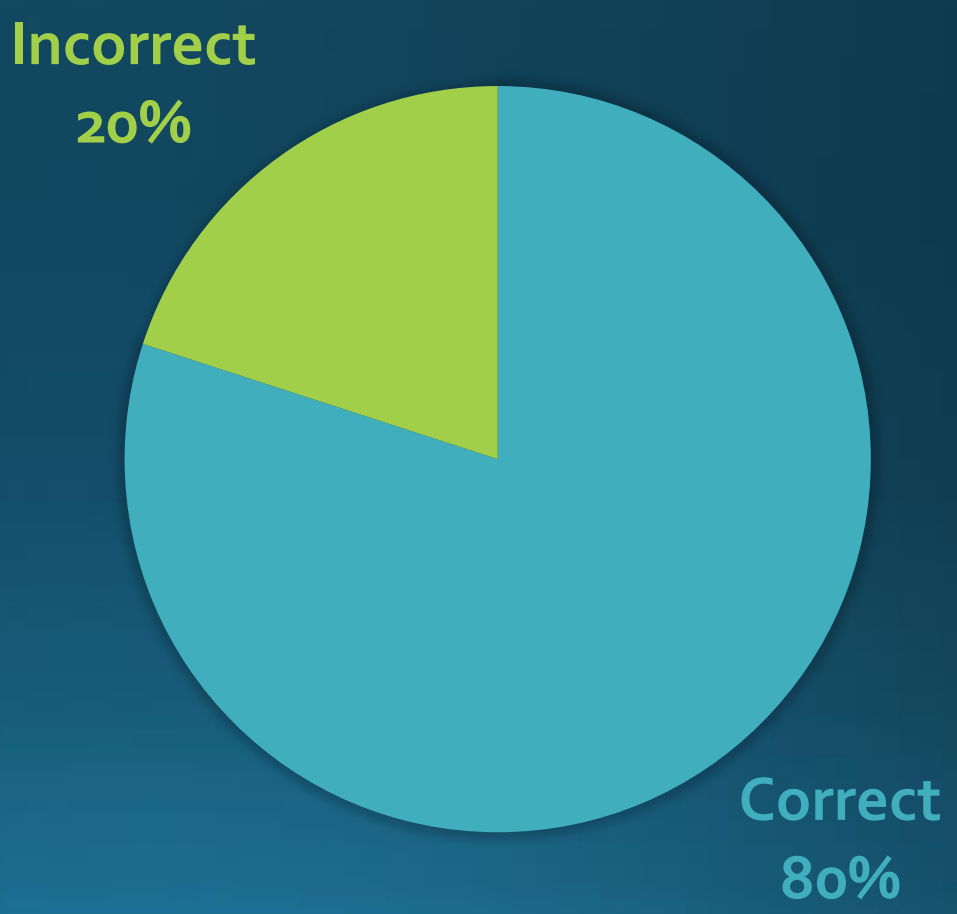
In the United States it is estimated that only

8-10% of GIST patients have mutational testing performed

…by contrast 41% of LRG Patient Registry members know their mutation

Power of the Tissue Bank

- 20% of the tissue from Latin America was shown to have been misdiagnosed (unintended benefit)
- Increased GIST diagnosis
- Paved the way to build testing capacity in Latin America



Role of Country Leaders

"Boots On The Ground"

- Key point person for patients
- Collect medical information to update in Patient Registry
- Coordinate with the LRG to help facilitate the collection of tissue for mutational analysis

Global Representatives in 60 countries

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Moving Forward

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Presenting InterGR

The cure is in the numbers

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The Problem

Despite having insufficient data and tissue to draw meaningful conclusions by themselves, there is an

alarming lack of collaboration in rare cancer research.

Other voices are not being heard—Patient Groups gather richer, more honest and more comprehensive data which can cross institutional boundaries.

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InterGR provides an interactive cloud-based platform for global researchers and patient groups to store, visualize, share and analyze health data to foster rare cancer research collaboration.

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Thank you!

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