

# **NEW HORIZONS GIST VIRTUAL CONFERENCE 2021**

*Celebrating 20 Years of GIST Treatments*

October 20<sup>th</sup>-22<sup>nd</sup>, 2021



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# Acknowledgements

We would like to thank the following sponsors who supported the New Horizons Virtual GIST Conference 2021 activities with an unrestricted educational grant:



## Sponsored By:



At the request of the Steering Committee, NEW HORIZONS GIST received grants from these three companies. This funding is not related to any objectives/content of the Virtual Conference 2021. The idea, planning, preparation, realization, management and the summary of the NEW HORIZONS GIST 2021 Conference are the responsibilities of the Steering Committee and the Life Raft Group (LRG) without any influence from the sponsors/funders. We are looking forward to continuing these open and transparent partnerships with the healthcare industry towards achieving our goal of collaboration among independent GIST patient organizations on an international level.

We also would like to thank our friends/colleagues from the medical GIST Expert Community. We are very grateful for the valuable, trusted and long-term partnership between leading medical GIST experts worldwide and the Global GIST patient advocacy community. Thank you also to our patients and advocates who joined us to share their expertise and experiences.

This report was compiled by the staff of the Life Raft Group.



# Introduction

## History

Every year country leaders gather to discuss scientific updates, regional issues, and unmet needs for the global GIST community. This international meeting was launched by Novartis Oncology in 2003 with the title, "New Horizons in Treating CML and GIST," with the goal of uniting patient organizations representing people living with CML and GIST.

A few years ago, the conference divided into two separate meetings—one focused on GIST and the other on CML. Since then, the New Horizons GIST Conference has been organized by a GIST Steering Committee that aims to unify the global GIST patient advocacy community with key opinion leaders and facilitate ways to increase survival worldwide. What you may not know is that these advocates have over the years become lifetime friends. We celebrate happy occasions together and commiserate when we lose dear friends. We have walked through GIST history together.

## Today

This year's meeting was once again held virtually and our audience included medical professionals, patient advocates plus patients and caregivers. Twenty-five countries were represented with over 200 participants. Over a dozen speakers from around the globe shared their time and experiences.

## NEW HORIZONS GIST Steering Committee

The 2021 conference was chaired and planned by a steering committee and Life Raft Group:

- Piga Fernandez, Fundación GIST Chile, AlianzaGIST (Chile)
- David Josephy, GIST Sarcoma Life Raft Group (Canada)
- Ginger Sawyer, GIST Support International (USA)
- Norman Scherzer, The Life Raft Group (USA)
- Markus Wartenberg, Deutsche Sarkom-Stiftung (Germany)
- Martin Wettstein, Swiss GIST Group (Switzerland)

Supported by Sara Rothschild and Laura Occhiuzzi (LRG USA).



## 2021 Presentations

### Day One

The conference was opened by Norman Scherzer, Executive Director of The Life Raft Group.

Norman shared, "Today we come together - 200 participants, over a dozen speakers, over 25 countries from around the world - and the thread that links us all together is that we each give one another hope. We underestimate in this world sometimes what hope is worth. And it's worth everything. We adopted a mantra at the LRG from the very beginning, "You are not alone." No matter where you are; whether it's Kenya, Switzerland, India, Canada or what-have-you. And thanks to the internet and a group of unusual people, you are not alone,"

Norman thanked conference sponsors Blueprint Medicines, Deciphera Pharmaceuticals, and Novartis for the support in bringing together today medical professionals, patient advocates, patients and caregivers from all over the world.

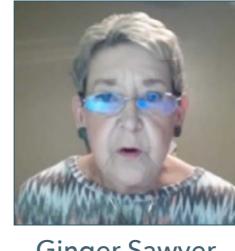
Before 'passing the baton' Norman commented that, "Many of us have known each other for many years. Some for only a few days. We are a brotherhood and sisterhood of survivors and caregivers that you can't understand unless you are a part of it. We share so many defining moments. Some sad. Loss. Some glorious. Survival. We, the Life Raft Group, welcome you all."



# Day One - History

## Session One

Ginger Sawyer, of GIST Support International (USA) introduced Dr. George Demetri of the Dana-Farber Cancer Institute.



Ginger Sawyer

[Video link](#)



Dr. George Demetri

### *"In the Beginning..." The Story of How GIST Treatments Began*

**Dr. George Demetri**

**Medical Oncologist, Director, Sarcoma Center  
Executive Director, Clinical and Translational Research  
at the Ludwig Institute for Cancer Research  
Dana-Farber Cancer Institute**

Dr. Demetri spoke of the GIST research journey as a transformational experience for us all throughout the years - for patients, and for the medical professionals and researchers who still look to GIST as an inflection point as to how cancer is understood. This is not a small thing. He thanked all for being partners in this progress, not just patients, subjects, or providers but as pre-patients, humans, partners in the research. We are all in this together.

As part of the reflections of the history of GIST, Dr. Demetri shared personal insights.



We thought of cancer in the 1970s as an out of control roaming group of horses breaking down fences everywhere.



Then it was realized that all cancers were not alike and some were more common than others.

