

NEW HORIZONS GIST

SEPTEMBER 5 – 7, 2018 – VIENNA, AUSTRIA

Patient Registry

- Research • Patient Support & Education • Advocacy



LRG Patient Registry Timeline

Information transferred
to Microsoft Excel
Spreadsheet (2004)
>100 records

First operating
database on Microsoft
Access (2008)
>500 records

Launch Web-based
Interactive Patient Registry
Database and Side Effects
Module (2017)
>1700

Index card record
keeping system (1998)
25 records

Establishment of
Tissue Bank with
Stanford (2007)

IRB Approved
Online Platform
created using SQL
(2013)
>1600 records

LRG Patient Registry

Today

Total Patients: **>1800**

Total # of Tissues: **778**

Countries: **68**

About the Patient Registry

- Largest GIST registry in the world
- Uses observational study methods to collect data
- Contains 450 specific data fields
- High standard quality control



The Importance of the Patient Registry

- Powerful tool to observe the course of the disease
- Helps understand variations in treatment and outcomes
- Provides real world data- RWE



Why Are Registries Needed?



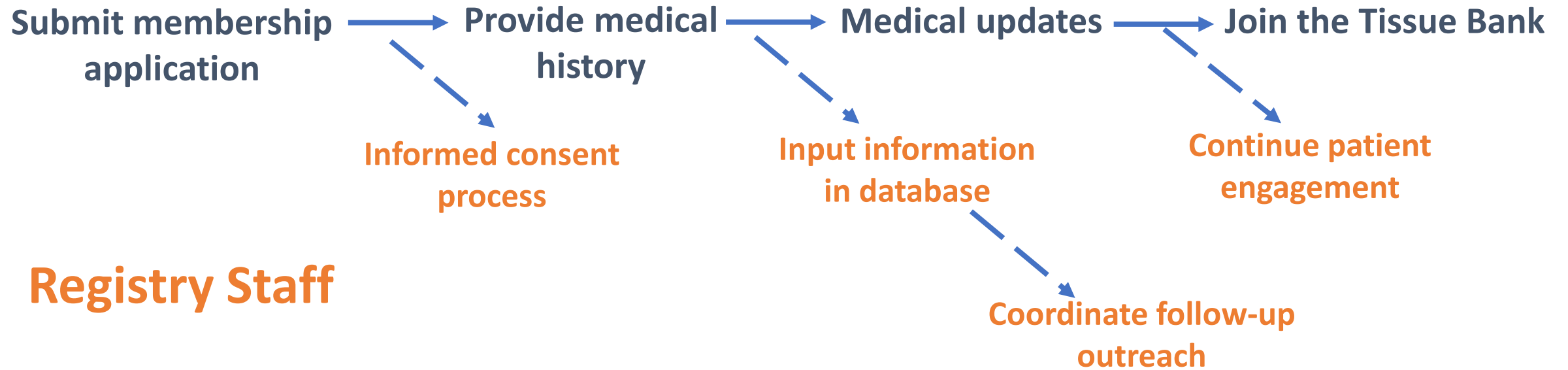
- First-hand information
- Track trends of diseases and treatment
- Determine the disease' s natural history

But most importantly....

They provide the entire patient experience

Joining the Registry

Patients



LRG Patient Registry

1868 Patients

 929 **Males** |  939 **Females**

 1365 **United States** | 502 **International** 

High Risk 35.2%(n=656) Frankly malignant 26.9%(n=501)

Recurrence Free Survival: 6.4 years

Median Overall Survival: 89.7 months

Data Collection and Integration

- Self-reported patient data
- Different methods of data collection to accommodate patient's preferences
- LRG registry captures specific data elements:
- A GISTOry is provided to every patient after an update



