

# **GIST Patient Journeys: Analysis of Patient-Reported Real World Experience Highlights Progress and Remaining Medical Need**

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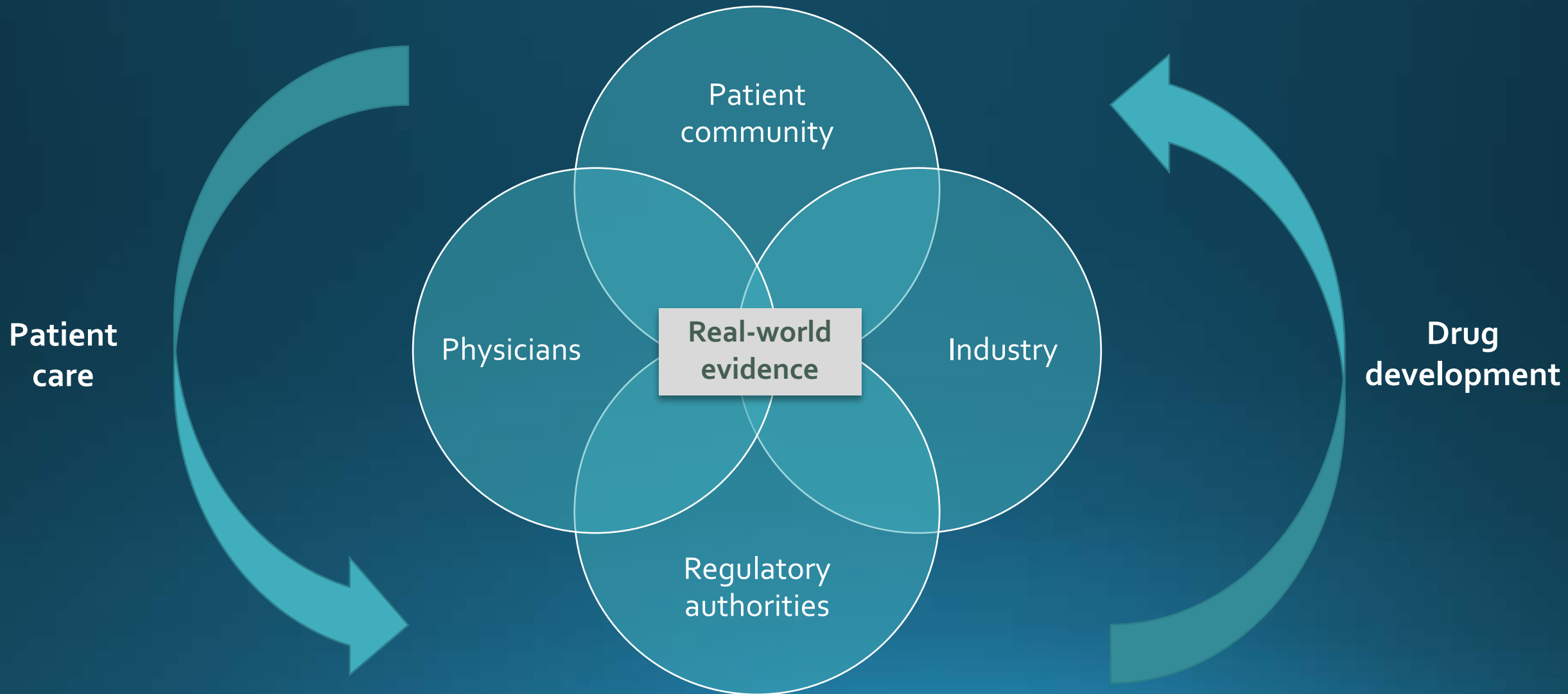
Real-world evidence is evidence from any and all sources of data that may contribute to more effective health care, including health care best tailored to the needs of individual patients.

