



## The Life Raft Group Annual Report 2021

Our theme for 2021 was “It’s Time.” The year challenged us all, but most especially our patients and caregivers who were striving to survive this rare cancer. In 2021, The Life Raft Group acknowledged that as more scientific breakthroughs in research were continuing, patients were still not receiving the most effective diagnostic tests to assure they were prescribed the most effective personalized treatment. We launched our It’s Time Campaign to encourage standardization of advanced mutational testing for all GIST patients.

### Our Executive Director’s Message

Our major theme in 2021 was “It’s Time” as we launched our campaign to close the lethal time gap between the availability of diagnostic (mutational) tests critical to matching each patient with the best targeted drug and their actual usage by the prescribing physician. It is ironic that the further we push our scientific platforms for personalized medicine, the more difficult it is for the average physician to keep up with those advances as part of best practice.

To address that issue, we pursued a three-part strategy.

1. Educate general physicians, particularly oncologists.
2. Keep identifying expert GIST specialists and encourage patients to seek them out, at least for a consult.
3. Educate and empower patients and their caregivers.

It is tragic for anyone to die from a rare cancer for which there is no available targeted treatment. It is simply unacceptable for anyone to die from a rare cancer for which there is a targeted treatment that was not prescribed due to medical ignorance of the right mutational test. In 2022, the Life Raft Group will continue to work hard to close this lethal time gap.

There is no greater gift than to help save a life.

We stand on the shoulders of so many unsung patient and caregiver heroes, including too many who have been taken home by their angels. Let’s not disappoint them.

- Norman J. Scherzer, Executive Director (2000-March 2022)



## Research

In 2021, the research community continued to adapt to deal with the ongoing challenges of the Covid 19 pandemic. New models for clinical trials were developing, with the continued availability of telemedicine. With more sophisticated methods of identifying biomarkers, new opportunities for research into treatments for rare subsets became a priority.

The LRG launched an [It's Time Campaign](#), creating new materials and communication vehicles to educate patients, caregivers and medical professionals about the importance of biomarker/mutational testing.

Our Global Surveillance Group collaborated on a number of unusual case studies, providing information that shaped clinical interventions. Topics ranged from Carney Triad and pregnancy to lines of recommended treatment for complex metastatic cases. Patient cases discussed were from various countries including Brazil, India, Portugal and the United Kingdom.

We continued to publish [LRG Science](#), our quarterly publication dedicated to reporting data and research, both traditional and patient-generated as it relates to GIST. The four issues reflected relevant topics including: "Meet The Life Raft Group Medical Advisory Board", "The Importance of Biomarker Testing for Effective Treatment of GIST Patients", "It's Time to Rethink Placebos in Advanced GIST", and "Additional Commentary on 'It's Time to Rethink Placebos in Advanced GIST'".

Our Real World Evidence Team continued to meet on a weekly basis to discuss the data generated from the [Life Raft Group GIST Patient Registry](#), and to share that data where it would generate interest or have impact on research and treatment development. We invited guest speakers to present on topics of interest. Part of that data gathering included a number of surveys, including topics such as: Medicare/Medicaid and Gleevec/Imatinib Patient Assistance Programs Survey; a Mutational Testing Survey/Poll; a Qinlock/Ripretinib/ Experience Survey; and a Patient Registry Medical Update Outreach Survey.

We continued to explore and expand the concept of personalized side effects management, revamping our Patient Registry to efficiently capture data on side effects common to our patients.

Two members of our Patient Registry team presented at virtual international conferences, one in India, the other in Chile. Several staff members attended the virtual CTOS conference in

November, bringing back information on trends in GIST research and treatment. In addition, we published informal case studies and interviews with GIST experts on the latest treatments in our newsletter and discussed pertinent clinical trials.

As founding members, the LRG continued to participate virtually in the Pediatric & Wildtype Clinic at the National Institutes of Health (NIH) by organizing and facilitating several Virtual GIST Tumor Boards. Due to the pandemic, the NIH Clinic couldn't be held in person, so they requested that the LRG facilitate a Virtual Clinic. The LRG identified patients who qualified for the clinic, collected data, and created a relevant slide deck, relaying outcomes to physicians and patients.

