



Research • Patient Support & Education • Advocacy

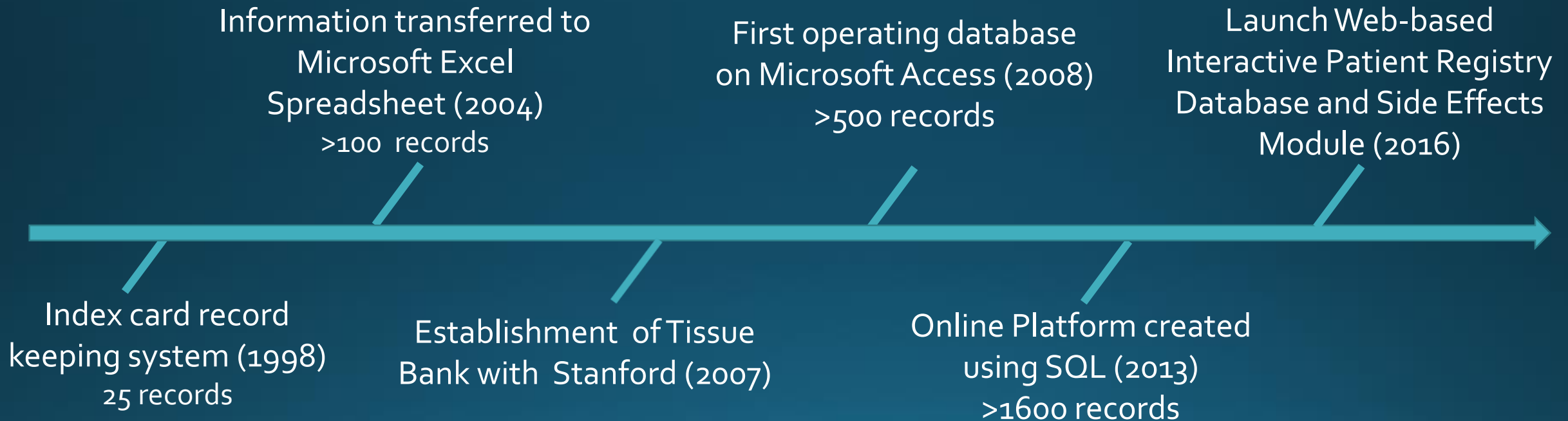
# Understanding GIST

- 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



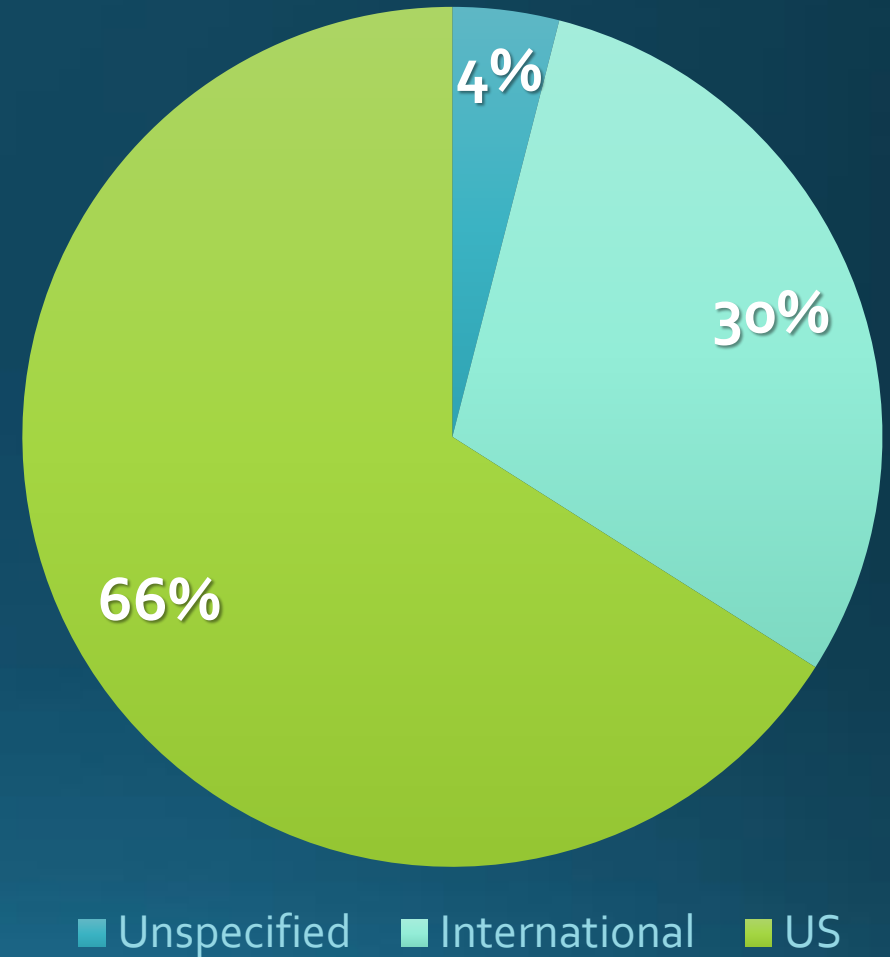
# LRG Patient Registry

# Initial Development of the Patient Registry



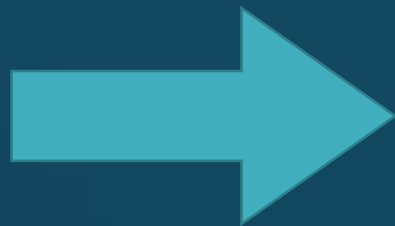
## Registry Population

- 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

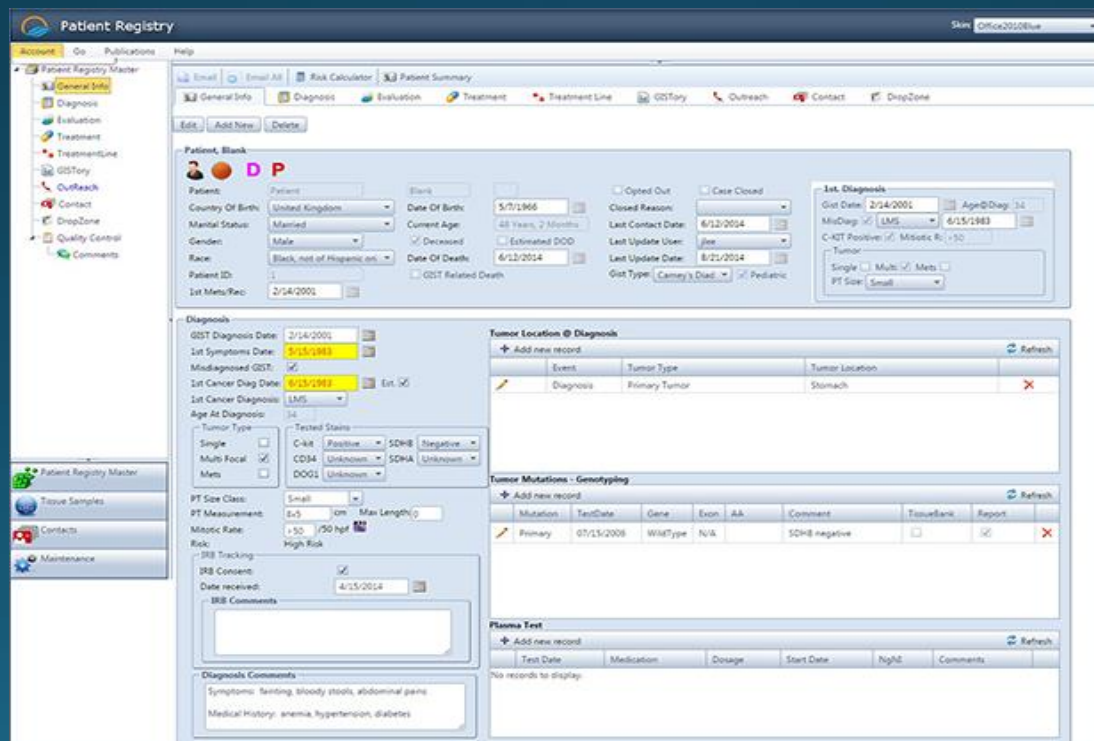


# Data Collected

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



Treatment Patterns  
Treatment Outcomes  
Better Disease  
Management




The screenshot displays the 'Patient Registry' software interface. The left sidebar contains navigation links: Account, Go, Publications, Help, Patient Registry Master, General Info, Diagnosis, Evaluation, Treatment, Treatment Line, GISTory, Outreach, Contact, DropZone, Quality Control, and Comments. The main content area is divided into several sections:

- Patient, Blank:** Fields for Patient, Country Of Birth (United Kingdom), Date Of Birth (5/7/1966), Current Age (48 Years, 2 Months), Marital Status (Married), Gender (Male), Race (Black, not of Hispanic or...), Patient ID (1), and 1st Meta/Rec (2/14/2001). It also includes checkboxes for Opted Out, Case Closed, Decedent, and GIST Related Death.
- Int. Diagnosis:** Fields for Gist Date (2/14/2001), Age@Diag (34), MuDiag (LMS), C-42T Positive (checked), MStom R (-50), and PT Size (Small).
- Diagnosis:** Fields for GIST Diagnosis Date (2/14/2001), 1st Symptoms Date (5/15/1983), Middiagnosed GIST (checked), 1st Cancer Diag Date (6/15/1983), 1st Cancer Diagnosis (LMS), Age At Diagnosis (34), Tumor Type (Single), PT Size Class (Small), PT Measurement (8x5 cm), Mitotic Rate (150 /50 hpf), and Risk (High Risk).
- Tumor Location @ Diagnosis:** A table with columns for Event, Tumor Type, and Tumor Location. The first row shows 'Diagnosis', 'Primary Tumor', and 'Stomach'.
- Tumor Mutations - Genotyping:** A table with columns for Mutation, Test Date, Gene, Exon, AA, Comment, TissueBank, and Report. The first row shows 'Primary', '07/15/2008', 'Wildtype', 'N/A', 'SDH-B negative', and 'checked'.
- Plasma Test:** A table with columns for Test Date, Medication, Dosage, Start Date, NgH2, and Comments. It shows 'No records to display'.
- Diagnosis Comments:** A text area containing 'Symptoms: fainting, bloody stools, abdominal pain' and 'Medical History: anemia, hypertension, diabetes'.

# 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


**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.
8/15/2013 -	Treat - Surgery PT & Mets - Clear	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.
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.

# GIST Collaborative Tissue Bank

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





600 tissue donations linked to clinical histories in the Patient Registry

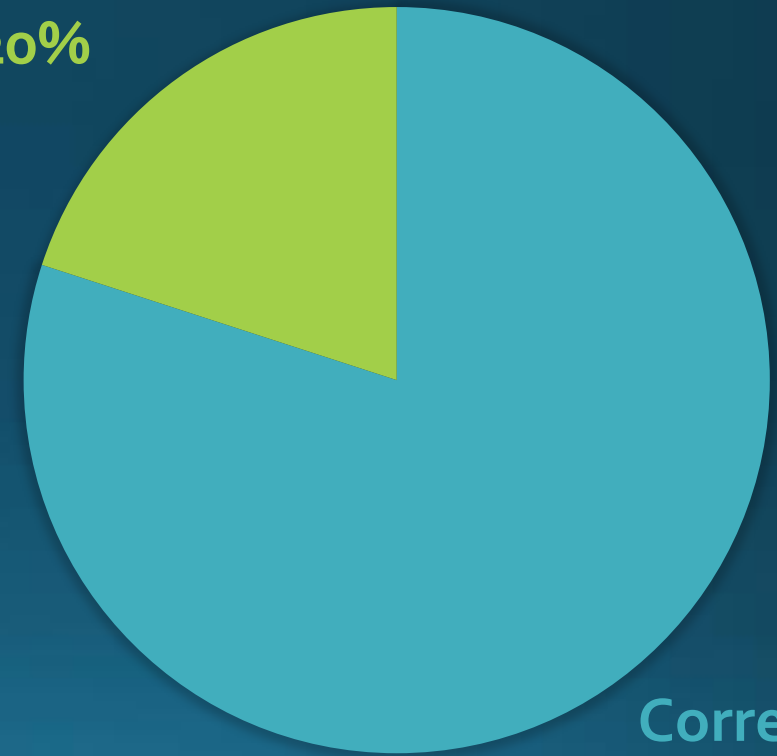
In the United States it is estimated that only  
**8-10%** of GIST patients have mutational  
testing performed

...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

**Incorrect**  
**20%**



**Correct**  
**80%**

# 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

# Moving Forward

# Presenting InterGR

The cure is in the numbers

# 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.

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.



# Thank you!

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