*- Network for Excellence in Health Innovation*

# Presentation Outline

- Collaborative Research Project Overview
- Opportunities to Enhance Molecular Testing
- Areas of Patient Need
- Future Directions

Real-world evidence has potential to catalyze improvements in outcomes



# The patient's voice and experience are key to drug development

## *FDA Patient Focus Drug Development Initiative*

- Initiative to encourage drug development informed by patient perspectives<sup>1,2</sup>:
  - Patient-focused drug development meetings (22 to date)
  - Patient-reported outcomes guidance (2009)

## *21<sup>st</sup> Century Cures Act*

- Legislation directs FDA to incorporate real-world evidence in regulatory decision making<sup>3</sup>:
  - Fit-for-purpose tools to collect key data on patient experience
  - Draft guidance on methods for data capture and analysis expected in 2018

<sup>1</sup>Prescription Drug User Fee Act V (PDUFA V) authorized on July 9, 2012

<sup>2</sup>FDA Guidance for Industry on Patient-reported Outcomes Measures to Support Labeling Claims

<sup>3</sup>Title III Section 3002. Signed into law December 13, 2016

# The Life Raft Group GIST patient registry is a rich source of real-world data



**Welcome to GIST/PRIME**  
An interactive patient registry for GIST patients and caregivers.

**Register**  
Already a Member? [Login](#)

**Supporting Patients**

- Take advantage of resources to keep your GIST medical records organized and up-to-date.
- Get individualized feedback on your care to help you and your medical provider's stay current with the latest GIST treatments and medical breakthroughs.

**Helping the GIST Community**

- See how your experiences compare to other GIST patients.
- Become part of the global GIST community by contributing your clinical data and building the largest GIST database in the world.

**Accelerating Research**

- Learn how you can participate in cutting-edge research that will accelerate the development of new GIST treatments.
- Donate tissue to the GIST Collaborative Tissue Bank and be part of a unique patient-powered research initiative.

- Registry goal: Improve disease understanding and accelerate research
- Benefits: access to the registry team, care management, education/training and research/consultation

All Patients (throughout history of registry)	
Total Patients	N= 1709
Localized at Dx → unresectable / metastatic recurrence AND Metastatic at Dx	n= 1089
Geographies Represented	67
Average Duration of Follow Up	64 months
Patients Alive / Deceased (as of Sept 22 2017)	n=1061 / n=648

**Registry dataset contains a large number of patients representative of a broad patient experience**

# Countries reached by the LRG

<b>US Patients</b>	<b>1282</b>
<b>International Patients</b>	<b>427</b>
<b>Total</b>	<b>1709*</b>

United States	1282
Canada	72
Netherlands	46
United Kingdom	44
Unknown	31
Australia	30
Chile	30
Mexico	17
Colombia	11
Germany	11
All other	135

Other Countries					
Italy	9	Venezuela	2	Costa Rica	1
Argentina	8	China	2	Sri Lanka	1
India	8	Sweden	2	Morocco	1
Japan	7	Korea, South	2	Cyprus	1
Israel	7	Greece	2	Denmark	1
France	6	Lithuania	2	Thailand	1
#N/A	6	Philippines	2	Saudi Arabia	1
Kenya	5	Uruguay	2	Turkey	1
Switzerland	5	Taiwan	1	Bermuda	1
New Zealand	5	Spain	1	Bolivia	1
Brazil	4	Ukraine	1	Slovenia	1
Finland	4	Peru	1	Pakistan	1
Norway	4	Austria	1	Macedonia	1
Singapore	3	Kuwait	1	Puerto Rico	1
Bulgaria	3	Tunisia	1	Vietnam	1
Belgium	3	Poland	1	Russian Federation	1
Malaysia	3	Ireland	1	Dominican Republic	1
South Africa	2	Venezuela	2	Samoa	1

\*Reflects the total patients contained within registry and country of residence

# Collaboration goal to understand the GIST patient experience through real-world data

- Key Project Goals

- Enhance understanding of real-world treatment of GIST patients
- Identify areas of collaboration to support patient empowerment
- Support Blueprint Medicines Blu-285 drug development program