## Day One - History

People with sarcomas were viewed as zebras. In the 1980s, he and his colleagues reviewed the many sarcomas and GIST was known to be as a particularly nasty sarcoma. Eventually enough information (zebras) was gathered to understand that not all sarcomas were GIST but surprisingly this disease called GIST turned out to be the most common type of sarcoma.

Teamwork was a major player at this time in history. Researchers began working with patients who in turn talked to regulators, payers, and pharma. And things got done. Investigators in Japan discovered that the KIT mutation was a key driver in GIST. Dr. Brian Druker (OHSU) believed that the drug he was working on (imatinib) for CML would turn off that driver. Subsequently, Gleevec became the miracle drug. When researchers tied CML and GIST together this helped patients become aware that they might have GIST which improved misdiagnosis rates. Demetri shared his belief that a future cure is possible through combination drug therapy.

*"If you want to go fast, go alone. If you want to go far, go in a group."* African Proverb

### Video link

## Session Two

Sara Rothschild, LRG Vice President of Program Services, The Life Raft Group, introduced a long-term survivor from the first GIST trial who shared his GIST journey.



Sara Rothschild



Chuck Korte

### Long-Term Survivor Story

#### Chuck Korte, Patient

Chuck is a retired professor of social psychology and gerontology, has been married to his college sweetheart for over 50 years, is very active in our GIST community, and is a GIST survivor of 20+ years.

He graciously shared the details of his GIST story and how being a patient researcher and advocate, and connecting with others made a difference his GIST journey.

# Day One - History

[Video link](#)

## Session Three

Martin Wettstein, Board Member of the Swiss GIST Group (Switzerland), introduced the final session of Day One. Part one of Clinical Trials was presented by David Josephy and part two was presented by Jim Hughes.



Martin Wettstein



Dr. David Josephy

### Clinical Trials: 2000 - 2021

**David Josephy, PhD**

**President, GIST Sarcoma Life Raft Group Canada**

Dr. Josephy began this session defining clinical trials as medical experiments on patients for new therapies including new drugs, techniques, surgery, radiology, or preventative methods. He then went on to describe the three phases of clinical trials: Safety testing (Phase 1), Efficacy testing (Phase 2), and comparative/standard of care (Phase 3).

Unfortunately, as he related, many trials fail to recruit participants due to low enrollment. Nearly 1/3 of clinical trials fail to involve a single patient, and ½ of clinical trials are not completed because of low enrollment. Dr. Josephy compared clinical trials to real world evidence and explained how the LRG acquires and analyzes real life data (aka real world evidence.)

"Clinical trials are **experiments** – highly organized and complex experiments that test new therapies in volunteers"

"New therapies" may be:

- New drugs (or drug combinations)
- Surgical or radiological techniques
- Diagnostic techniques, e.g., genetic testing
- Preventive methods, e.g. anti-estrogens / breast cancer

"Nearly **one in three** clinical trials fail to enroll a single patient". About **half** "fail to attract enough participants to finish the job, even when they are conducted at dozens of centers".

(Beer and Aymakar)

Greater participation in clinical trials would speed the development of new cancer treatments.

[View PDF of presentation by David Josephy](#)

# Day One - History

Presentation begins at 21:18



Jim Hughes

## Clinical Trials: 2000 - 2021

**Jim Hughes**

**Clinical Trials Coordinator, The Life Raft Group**

Jim Hughes, the LRG's Clinical Trials Coordinator (& an LRG Board Member), shared the personal story of his daughter Nancy's GIST journey from delayed GIST diagnosis in 2003 to her diagnosis of SDHB deficiency in 2014 (post mortem). He recommended discussing the possibility of clinical trials early on with your oncologist, always considering what might be next if/when a current treatment fails.

Jim also touched on state licensing for telehealth trials and treatments noting that, "There will be greater opportunity to treat patients remotely once we have better broadband internet everywhere.

Telehealth is a real gamechanger that allows for a more collaborative environment. We can allow patients to participate in trials who cannot travel. There is a barrier between states. It is a good point for advocacy." He also touched upon some notable trials currently in process.

### Researching Clinical Trials Options

- The LRG curated database
  - Specifically GIST Trials
  - Updated from NIH registry
  - Includes trials that do not specify GIST but have relevance in GIST
  - Does not include all observational studies
  - Includes links to trial reports and drug specific web pages
  - <https://gisttrials.org/iLRG/search.php>

[View PDF of presentation by Jim Hughes](#)

# Day One - History

[Video link](#)

## Session Four

Norman Scherzer introduced long-time friend, CML advocate and patient Giora Scharf for this joint presentation on perspectives from their respective patient communities.



Norman Scherzer



Giora Scharf

### *My CML Story as a Reflection of the Revolution in the Last 20 years*

**Giora Scharf**

**Co-Founder, CML Advocates Network  
Director, Israeli CML Patient Organization, Israel**

Giora shared that he and Norman have known each other since the development of Gleevec and acknowledged many friends among the participants. The CML and GIST communities have many overlapping interests. He called his personal story "From darkness to light". Diagnosed with CML in 2000, Giora was in the Phase III trial for STI-571(imatinib) as a first line drug for CML in Germany, which is the closest country where he had access to this trial. He was in the interferon + chemo arm of the trial and did not fare well and had a grim prognosis. He was switched to imatinib which changed the trajectory of his life forever. Giora has since achieved dreams and goals he had thought he'd never get a chance to experience.