The Life Raft GISTory
Patient: Sebestin, Wanda

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

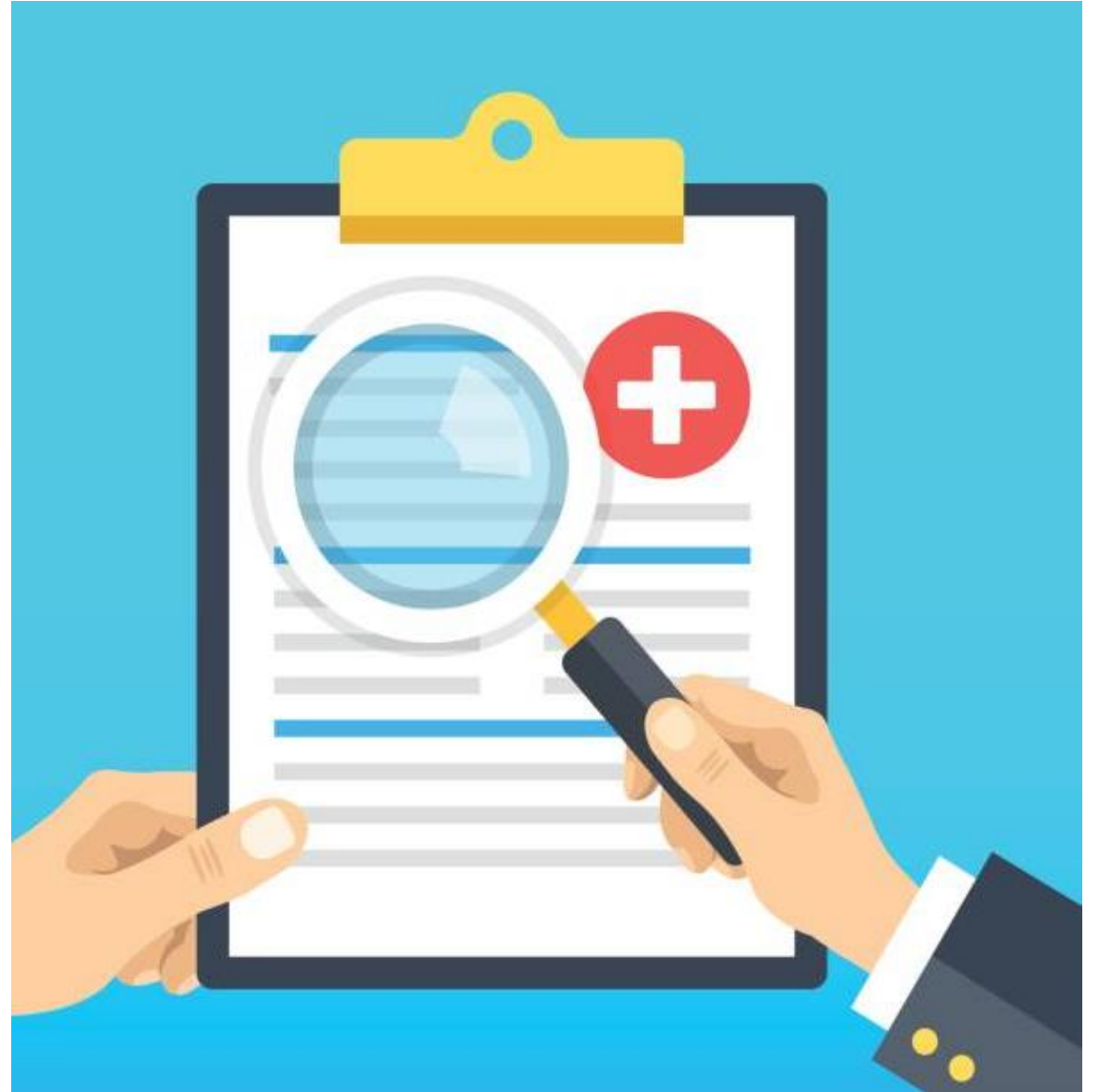
Diagnosis		Tumor Locations		
GIST Diag Date:	8/15/2013	Event	Tumor Type	Tumor Location
Age @ Diag:	60	Diagnosis	Primary Tumor	Stomach
Mitotic Rate:	65	Diagnosis	Mets	Pelvis
CKIT Positive:	<input checked="" type="checkbox"/>	Diagnosis	Mets	Peritoneum
Misdiagnosed:	<input type="checkbox"/>			
1st Diagnosis Date:				
1st Diagnosis:				
Tumor @ Diagnosis:				
Single Tumor At Diagnosis:	<input type="checkbox"/>			
Multi Focal At Diagnosis:	<input type="checkbox"/>			
Mets At Diagnosis:	<input checked="" type="checkbox"/>			

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

Date	Events	Comments
8/10/2013 -	Eval - Scan-Other Scan->New Tumors	2R 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 ++, mitosis increased (13/10 hpfs), CD -117 Positive ++, DOG1 Positive ++, Negative for s-100, desmin, smooth muscle actin.
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 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	Surgery removed malignant GIST from pelvis (38 cms, 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.
9/15/2013 -	Eval - Scan-CT->Ned	Doing well and tolerating Gleevec with few side effects.
12/12/2013 -	Eval - Scan-CT->Ned	Have had 2 scans since 9/2013. Next scan (FET) scheduled for May 8th.
3/21/2014 -	Eval - Scan-CT->Ned	Reported as no evidence of recurrence.
5/08/2014 -	Eval - Scan-CT->Ned	CT on 3/21/2014, progressively/worsening in mass effect in mid abdomen aorta caval region. Nonsinal 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 defect cardia of the stomach also is slightly more prominent than seen previously. Pelvis does not show progression.
5/08/2014 -	Eval - Scan-Pet->Cold	
8/28/2014 -	Eval - Scan-CT->Ned	
3/13/2015 -	(P) Eval - Scan-CT->New Tumors	Next scan on March 13, 2015
3/14/2015 - 3/30/2015	Treat - Medication Gleevec@800-mg	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.
		Will have liver surgery tomorrow 4/1/2015. Will provide update after procedure.