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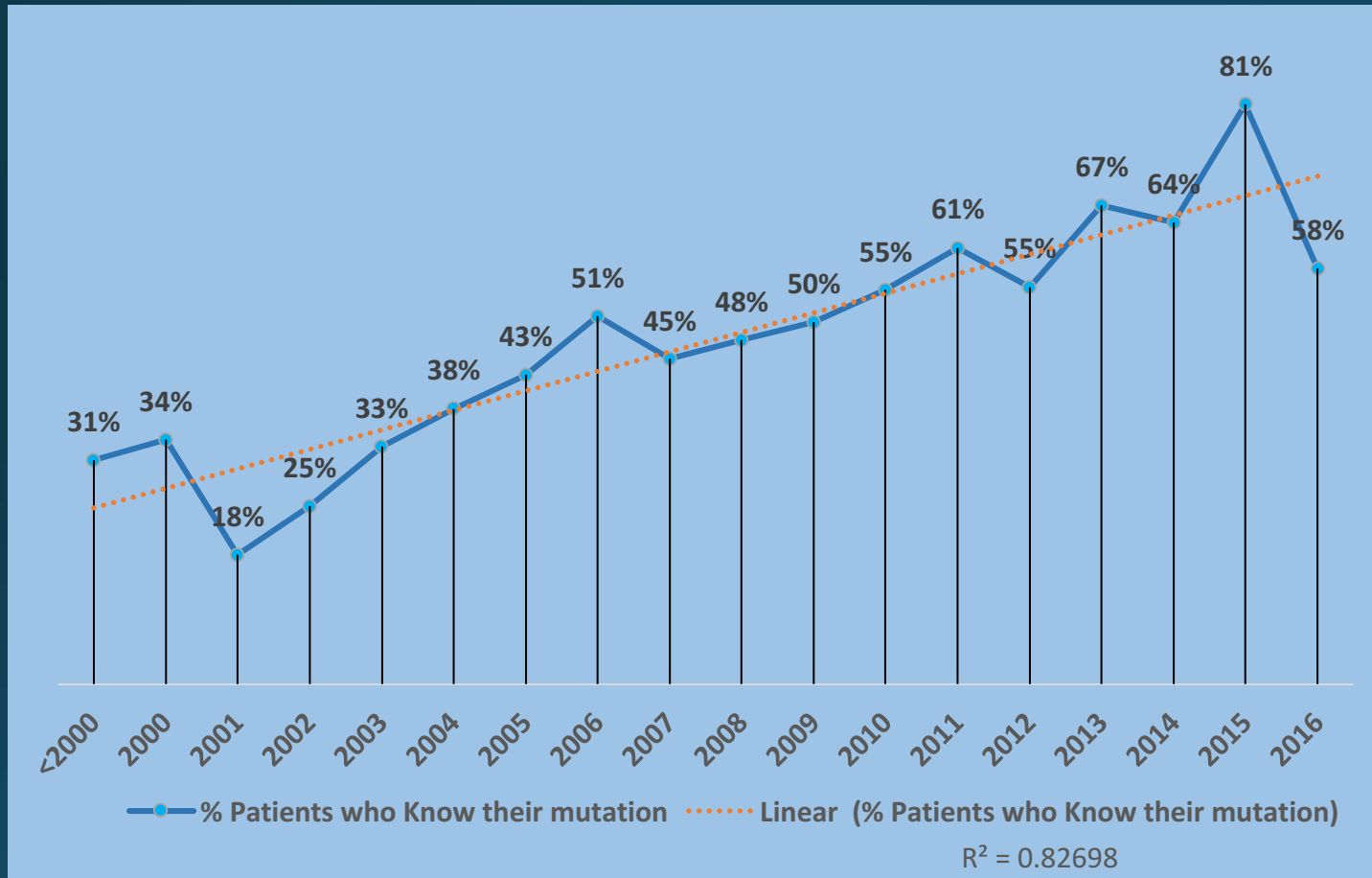
Dr. Jonathan Trent