The CML Advocacy Group is global network of leukemia patient groups with the intention of having a directory of all the CML information available, building a social media platform, coordinating awareness campaigns, building partnerships and sharing knowledge. Today, the network has 126 patient organizations globally and is involved in patient-led research, participating in new drug development, educating and empowering patients. Giora concluded with a discussion of what's next in CML research covering access, new treatments, pregnancy, side effects management, adherence, relapse, and future challenges.

# Day One - History

**CML Advocates Network:**  
global network of leukemia patient groups

- Public directory of Chronic Myeloid Leukemia groups
- “Social media platform” to have **advocates** collaborate
- Build skills, coordinate campaigns, build partnerships, share knowledge
- Founded 2007 by 4 advocates
- 126 patient organisations in 95 countries

<http://www.cmlan.org>



[View PDF of presentation by Giora Scharf](#)

**So what are we doing about these challenges?**

- Research to understand patients perspectives (Adherence, TFR)
- Community Advisory boards (CABs) with companies on improving access
- participating in new drug development as members of investigators committee
- Working closely with the CML Hematologists groups like ICMLF, EICML
- Education and empowerment of patients and advocates.

Presentation begins at 18:45

## Lessons Learned from the GIST Community

**Norman Scherzer**

**Executive Director, The Life Raft Group**

Norman talked about lessons learned over twenty years in the GIST community commenting that science is hard and rare cancer research is daunting. A consequence of cancer being non-contagious is that there is no urgency to find new treatments or cure it. In 2020, Covid-19 dominated the world's attention in research due to the sheer number of cases. Contagious = Urgent. Cancer culture is designed in a way that it is not inherently collaborative because there are too many stakeholders working separately with their own agendas. Patients and caregivers are an afterthought.

Over the decades, the LRG has gathered a ‘secret army’ to support GIST patients including doctors, researchers, innovators, politicians, scientists, pharma execs, and advocacy groups. Among the results from these efforts came the NIH’s Pediatric & Wildtype Clinic in which to this day the LRG has an active part. Fueled by the same persistence that birthed the LRG, our current efforts are focused on helping kids with GIST, finding a cure, increasing access to treatments, improving and closing the lethal time gap between scientific advancement and current best practices.

[View PDF of presentation by Norman Scherzer](#)

## UNFINISHED BUSINESS: CLOSE THE LETHAL TIME GAP



Closing the lethal time gap between  
*Scientific Advancement and Best Practices*

- Connect the pertinent diagnostic test to the right targeted drug
- Make it available to the patient community
- Make it affordable to the patient community
- Help the patient manage the drug side effects
- Confirm the patient takes the drug

# Day Two - Current

[Video link](#)

## Session One

Sara Rothschild introduced Dr. Jonathan Noujaim and Dr. Yoon-Koo Kang who presented on current treatment options in GIST.



Dr. Jonathan Noujaim

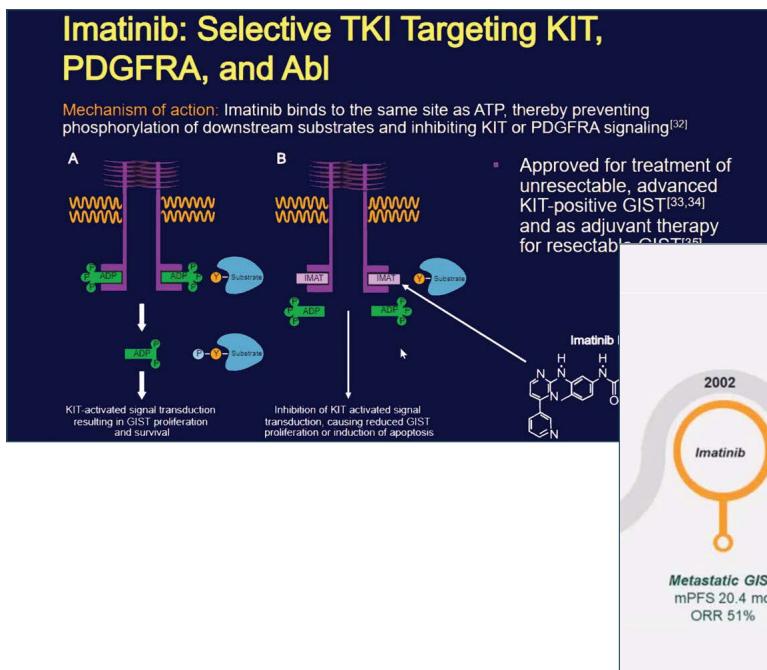
### Current GIST Treatment Options, an Update

**Dr. Jonathan Noujaim**

**Medical Oncologist, Clinical Assistant Professor  
Université de Montréal, Canada**

Dr. Noujaim shared that current treatment options are continually expanding. He briefly shared data from several studies to give an update on the role of adjuvant imatinib for localized resected GIST. A ten-year update showed that three years of treatment on imatinib

showed a significant improvement in preventing recurrences and increasing survival vs. one year of treatment. In this study, more than 20% are still on imatinib and NED. About 12% developed resistance. Dr. Noujaim then reviewed the current standards of care for managing advanced GIST.