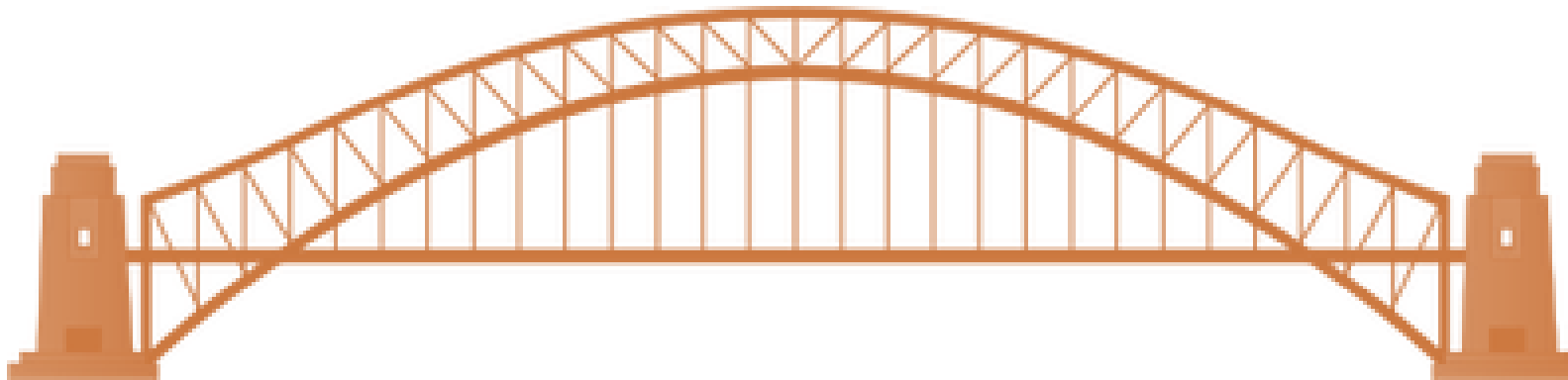
Quality Assurance

- Manual data cleaning
- Monthly query reports
- Data tracking
- Multi-level access based on user's role
- Storing and securing data



Patient Reported Outcomes

Bridge the gap between the clinical reality
and the patient world



Let's take a look of the LRG
Patient registry

Navigation bar with tabs: Email, Email All, Risk Calculator, Patient Summary, General Info, Diagnosis, Evaluation, Treatment, Treatment Line, GISTory, Outreach, Contact, DropZone

Edit Add New Delete

A

Patient:	<input type="text"/>	<input type="text"/>	<input type="text"/>	<input type="checkbox"/> Opted Out	<input checked="" type="checkbox"/> Case Closed
Country Of Birth:	USA	Date Of Birth:	<input type="text"/>	Closed Reason:	Death
Marital Status:	Married	Current Age:	54 Years, 2 Months	Last Contact Date:	4/22/2013
Gender:	Male	<input checked="" type="checkbox"/> Deceased	<input type="checkbox"/> Estimated DOD	Last Update User:	<input type="text"/>
Race:	White, not of Hispanic or	Date Of Death:	8/4/2013	Last Update Date:	7/2/2014
Patient ID:	2	<input checked="" type="checkbox"/> GIST Related Death		Gist Type:	GIST <input type="checkbox"/> Pediatric
1st Mets/Rec:	2/15/2004				

1st. Diagnosis

Gist Date: 7/1/2002 Age@Diag: 43

MisDiag:

C-KIT Positive: Mitotic R:

Tumor

Single Multi Mets

PT Size: Large

Update

Diagnosis

GIST Diagnosis Date:
 1st Symptoms Date:
 Misdiagnosed GIST:
 1st Cancer Diag Date: Est.
 1st Cancer Diagnosis:
 Age At Diagnosis:

Tumor Type
 Single
 Multi Focal
 Mets

Tested Stains
 C-kit SDHB
 CD34 SDHA
 DOG1

PT Size Class:
 PT Measurement: cm Max Length: 20.1
 Mitotic Rate: /50 hpf
 Risk: High Risk

IRB Tracking
 IRB Consent:
 Date received:
IRB Comments

Diagnosis Comments

Primary Mutation
 Gene: Test Date: Comment:
 Exon: Tissue Bank:
 AA: Report:

Secondary Mutations

[+ Add new record](#) [Refresh](#)

Mutation	TestDate	Gene	Exon	AA	Comment	TissueBank	Report
No records to display.							