# Presentation Outline

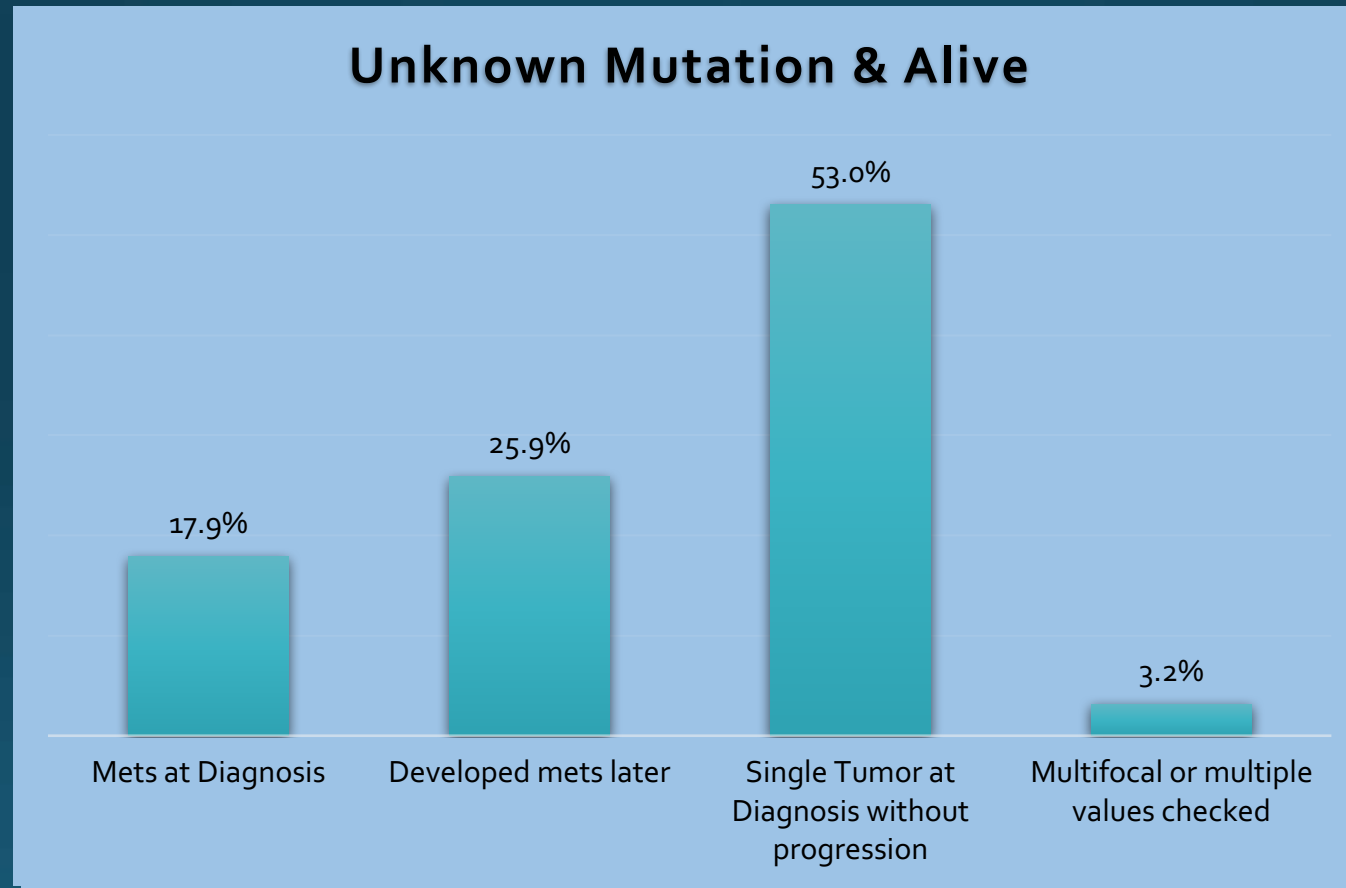
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# Rates of molecular testing have steadily increased over time