[View PDF of presentation by Dr. Jonathan Noujaim](#)

## Day Two - Current

Presentation begins at 19:51



Dr. Yoon-Koo Kang

### Current GIST Treatment Options

**Dr. Yoon-Koo Kang**

**Medical Oncologist  
Professor of the Division of Oncology  
Asan Medical Center, South Korea**

Dr. Kang continued the treatment update theme focusing his part of the presentation on avapritinib (Aykavik) which is indicated for PDGRFa Exon 18 D842V mutant GIST, covering trial data, efficacy, and management of adverse effects. He addressed the subject of resuming imatinib after failure of all available treatments stating that the rationale in oncology is that rechallenge of any chemotherapeutic agents is not recommended. However, Dr. Kang shared that an 'expert consensus' believes that a rechallenge of TKIs that have previously failed can be retried in GIST because there is potential benefit. The final part of this update concerned the surgical resection of residual lesions after control with imatinib.

#### Surgical resection of residual disease after control with imatinib: Rationale

- Pathologic examination reveals that most of the grossly residual lesions contain suppressed but viable cancer cells.
- Clinical resistance to imatinib can develop from these viable cancer cells present in grossly residual lesions (if not resected).
- Resection of these residual lesions can prevent or delay the emergence of clinical resistance to imatinib.

**Study Design: RIGHT**  
**(Rechallenge of Imatinib in GIST Having no effective Treatment)**

Patients with 1) Prior clinical benefit from 1<sup>st</sup>-line imatinib, and 2)  
Progression with both 1<sup>st</sup>-line imatinib and 2<sup>nd</sup>-line sunitinib, (Prior use of 3<sup>rd</sup>-line TKI is permitted)

Stratification:  
1) ECOG PS: 0-1 vs 2-3  
2) Use of 3<sup>rd</sup> line TKI: yes vs no

Presented by: Yoon-Koo Kang      Kang, et al. Lancet Oncol 2013;14: 1175-82      PRESENTED AT: ASCO Annual '13 Meeting

[View PDF of presentation by Dr. Yoon-Koo Kang](#)

This presentation transitioned into the next, which featured Dr. Kang and Dr. Sameer Rastogi of AIIMS, India, discussing global access to treatments.

## Day Two - Current

[Video link](#)

### Session Two

The current treatment options session transitioned into two presentations on global access to treatments.

#### Access Issues in South Korea

**Dr. Yoon-Koo Kang**

**Medical Oncologist,  
Asan Medical Center, South Korea**

[View PDF of presentation by Dr. Yoon-Koo Kang](#)

Presentation begins at 6:44



Dr. Sameer Rastogi

In this session, these doctors shared a snapshot of the healthcare landscape for South Korean and Indian patients regarding access to experts, treatments, drug approvals, clinical trials and healthcare insurance reimbursement, and how some of these challenges might be overcome moving forward. Dr. Rastogi closed the session with this comment, "It is unacceptable for any patient, anywhere in the world, to die from a disease when there is a treatment available."

At 18:00, a Q&A with Dr. Kang and Dr. Rastogi was lead by Pete Knox, Director of Research, The Life Raft Group and Sara Rothschild.

#### Strength of Korea in the Treatment of GIST

- Nationwide medical insurance system
- Easy access to expert physicians and hospitals because Korea is a small country
- Strong patient advocacy group



#### Newer Drugs in GIST - Global Access Issues

**Dr. Sameer Rastogi**

**LRG Medical Advisory Board Member  
Additional Professor, Sarcoma and GIST Clinic  
Department of Oncology, AIIMS New Delhi, India**

#### How Access to Novel Drugs Impacts Treatment

Better access to novel drugs:

- Better decision making towards treatment
- Better quality of care administered
- Improves the disease outcomes

Lessons to learn: Availability of better targeted therapies e.g. Osimertinib is revolutionizing management of lung cancers in India, increasing survival

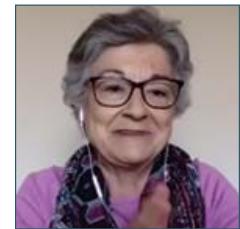
[View PDF of presentation by Dr. Sameer Rastogi](#)

# Day Two - Current

[Video link](#)

## Session Three

Session Three was introduced by Piga Fernández, Executive Director of Fundación GIST Chile and the LRG's Global Relations Coordinator. In this session patient advocates discussed global advocacy efforts in each of their countries. Presenting for this session are Sarah McGoram, Carolina Goič, Florence Thwagi, and Tania Carolina Diaz.



Piga Fernández



Sarah McGoram

to certain treatments. Currently Gleevec and Sutent are the only subsidized treatments available for GIST patients. In response to patient advocacy efforts Qinlock will soon be funded as well. Sarah presents an amazing case study in advocacy.

Presentation begins at 22:16



Senator Carolina Goič

## Advocating for better access: a case study

### Sarah McGoram, Patient Advocate, Australia

Sarah presented a view of the healthcare situation in Australia and how a wide range of healthcare systems at low or no cost could still hinder access

**Mobilising GIST Community**

- Explain the challenges to the patient community
  - Share own story
  - Reach out to individuals
  - Respect boundaries
  - Build trust
- Build on existing networks
  - Personal
  - GIST patient Groups
  - Cancer patient and advocacy groups
- Everyone's story is unique
  - Support patients at every point of the process.
  - Keep GIST community up to date with each step of the process

[View PDF of presentation by Sarah McGoram](#)

The senator shared her story about advocacy from a personal and legislative perspective. She noted that participation is not just one moment, but that it is important to participate in design, drafting, implementation, and evaluation of public policies, and shared the process of creating a 'Cancer Law' for cancer patients.