Tumor Location @ Diagnosis

[+ Add new record](#) [Refresh](#)

	Event	Tumor Type	Tumor Location	
	Diagnosis	Primary Tumor	Stomach	

Plasma Test

[+ Add new record](#) [Refresh](#)

	Test Date	Medication	Dosage	Start Date	NgNI	Comments	
	12/12/2014	Gleevec	600	9/16/2001	3694	On 500 mg, reduced from 600 mg due to rising creatinine levels.	
	7/7/2008	Gleevec	600	9/16/2001	1600		

[Edit](#) |
 [Add New](#) |
 [Delete](#)

Evaluations

Date	Type	Subtype	Measurement	Results
7/15/2013	Scan	CT	Tumor	Stable
4/22/2013	Scan	CT	Tumor	Growth
11/2/2012	Scan	CT	Tumor	Mixed
7/27/2012	Scan	CT	Tumor	Mixed
4/25/2012	Scan	CT	Tumor	Growth
1/27/2012	Scan	CT	Tumor	Mixed
6/14/2011	Scan	CT	Tumor	New Tumors
4/15/2011	Scan	CT	Tumor	Growth
8/15/2009	Scan	CT	Tumor	Ned
6/23/2009	Scan	CT	Tumor	Growth
6/26/2008	Scan	CT	Tumor	Other
4/1/2008	Scan	CT	Tumor	New Tumors
9/15/2007	Scan	CT	Tumor	New Tumors
5/15/2007	Scan	CT	Tumor	Ned
2/15/2007	Scan	CT	Tumor	Ned
11/20/2006	Scan	CT	Tumor	Ned

Refresh

[K](#) |
 [<](#) |
 [1](#) |
 [>](#) |
 [X](#)

23 items in 1 pages

Evaluation Detail:

Evaluation: Evaluation Date: <input type="text" value="7/15/2013"/> Frequency: <input type="text" value="0"/> months Evaluation Type: <input type="text" value="Scan"/> Reporter: <input type="text"/> Last Update User: <input type="text"/> Last Update on: <input type="text" value="11/11/2013 1:34 F"/>	Evaluation is: Estimated Date <input checked="" type="checkbox"/> For Diagnosis <input type="checkbox"/> Questionable <input type="checkbox"/>	Progression Type: Growth <input type="checkbox"/> Local Recurrence <input type="checkbox"/> Mets <input type="checkbox"/> Overall <input type="checkbox"/> Suspected <input type="checkbox"/>
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Evaluation Results:

+ Add new record					
	Type	Sub Type	Measurement	Results	
	Scan	CT	Tumor	Stable	

Tumor Location

+ Add new record				
	Event	Tumor Type	Tumor Location	
	Evaluation	Mets	Liver	
	Evaluation	Recurrence	Stomach	

Comments

Stable on highest dose of Stivarga.

Treatment Details

Treatment Date	TL	Treatment Detail
1/19/2013 - 8/4/2013	9	Medication Stivarga @ 160-mg
11/28/2012 - 1/9/2013	9	Medication Stivarga @ 160-mg
4/26/2012 - 11/25/2012	8	Medication Votrient @ 800-mg
4/26/2012 - 11/25/2012	8	Medication Afinitor @ 5-mg
2/21/2012 - 2/23/2012	7	Medication Chemo Embolization @ 0-mg
12/15/2011 - 4/25/2012	7	Medication Votrient @ 800-mg
6/15/2011 - 12/14/2011	6	Medication Gleevec @ 600-mg
6/15/2011 - 12/14/2011	6	Medication Afinitor @ 2.5-mg
6/13/2011 - 6/14/2011	5	Medication Bland Embolization @ 0-mg
5/5/2011 - 5/6/2011	5	Medication Bland Embolization @ 0-mg
6/16/2010		Surgery Metastasis
6/16/2010 - 5/4/2011	4	Medication Nexavar @ 400-mg
4/16/2010 - 6/15/2010	3	Medication Tasigna @ 800-mg
7/27/2009		Surgery Metastasis
6/15/2008 - 4/15/2010	2	Medication Sutent @ 50-mg
5/15/2008		Surgery Metastasis
10/15/2007		Surgery Metastasis - Clear Margins