We continued to be a resource for pharmaceutical companies, aiding them in the development of and the recruitment for clinical trials. Collaborating with Bayer Pharmaceuticals, we facilitated next generation sequencing for more than 54 patients, identifying two NTRK patients. Collaborations with Blueprint Medicines and Deciphera Pharmaceuticals focused on data collection and summary and analysis of the data.

New clinical trials continue to hold out promise for new treatment modalities, as combination therapy and immunotherapy are explored. Our participation in CTTI (Clinical Trials Transformation Initiative) gives us a seat at the table and a voice in the development of innovative clinical trial design.

Our most important efforts remain our impact on patient survival. Several case studies emerged that demonstrated that our efforts directly aided patients in increased survival and quality of life, emphasizing the importance of biomarker/mutational testing.



## Outreach and Support

The Life Raft Group staff continued to reach out by phone, emails, and through new programs we created to serve our patients and caregivers.

We welcomed 249 new members into our community.

Our [GIST Mentor program](#) has continued to expand, with 27 mentors and over 200 mentees now engaged. The program expanded this year, creating an International Mentor Program with

eight mentors from five countries. Support for mentors is provided through a monthly meeting and trainings.

Our ongoing support groups for patients, SDH patients and for caregivers continued to grow, meeting virtually. We now service over 100 participants. We included one support group specifically for women this year. Ongoing regional support groups continued to meet virtually.

The heart of our outreach and support efforts is the Outreach & Engagement team, who continue to provide personal attention to patients and caregivers on a daily basis. The team aims to offer information and advice to help patients survive and thrive. These efforts are enhanced by the dedication of our Patient Registry Team, who work individually with patients via phone calls and email correspondence, from the initial call to regular medical updates, helping them to navigate their treatment journey. This was especially important during the stress of the ongoing pandemic, when patients were feeling anxious.

Support also was provided by helping patients navigate finding telemedicine appointments and guiding them towards clinical trials.

**[Member stories](#)** are powerful tools to evoke both hope and provide information. We continued to distribute our Patient of the Month and Caregiver of the Month profiles, highlighting a member of our Patient Registry on our website and in our newsletter, thereby bringing the patient journey to life.

We continued to support the GIST community in times of celebration, and in times of loss. We offered support for those grieving on our **[In Memoriam](#)** website, and called attention to this type of loss in our holiday campaign.

**[GIST Days of Learning](#)** (GDOLS) were held remotely in: California, NY/NJ Region, Pittsburgh, Phoenix and Miami, providing 130 patients, caregivers access to the latest information on GIST treatments and research, as well as providing a forum for supporting each other.

Events remained an important part of networking with members of our community, as well as presenting the Life Raft Group to the community-at-large.

GIST Awareness Day is celebrated each year on July 13. Our theme of “It’s Time” highlighted the importance of mutational testing, with global organizations joining in to advocate for this important cause. Our social media campaign alone reached over 9,000 people. The LRG presented a Q&A webinar on SDH-deficient GIST presented by members of our Pediatric & SDH-Deficient GIST Consortium.

Important fundraisers for the LRG continued in 2021, although virtually. Our Night to Fight Cancer event was hosted online in January and in November by LRG Board President Jerry Cudzil and Matt Knopman, providing an evening of fun and fundraising. Between the two events, over 145 guests enjoyed the fun. The LRG supported two unique Water of Life events,

hosted by Dr. Matt Lurin. Dr. Lurin hosts these unique whiskey tasting events, that are popular even in a virtual setting. New this year was the Water of Life Feis Off, which engaged 75 whisky aficionados in July. The annual WOLF (Water of Life Fall) event in November drew 55 participants virtually. Both events were sold out. It is an accomplishment when recognized that the attendance in these virtual events matched or exceeded the number of participants for previous in-person events.

Our monthly giving program [\(GEMs\)](#) expanded from five to 30 monthly donors generating over \$13K of additional funding per year. Some of these monthly donors are also volunteers.

Our Annual Holiday Campaign, "GIST Wishes", focused on the wishes we have to help our community survive and thrive, as well as on the 'wishes that came true' for our patients and caregivers who have been able to experience valuable life events because they have received effective treatment. The ongoing support of our GIST community continues to fuel our efforts in both research and patient support.