	National Cancer Law
2012	Creation of the National Cancer Forum
04.11.2014	Entry of the "National Cancer Law" bill into Parliament.
18.11.2018	Public protest for the National Cancer Law
01.04.2019	Digital platform for public participation in the formulation of the National Cancer Law
02.09.2020	Approval and official publication of the National Cancer Law
04.09.2020	Public participation process to determine the contents of the operating regulation of the National Cancer Law
06.04.2021	Official publication of the operating regulation of the National Cancer Law
2021	Election of the National Cancer Commission

Carolina Goič

LEY NACIONAL del CÁNCER

[View PDF of presentation by Carolina Goič](#)

## Day Two - Current

Presentation begins at 37:19



Florence Thwagi

Florence Thwagi, our LRG representative at Henzo Kenya, and Elo Malepu, Chairman of Henzo Kenya, a patient advocacy organization in Kenya, Africa, shared the challenges of GIST patients in their country, one of which was that Glivec was the only treatment available and it was administered in a central location proving it difficult for patients to access. Henzo Kenya worked to overcome what treatments were available, provided educational awareness of GIST and worked to advocate for zero insurance reimbursement. Florence also shared the successes thus far and the current challenges facing patients in Kenya.

### Best Practices, How to Advocate for Better Access

#### Florence Thwagi

#### Patient Advocate, Henzo Kenya

Florence Thwagi, our LRG representative at Henzo Kenya, and Elo Malepu, Chairman of Henzo Kenya, a patient advocacy organization in Kenya, Africa, shared the challenges of GIST patients in their country, one of which was that Glivec was the only treatment available and it was administered in a central location proving it difficult for patients to access. Henzo Kenya worked to overcome what treatments were available, provided educational awareness of GIST and worked to advocate for zero insurance reimbursement. Florence also shared the successes thus far and the current challenges facing patients in Kenya.

#### Challenges

- ▶ Limited resources for patient's monitoring and follow ups.
- ▶ Government Bureaucracy delays policy implementation and drugs delivery
- ▶ We only have access to 1<sup>st</sup> and 2<sup>nd</sup> line treatments which is a challenge for patients with advanced Gist and non responsive mutations.
- ▶ No mutation testing.

[View PDF of presentation by Florence Thwagi](#)

Presentation begins at 51:11



Tania Carolina Diaz

### Access for Patients in Colombia

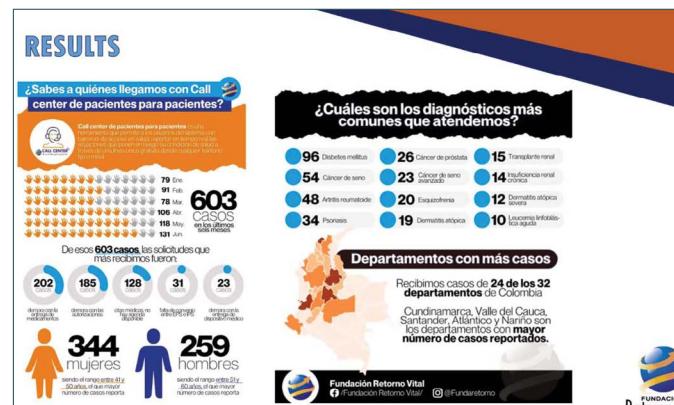
#### Tania Carolina Diaz

#### Projects & Program Analyst, Fundación Retorno Vital, Colombia

Tania is a 24-year-old cancer survivor,

anthropologist, and data analyst who works with a non-profit that supports patients with many chronic and high-cost health conditions.

Tania shared Retorno Vital's work to solve Colombia's problems with healthcare access with personalized guidance and support for patients, by building strategic alliances, improving therapeutic adherence, impacting public policies, identifying problems, raising awareness, and building a national support network as well as current advocacy challenges.



[View PDF of presentation by Tania Diaz](#)

# Day Two - Current/Day Three - Future

## Discussion on Access Issues and Potential Solutions

At 1:03:01, this panel, led by Piga Fernández, answered questions and discussed challenges in policy around patient representatives in decision-making/stakeholder positions.

## Day Three Session One

GIST Treatment Updates was moderated by Dr. David Josephy, Director, GIST Sarcoma Life Raft Group Canada, with a discussion afterwards on 'How do we address unmet research needs?"