Edit Add New Delete

Treatment:

Treatment Date: 1/19/2013 Est. Treatment End Date:

Treatment Type: Medication Place of Treatment:

Medication Details:

Start Date: 1/19/2013 Medication: Stivarga Dosage 160 Units: mg Period: Daily

End Date: 8/4/2013 Taken by: Oral Continuous Cycling

Cycling: On: Off: Concentration: Measure:

Stop taking / Ending Dosages Reason: Death Verification Date:

Medication Type: Unknown Adjuvant NeoAdjuvant Mets/Advance

TL: TreatmentLine = 9

Clinical Trial Medication:

Placebo In Trial: Clinical Trial Name:

Clinical Trial	Drug
No records to display.	

K < 1 > X

24 items in 1 pages

Comments:

1-19-13 reports weight at 112 lbs. Experiencing severe diarrhea. Taking 4, 40mg tablets once daily. Was off of stivarga for 10 days due to side effects. Was back on for 2 weeks and side effects were kicking in again. Spouse reported unable to endure

Last Updated By:

Last Update User:

Last Update Date: 3/12/2015

Patient ID: 2

Date of Death

Last Contact Date

Show Detail

8/4/2013

4/22/2013

Set TreatmentLine ReCalculate New Treatment

Refresh

T-Line	Medication	Type	Start Date	End Date
<input type="text"/>	<input type="text"/>	<input type="text"/>	<input type="text"/>	<input type="text"/>

T-Line	Medication	Type	Start Date	End Date	
1A	Gleevec for Adjuvant @ 400-mg	Adjuvant	8/27/2002	8/27/2003	X
1M	Gleevec for Mets @ 400-mg	Mets	2/16/2004	3/1/2006	X
1M	Gleevec for Mets @ 800-mg	Mets	4/15/2006	6/14/2006	X
1M	Gleevec for Mets @ 600-mg	Mets	6/15/2006	5/12/2008	X
2	Sutent for Mets @ 50-mg	Mets	6/15/2008	4/15/2010	X
3	Tasigna for Mets @ 800-mg	Mets	4/16/2010	6/15/2010	X
4	Nexavar for Mets @ 400-mg	Mets	6/16/2010	5/4/2011	X
5	Bland Embolization for Mets @ 0-mg	Mets	5/5/2011	5/6/2011	X
5	Bland Embolization for Mets @ 0-mg	Mets	6/13/2011	6/14/2011	X
6	Gleevec for Mets @ 600-mg	Mets	6/15/2011	12/14/2011	X
6	Afinitor for Mets @ 2.5-mg	Mets	6/15/2011	12/14/2011	X
7	Votrient for Mets @ 800-mg	Mets	12/15/2011	4/25/2012	X
7	Chemo Embolization for Mets @ 0-mg	Mets	2/21/2012	2/23/2012	X

Filter Ablation Hospice Medication No Treatment Other Radiation Surgery

Refresh

T-Line	Medication	Type	Start Date	End Date
<input type="text"/>	<input type="text"/>	<input type="text"/>	<input type="text"/>	<input type="text"/>

T-Line	Medication	Type	Start Date	End Date
1A	Gleevec	Adjuvant	8/27/2002	8/27/2003
1M	Gleevec	Mets	2/16/2004	5/12/2006
2	Sutent	Mets	6/15/2008	4/15/2010
3	Tasigna	Mets	4/16/2010	6/15/2010
4	Nexavar	Mets	6/16/2010	5/4/2011
5	Bland Embolization	Mets	5/5/2011	6/14/2011
6	Afinitor + Gleevec	Mets	6/15/2011	12/14/2011
7	Chemo Embolization + Votrient	Mets	12/15/2011	4/25/2012
8	Afinitor + Votrient	Mets	4/26/2012	11/25/2012
9	Stivarga	Mets	11/28/2012	8/4/2013

PFS Progression Free Survival

- Time spent on specific treatment without progression

OS Overall Survival

- Time living with GIST since date of diagnosis

TOM Time on Medicine/Drug

- Time of treatment on a particular drug

Save
ReCalculate

1M : Gleevec

	Date	Dose
Starting:	<input type="text" value="2/16/2004"/>	<input type="text" value="400"/>
Progression:	<input type="text" value="2/13/2006"/>	<input type="text" value="400"/>
Ending:	<input type="text" value="5/12/2008"/>	<input type="text" value="600"/>