In addition to extraordinary support for our GIST community, in 2021 we mentored other disease groups called Tenosynovial Giant Cell Tumor (TGCT) and Giant Cell Tumor of the Bone, rare diseases whose communities are in the beginning stages of establishing resources for their patients and caregivers. Mentoring extended to the mentoring/training of TGCT and GCT volunteers in establishing support groups, mentor programs and fundraising initiatives.



## Information, Education and Advocacy

Real world evidence indicates that empowered and educated patients have higher survival rates, and that by raising awareness and providing information about GIST, we can help save lives.

### *Information & Education*

Educating patients and caregivers as well as the community-at-large is a core function of the Life Raft Group. From the initial call when we connect with a patient, to ongoing webinars presented by medical experts, regional GIST Days of Learning meetings, an article in our Newsletter, content on our website or through our biennial Life Fest conference, we are

continually encouraging patients to take a greater role in their treatment journey and providing the resources to help them.

In 2021, our website continued to be an information hub for newly diagnosed patients and for anyone seeking general information about GIST. We had 208,100 page views on our site from 206 countries this year alone. Through our posts and articles, we educated patients about new treatments and about navigating clinical trials.

Informative webcasts in 2021 included:

- hPPGL Syndrome, Finding Biomarkers that Cause GIST
- Conversations with International Mentors
- What Testing Means for Me and My Family
- Conversations with Long Term Survivors
- Mutational Testing for the Global Community
- Management of Metastatic GIST
- New Treatment Updates for Advanced GIST
- SDH Q &A for GIST Awareness Day
- Clinical Trials in GIST
- Conversations with International Mentors
- Perspectives in Biomarker Testing
- Caring for the Caregiver
- Immunotherapy in GIST
- Personalized Medicine and Mutations in GIST

Our newsletter is an important source of information for our member community, sharing inspiring personal stories, informative articles on the latest trends in GIST treatment and updates on the LRG's collaborative efforts worldwide. The LRG Science Bulletin provides scientific content to educate and inform and engages GIST specialists and researchers in commentary.

At all of our GIST Days of Learning, informative presentations reach a larger regional audience, providing important information for patients and caregivers. We managed to hold these virtually in California, New York/New Jersey, Pittsburgh, Phoenix and Miami.

### *Advocacy*

The Life Raft Group continues to be a powerful advocate for change for patients and caregivers both nationally, and internationally.

We advocated personally on a case-by-case basis for patients to be able to access treatment including through telemedicine, as well as participated virtually in several organizations both nationally and internationally as collaborative efforts.

On the international front, our Global Relations Coordinator, Piga Fernández continues to aid patients around the world, while making a vital impact in Latin America. Her ongoing efforts have influenced legislation to support access to cancer treatment in Chile despite working through a lockdown.

Executive Director Norman J. Scherzer joined a renowned group of speakers for the Chronic Disease Day Advocacy Training Session which was presented live on Facebook by Good Days and United for Charitable Assistance. The training session focused on “Telling Your Story and Using Your Voice to Advocate for Progress”.

We continue to be involved in NORD (National Organization for Rare Disorders), with staff members virtually attending their annual conference and meetings of the Rare Disease Council of NJ. Carolyn Tordella, Assistant Director of Communications, both attended and presented at the NORD Rare Summit on a panel on diversity issues.

We maintain a presence as members of the Sarcoma Coalition joining with other rare disease non-profits to share best practices and discuss national advocacy issues with pharma, patients, and patient advocacy organization staff. The LRG remains part of the Milken Institute's Faster Cures, as one of the original members of TRAIN (The Acceleration and Innovation Network). Locally, we maintain ties with other non-profit organizations by regular attendance at conferences and roundtables, albeit virtually this year.

Through the power of social media, we continue to rally our global community in support of issues relating to the access and affordability of treatment. We also reach out to the global community via the Facebook Group we moderate named GIST Survivor, which engages hundreds of international patients and caregivers.

We launched our It's Time Campaign to advocate that all GIST patients receive mutational testing, which was strengthened by our Tissue Testing initiative with Bayer and Tempus to provide free genomic sequencing for eligible patients. Our voice was heard globally concerning this important issue.