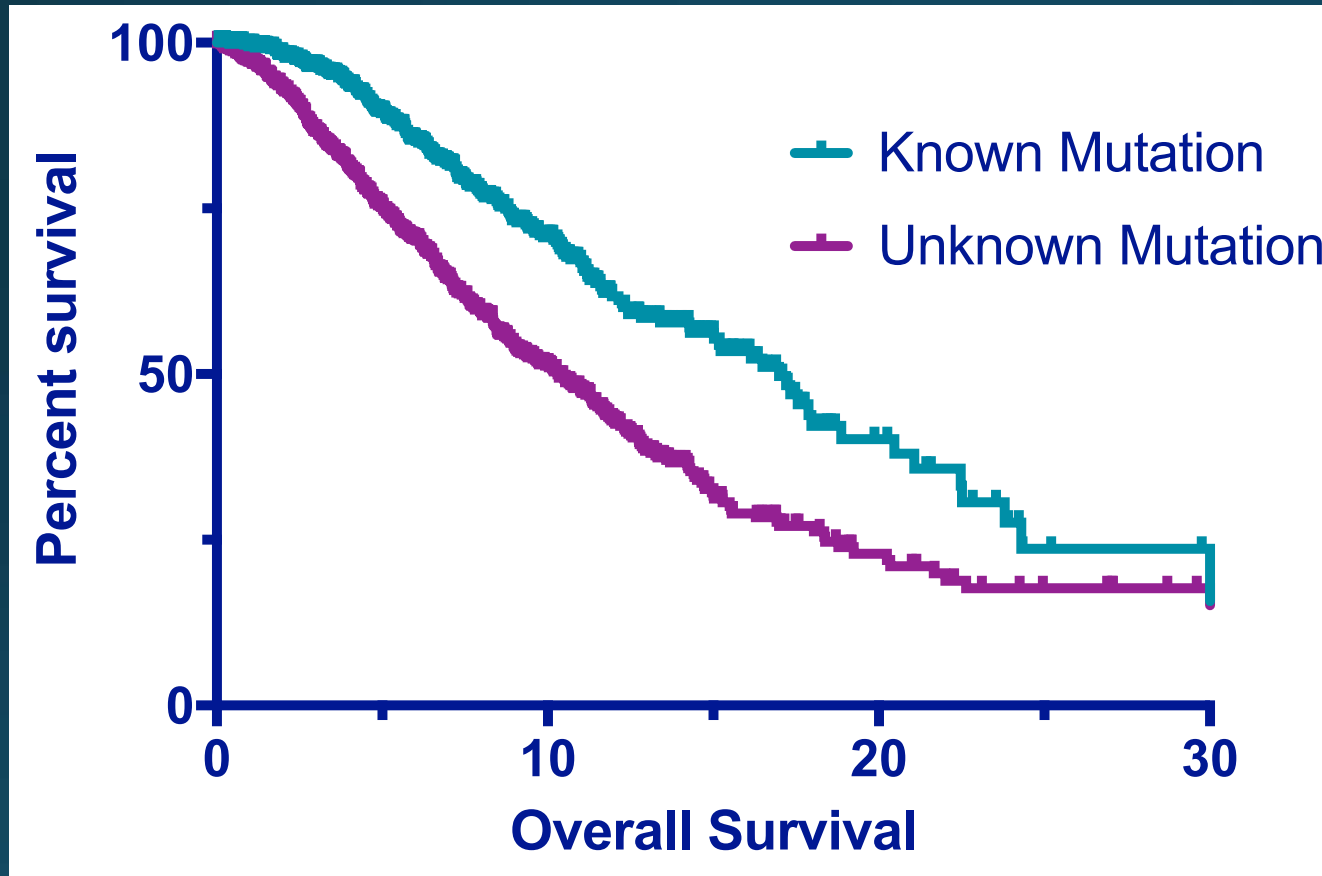
*Rates of testing within this report likely higher than in the broader community given Life Raft Group encouragement of registry participants to be tested and connection with academic site*

# Educational barriers remain with a significant portion of registry patients with unknown mutations



\*Reflects total patients in the LRG Patient Registry, US and non-US

# Awareness of mutational status may positively impact overall survival



Overall Survival (yrs.), Known vs Unknown mutation.  
Reflects total patients in the LRG Patient Registry, US and non-US.