	Min	Avg.	Max
Dose:	<input type="text" value="400"/>	<input type="text" value="493.72"/>	<input type="text" value="800"/>

Progression:

	System Calc	Override
Date:	<input type="text" value="2/13/2006"/>	<input type="text"/>
Dose:	<input type="text" value="400"/>	<input type="text" value="400"/>
Ending Dose:	<input type="text" value="600"/>	

Last Treatment:

	Dose	Start	End
9 : Stivarga	<input type="text" value="160"/>	<input type="text" value="1/19/2013"/>	<input type="text" value="8/4/2013"/>

Comments:

Current Treatment Line (Months):

PFS:	<input type="text" value="24"/>	KM_PFS	<input type="text" value="1"/>
OS_TL:	<input type="text" value="114"/>	KM_OS_TL	<input type="text" value="0"/>
TOM:	<input type="text" value="51"/>	KM_TOM	<input type="text" value="1"/>

Patient Overall (Years):

RFS:	<input type="text" value="2"/>	KM_RFS	<input type="text" value="1"/>
OS:	<input type="text" value="11"/>	KM_OS	<input type="text" value="1"/>

1M : Gleevec

Starting:	Date: 2/16/2004	Dose: 400
Progression:	Date: 2/13/2006	Dose: 400
Ending:	Date: 5/12/2008	Dose: 600

Min Dose: 400	Avg.: 493.72	Max: 800
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Current Treatment Line (Months):

PFS: 24	KM_PFS: 1
OS_TL: 114	KM_OS_TL: 0
TOM: 51	KM_TOM: 1

Patient Overall (Years):

RFS: 2	KM_RFS: 1
OS: 11	KM_OS: 1

Progression:

System Calc	Override
Date: 2/13/2006	
Dose: 400	400
Ending Dose: 600	

Last Treatment:

Dose	Start	End
9 : Stivarga 160	1/19/2013	8/4/2013

Comments:

QC Rating: 1 ?

Comments:

Some question about whether or not the stopped for procedure date was actually the date of progression.

8/27/2014 - Katherina Aljallad
 Patient began Gleevec after progression on 2/15/2004. Patient had surgery on 5/15/2008 (estimated date), did not continue back on Gleevec.

MDURBOROW, 12/11/2014 5:47 PM
 Changed rating from 2 to 1 after further review of records

Mark For Registrar Follow-up with Hard Copy
 Needs Patient Follow-up
 Needs Admin Review
 QC Rating:

Investigating
 Follow-up Complete
 Fail-Archive/Purge Record

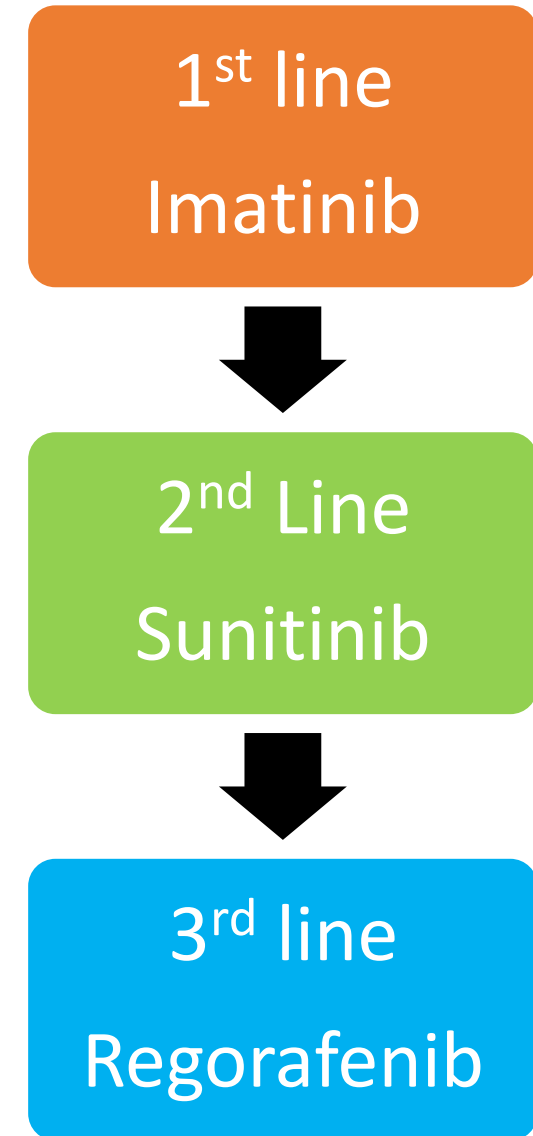
T-Line	Medication	Start Date	End Date	Reason for Stop
1M	Gleevec for Mets @ 400-mg	2/16/2004	3/1/2006	Progression
1M	Gleevec for Mets @ 800-mg	4/15/2006	6/14/2006	Side Effects
1M	Gleevec for Mets @ 600-mg	6/15/2006	5/12/2008	Stopped for Procedure