### [Video link](#)



Dr. Ciara Kelly

## Immunotherapy & GIST

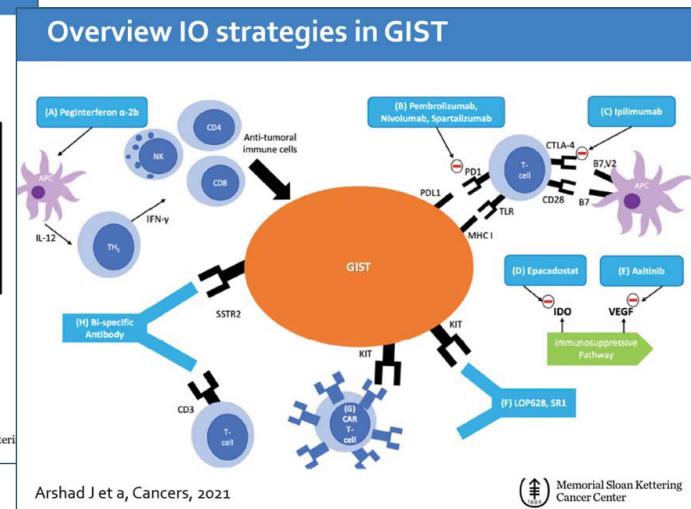
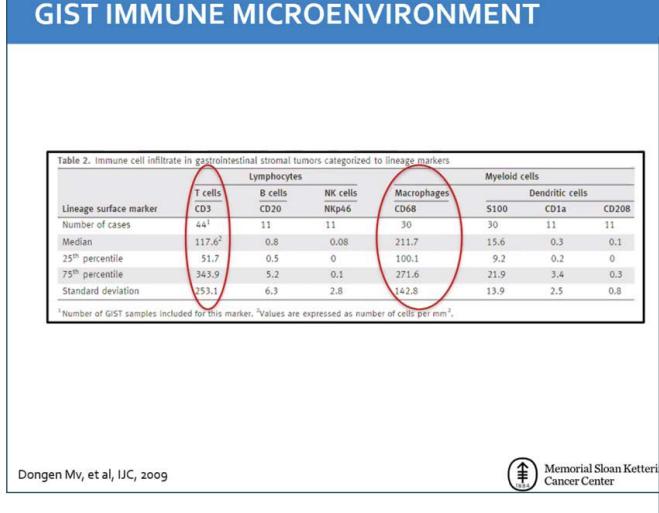
Dr. Ciara Kelly

Assistant Attending, Sarcoma Medical Oncology Service  
Memorial Sloan Kettering Cancer Center, New York

Dr. Kelly explained the GIST immune microenvironment and talked about cytokine-based therapy, the significance of immune checkpoint blockage, and its use in combination with other therapies such as chemotherapy, IDO1 inhibition, and TKIs. Not every treatment option

has all positive results. Dr. Kelly suggested that combination studies may be necessary. Several IO studies in GIST are currently in progress and the correlative data will be important to future IO trial design in GIST.

[View PDF of presentation by Dr. Ciara Kelly](#)



# Day Three - Future

Presentation begins at 19:57



Dr. Suzanne George

## GIST Future Research Directions: Highlights from recent Phase 1 trials and future directions

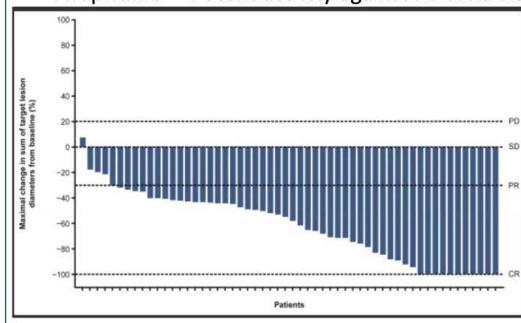
Dr. Suzanne George

Clinical Research Director, Sarcoma Center  
Dana-Farber Cancer Institute, Boston

Dr. George discussed goals of Phase 1 clinical trials - determining optimal dose and side effects. She covered evaluating new combinations of drugs that have been studied and creating expansion cohorts to look for further signals or benefit, and the translation of that research from bench to bedside. Dr. George also shared Phase 1 trial data from avapritinib which shows potent activity against PDGFR $\alpha$  D842V mutant GIST and the Phase 1 trial that examined ripretinib versus sunitinib as a second-line treatment, explaining that all currently approved treatments for GIST are KIT inhibitors and new studies are exploring combining KIT inhibitors with drugs that target other mechanisms of action (such as a KIT inhibitor with a MEK inhibitor.)

### Phase I trials have led to drug approvals in GIST

- Avapritinib – Potent activity against PDGFR $\alpha$  D842V mutant GIST

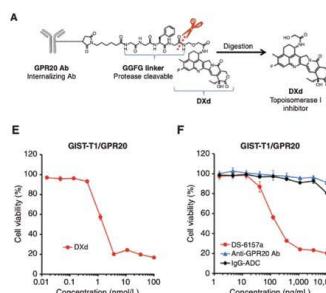


GPR20 is a novel protein expressed on GIST cells

- DS-6157a
- Antibody-drug conjugate is being studied to see if this new finding can be used to control GIST

DS6157a is being studied in Phase I trial

Iida et al, Cancer Discov. 2021 Jun;11(6):1508-1523



Dana-Farber  
Cancer Institute

[View PDF of presentation by Dr. Suzanne George](#)

# Day Three - Future

Presentation begins at 37:57



**Medical Oncologist, Associate Professor of Medical Oncology University Campus Bio-Medico, Rome, Italy**

**Dr. Bruno Vincenzi**

**Medical Oncologist, Associate Professor of Medical Oncology University Campus Bio-Medico, Rome, Italy**

Dr. Bruno Vincenzi

Dr. Vincenzi shared the current lines of research at the University. First, the role of different doses of adjuvant imatinib in patients for KIT exon 9 mutation, the effects of treatment with adjuvant imatinib on bone density and sarcopenia, and overcoming imatinib resistance in GIST. Part of that last study concerned the hypothesis that GIST cells increase Cyclin D1 expression and launching an exploration of Cyclin D1 expression and imatinib resistant tumor spread.