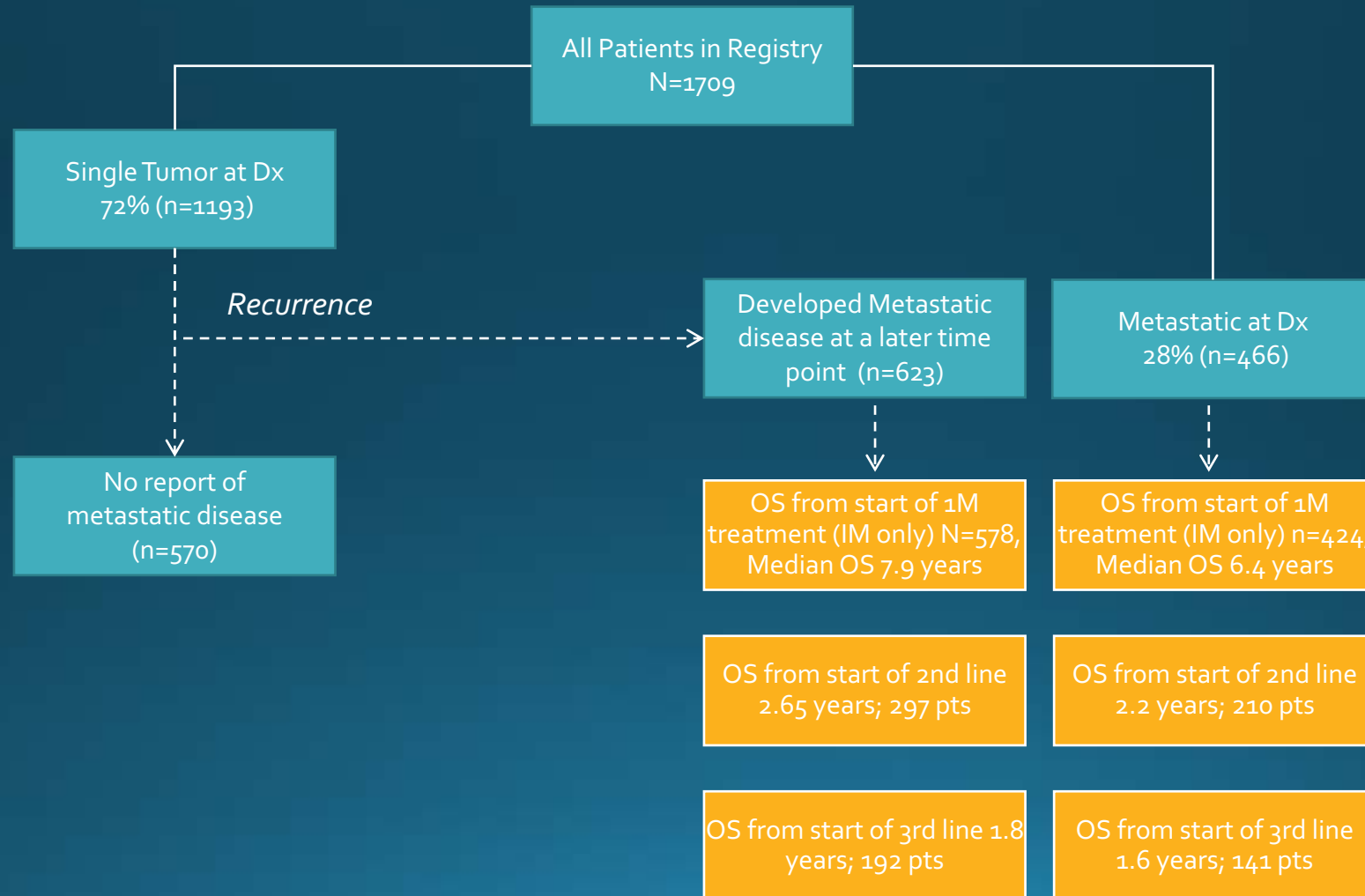
- A variety of factors may confound this data:
- LRG members tend to be more pro-active in their care
  - The LRG Tissue Bank Initiative encouraged testing
  - Testing may not be available in other countries

Important to caveat this data may be confounded with a variety of factors, however it highlights the need for GIST patients to see experts (e.g., presumably the individuals who are testing)