Evaluation Date	Progression		Eval. Type	Results	Comments
	Overall	Suspected			
4/1/2008	✓	<input type="checkbox"/>	Scan	New Tumors	
9/15/2007	✓	<input type="checkbox"/>	Scan	New Tumors	
5/15/2007	<input type="checkbox"/>	<input type="checkbox"/>	Scan	Ned	
2/15/2007	<input type="checkbox"/>	<input type="checkbox"/>	Scan	Ned	
11/29/2006	<input type="checkbox"/>	<input type="checkbox"/>	Scan	Ned	

Treatment Lines

- Treatment lines compare effectiveness of a given sequence of drug therapies
- Treatments given early should be compared to other treatments given early, late vs. late etc. (i.e. 2nd line to other 2nd line, 3rd line to other 3rd line)

FDA Approved Drug Sequence



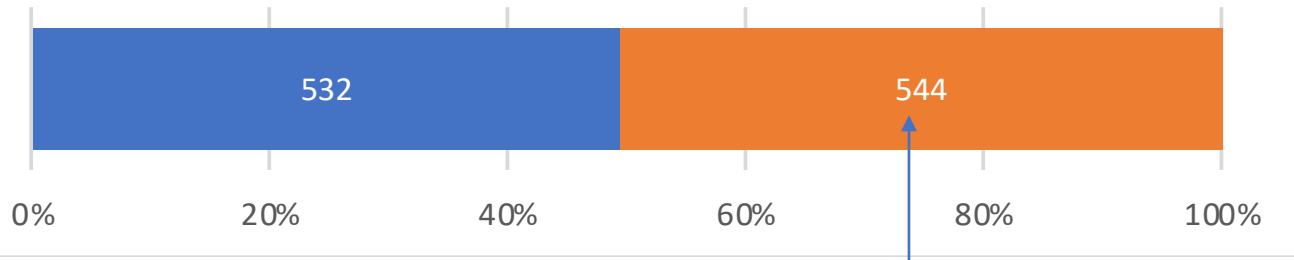
Treatment Lines: Rapid Hypothesis Generation

- Generation of **sortable waterfall charts** can rapidly reveal interesting treatment patterns
- **Rapidly** generated findings can be followed up with further, more stringent analysis such as Kaplan Meier curves.

BREAKDOWN OF MUTATIONS

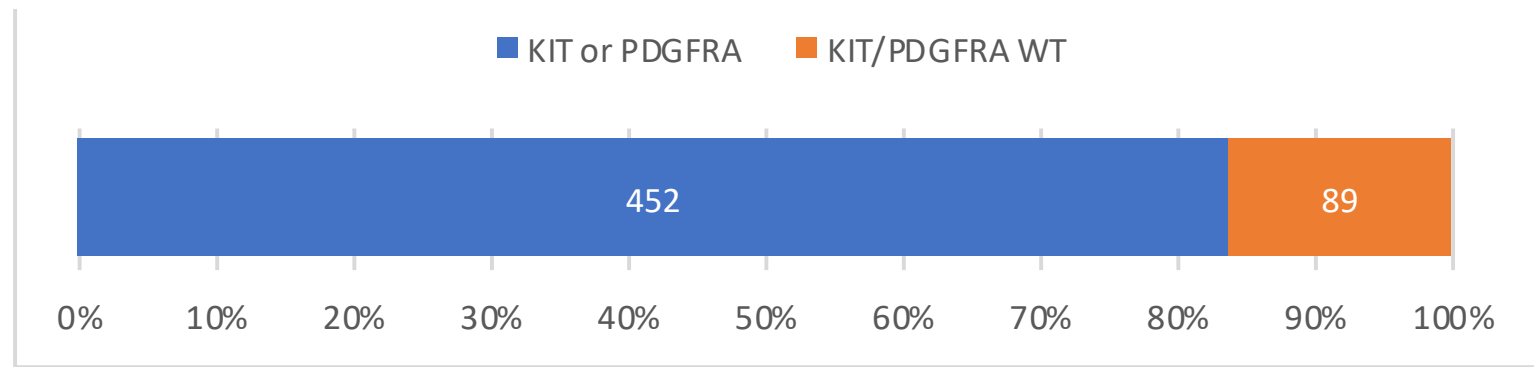
50.5% of living patients know their mutation