Current lines of <i>clinical</i> research		
1. ROLE OF DIFFERENT DOSES OF ADJUVANT IMATINIB (400 MG/DAY vs 800 MG/DAY) IN PTS WITH RESECTED KIT EXON9-MUTATED GIST		
2. EFFECTS OF TREATMENT WITH ADJUVANT IMATINIB ON BONE DENSITY AND SARCOGENIA		

Current lines of <i>translational</i> research		
1. TRANSCRIPTOMIC DIFFERENCES BETWEEN GIST WITH DIFFERENT MUTATIONS		
2. ESTABLISHMENT OF PATIENT-DERIVED CELL LINES WITH RARE K		

Adjuvant imatinib in Exon 9 GIST																																						
Table 2: Multivariate weighted Cox analyses																																						
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[View PDF of presentation by Dr. Bruno Vincenzi](#)

## How do We Address Unmet Research Needs?

At 53:56: Panel discussion and Q&A led by Dr. David Josephy followed these GIST updates with questions such as 'what are the research tools that we need?', and 'how can developing countries participate and promote GIST research?'

# Day Three - Future

[Video link](#)

## Session Two

Sara Rothschild introduced session two of the final day of New Horizons GIST featuring and SDH-deficient GIST Research Update



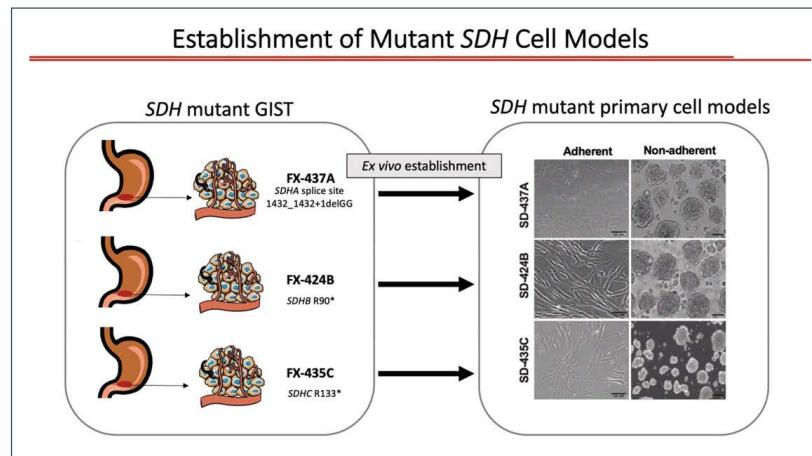
Dr. Shruti Bhargava

### ***Establishment of Patient-derived Succinate Dehydrogenase-deficient Gastrointestinal Stromal Tumor (GIST) Models for Predicting Therapeutic Response***

**Dr. Shruti Bhargava**

**Postdoctoral Fellow, Division of Surgical Oncology  
Department of Surgery  
University of California San Diego  
Moores Cancer Center**

In this very detailed presentation, Dr. Bhargava reviewed the general characteristics of GIST, and then explored the mechanism of SDH-deficient GIST extensively, including Carney Stratakis syndrome and Carney Triad. In the research at Moores Cancer Center, scientists have established SDH mutant cell models for SDHA, B, and C. These cell models express GIST markers and SDHB loss, and are fairly similar to the parent tumors. These models function like the parent and affect the cells with similar malfunction like, hypoxia, gene upregulation, and the typical insensitivity to imatinib and sunitinib. Established to mimic the parent, the models were then tested against temozolomide. Dr. Bhargava shared this data and future direction for these models.



[View PDF of presentation by Dr. Shruti Bhargava](#)

After the presentation Sara Rothschild shared a Q&A time with Dr. Bhargava covering questions such as 'what help do researcher scientists need to move research forward?', 'Are researchers considering immune combination therapies in wildtype GIST?', and 'can advanced SDH-deficient GIST have different mutations in different sites?'

# Day Three - Future

[Video link](#)

## Session Three

For the session on "Using Real World Evidence to Impact Future Clinical Trials and Improve Patient Survival: A Focus on Placebos", Life Raft Group's Sara Rothschild introduced Pete Knox with the final presentation of the New Horizons GIST Conference.



Pete Knox

### Rethinking Placebos - And what's RWE have to do with it?

**Pete Knox**

**Senior Director of Research  
The Life Raft Group**

Pete Knox explores the role of placebos in clinical trials and proposed that there is enough clinical trial evidence to do away with placebos in trials for treatments for advanced GIST patients. Studies clearly show that placebos in these trials shorten lives. Anecdotal information from patients, collecting information PFS and OS from the LRG registry and combining this information with placebo participant data inform this declaration.