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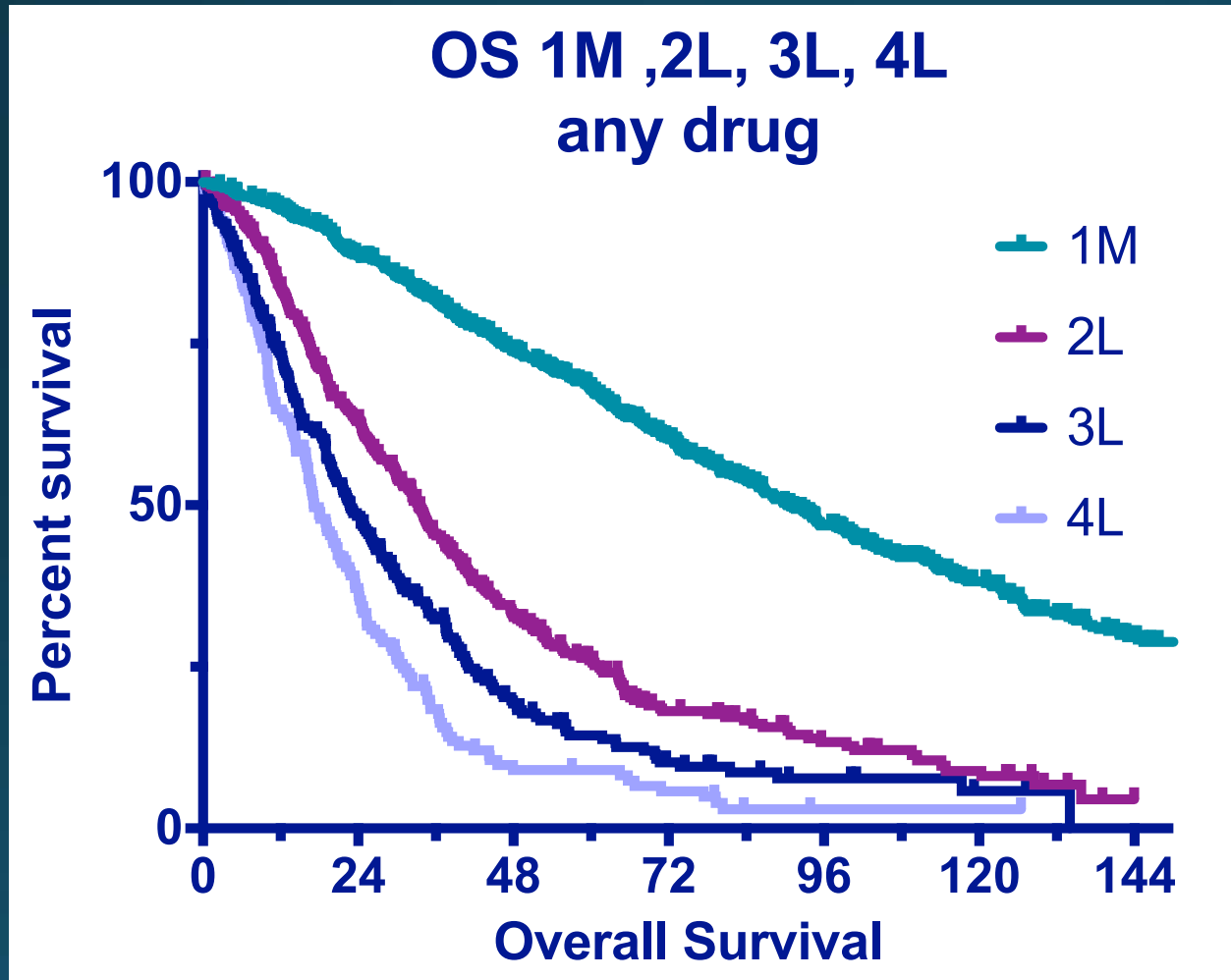
# Registry is a powerful tool to enhance our understanding of the patient treatment journey



# Real-world patient data highlights diversity of prescribed therapies following progression on Sutent

	% Unresectable/metastatic patients receiving each line of therapy (US only)	Most common therapies by treatment line
1L	793/822 = (95.8%)	Gleevec = 95.8% Imatinib (generic) + Gleevec = 0.9% Gleevec + embolization = 1.8% All others = 1.5%
2L	407/822 = (49.5%)	Sutent = 84.2% Tasigna = 2.7% Motesanib = 2.5% Nexavar = 1.7% All Others = 8.9%
3L	281/822 = (34.2%)	Gleevec = 23.8% Nexavar = 19.2% Stivarga = 18.5% Tasigna = 12.8% Sutent = 5.7% All Others = 19.9%
4L	181/822 = (22.0%)	Tasigna = 15.5% Gleevec = 14.9% Nexavar = 14.5% Sutent = 12.2% Stivarga = 9.9% Votrient = 5.6% Sprycel = 3.3% All Others = 24.3%

## High medical need in treatment of GIST in post-imatinib setting



Overall Survival (mos.) from Start of Treatment

Patients with metastatic disease at diagnosis and those with single tumors who developed metastasis at a later time (US only).

Includes any/all drugs in the referenced treatment lines.



## Several additional areas have been identified for further analysis

- 1 Delineate real-world treatment patterns
- 2 Determine key factors that impact GIST patients quality of life
- 3 Understand the side effects and reasons for discontinuation

**These pieces of information are key to understand from an industry perspective to ensure the patient's experience informs drug development**

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# Summary and Future Directions of the Collaboration

## Collaboration Summary

- Created key questions and mined registry
- Developed comprehensive patient journey
- Identified key barriers to ideal treatment of GIST patient

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## Next Steps

- Q4 2017: Finalize data extractions
- Q4 2017 – Q1 2018: Disseminate results through publications
- Q4 2017 – Q1 2018: Formalize future collaborations

**Continued support of the patient registry and encouragement of patients to join is essential for this rich resource to remain up-to-date and reflect the current status of GIST treatment**

# Project fosters a productive community-industry partnership

*Project Kickoff Meeting at Blueprint Medicines in Cambridge, MA*



# Questions and Answers