■ Unknown ■ Known



84% KIT/PDGFR mutations
16% KIT/PDGFR WT (NOS)

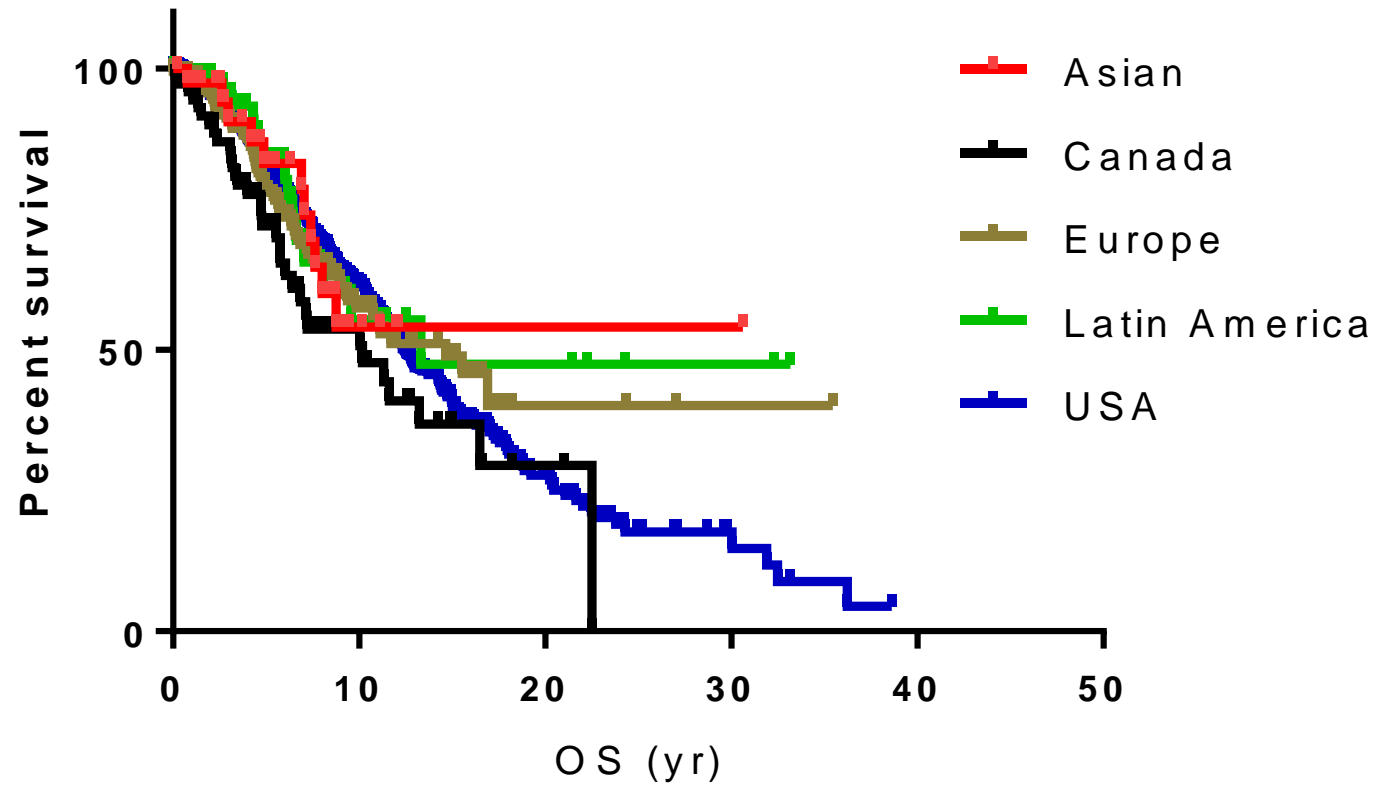
■ KIT or PDGFR ■ KIT/PDGFR WT



Known Mutations

*No Other Specified

GIST Patient Overall Survival in Different Geographic Regions



	Asian	Canada	Europe	Latin America	USA
# censored subjects	28	39	124	66	797
# deaths/events	11	33	66	16	484
Median survival	Undefined	10.1684	14.6393	13.3087	12.3587

Benefits of Data



- Describes the natural history of disease
- Studies clinical and treatments' effectiveness
- Evaluates specific patient outcomes
- Increases international collaborations
- Accelerates research development
- Improves patient-physician communication

“

Many times I think things are the way they are supposed to be and there is nothing I can do about them. Then someone reminds me that there are things that can be done.

”

Thanks for reminding me that there are options.

- Patient Registry Member Since 2011



Thank you!



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