The diagram consists of two main sections. On the left, a blue box contains the question "How does RWD/RWE fit into the overall data process?". Below this is a list of bullet points explaining its role: Complementary to traditionally acquired (clinical) data; Designed to supplement, **not** replace; Helps researchers see more of "the whole picture" (especially the "patient perspective"); Is increasingly being looked at or even required (i.e. 21<sup>st</sup> Century Cures Act); Is a "work in progress" as standards are still in development. To the right of this section is a graphic of four interlocking puzzle pieces in yellow, green, blue, and red. On the right, another blue box contains the question "But what does RWD/RWE have to do with placebos?". Below this is a list of bullet points: Anecdotal information obtained from years of talking to patients (RWD) has led us to believe that placebos have a harmful effect. (hypothesis generation); Collecting information on PFS and OS in our registry (RWD) and further analyzing it can provide evidence (RWE) to support that hypothesis.; Combining this information with that obtained from placebo participants in clinical trials gives a clearer and more complete idea of what may be happening. To the right of this section are three icons: a bar chart, a pie chart, and a line graph, followed by a bottle labeled "PLACEBO TABLETS" and two spoons.

[View PDF of presentation by Pete Knox](#)

After this final presentation, Sara Rothschild moderated the Q&A with Pete Knox and covered questions such as 'what is the FDA's role?', and 'how long do we see this change to trials happening?'

# Evaluation Report

- 42 participants of the New Horizons 2021 Conference responded to the post-conference evaluation through Google Forms.
- 100% of responding participants were satisfied or very satisfied with the virtual setting of the event as well as the content of the event sessions.
- 100% of responding participants were very satisfied with the organization of the event.
- 100% of responding participants were satisfied or very satisfied with the speakers or presenters.



Participants enjoyed the new information and research that was presented by GIST specialists from around the world. Doctors who attended feel they are more comfortable discussing Mutational Testing methods with patients.

One participant replied:

"Once again New Horizons assembled an amazing and diverse group of speakers...The facilitation of the conference and the sessions was so skillfully done... the event ran very smoothly and included sufficient break time, which is always important. I also liked the ad hoc discussions that emerged among the presenters... and I especially liked that attendance was opened up this year for patients and caregivers...and I really appreciate that the Slides and Videos are posted on the website. This is such a great conference."

When asked what participants gained from the conference, several participants replied "hope". One participant replied "Greater insight into (1) GIST research and (2) evolving best practices for treating advanced GIST and (3) a real appreciation for the research work being performed by bright, dedicated and articulate researchers, who kindly took the time to share their work and ideas with us."

# New Horizons GIST Conference 2021

## Agenda

### October 20

Time (Eastern Time/NY)	Presentation
9:00-9:45 am	"In the Beginning...The Story of How GIST Treatments Began" <i>Dr. George Demetri, Dana Farber Cancer Institute</i>
9:45-9:50 am	<i>Chuck Korte, a long-term GIST Survivor</i>
9:50-10:00 am	10 min break—Coffee Chat
10:00-11:00 am	Clinical Trials: From 2000 to 2021 <i>Dr. David Josephy, GIST Sarcoma Life Raft Group Canada</i> <i>Jim Hughes, Life Raft Group</i>
11:00-11:15 am	15 min break—Coffee Chat
11:15 am-12:00 pm	Lessons Learned from the GIST & CML Community <i>Giora Sharf, CML Advocates Network</i> <i>Norman Scherzer, Life Raft Group</i>

### October 21

Time (Eastern Time/NY)	Presentation
9:00-9:30 am	Current Treatment Options <i>Dr. Jonathan Noujaim, Université de Montréal, Canada</i> <i>Dr. Yoon-Koo Kang, Asan Medical Center, South Korea</i>
9:30-10:00 am	Global Access to Treatment <i>Dr. Yoon-Koo Kang, Asan Medical Center, South Korea</i> <i>Dr. Sameer Rastogi, AIIMS, New Delhi, India</i>
10:00-10:15 am	Q&A
10:15-10:30 am	15 min break—Coffee Chat
10:30-11:30 am	Best Practices: How to Advocate for Better Access <i>Sarah McGoram, Patient, Australia</i> <i>Senator Carolina Goic, Chile</i> <i>Florence Thwagi, Patient, Elo Mapelu, Chairman, Henzo Kenya</i> <i>Tania Carolina Diaz, Program Analyst, Retorno Vital, Colombia</i>
11:30 am -12:00 pm	Discussion on Access Issues and Potential Solutions

### October 22

Time (Eastern Time/NY)	Presentation
9:00-10:00 am	GIST Research Updates <i>Dr. Ciara Kelly, Memorial Sloan Kettering Cancer Center</i> <i>Dr. Suzanne George, Dana Farber Cancer Institute</i> <i>Dr. Bruno Vincenzi, University Campus Bio-Medico, Rome</i>
10:00-10:30 am	How do We Address Unmet Research Needs? <i>Dr. David Josephy, Facilitator</i>
10:30-10:45 am	15 min break—Coffee Chat
10:45 -11:15 am	SDH-deficient GIST Research Update <i>Dr. Shruti Bhargava, University of California, San Diego</i>
11:15am-12:00 pm	Using Real World Evidence to Impact Future Clinical Trials and Improve Patient Survival: A Focus on Placebos <i>Pete Knox, Life Raft Group</i>



## Conclusion

In summary, participants learned about the latest clinical and lab-based research updates and gained perspective on issues of access and advocacy across the globe. A special thank you goes out to all who presented and all who attended.

All presentations are available here:  
<https://liferaftgroup.org/new-horizons-2021/>

