



Annual Report 2017

CELEBRATING

15
years



It is our tradition to ask the Executive Director of the Life Raft Group to write an article for our Annual Report. You would think that after doing this so many times it would get easier. After all, we can recount so many accomplishments this past year in expanding our educational and research efforts and forming new high-level collaborations. Instead of doing that, however, permit me to turn to the topic of courage.

The Merriam-Webster dictionary defines courage as “the mental or moral strength to venture, persevere and withstand danger, fear or difficulty”. The instinct when reading that is to turn one’s attention to the military or to their civilian brothers and sisters in police and fire departments, all of whom stand in harm’s way. Their actions flash clearly in our minds as we imagine the visions of those who walk into the burning fire or across the field of battle. Rightly so, we honor them with medals and ceremony and hold them up as role models who gave much, if not all, to defend our ideals.

There is a less visible kind of courage that I would like to talk about.

A mom, newly diagnosed with cancer, strokes the heads of her children to reassure them that everything will be all right as she hides the sheer terror of her new status.

A son and daughter giving their consent to their dying father that it is o.k. for him to let go as they hide the total helplessness of losing someone they love so dearly.

Or the wife who, racked with pain, gets quietly out of bed to go to the bathroom, careful not to wake and concern that man lying next to her. And, of course, that man now wide awake, pretending not to notice the pain his wife is in.

Courage on the military battlefield often plays out in minutes.

Courage on the cancer battlefield often plays out into endless time.

On behalf of those who work in the Life Raft Group, I would dedicate this past year to the courage of the men and women of all ages and nationalities who venture forward every day to withstand the danger, fear or difficulty of cancer. Whatever we do or accomplish pales in the face of your bravery.

Thank You,

Norman Scherzer
Executive Director



LRG Members and Staff at GDOL NYC 2017

At the heart of the Life Raft Group, the support and education of patients is what empowers them to take a larger role in their care. Simply stated, empowered patients have higher survival rates.

In 2017, we held four GIST Days of Learning (GDOLS) in Miami, Salt Lake City, New York and San Diego. GDOLs continue to be an effective way to provide both the latest education on topics related to GIST presented by experts in the field, as well as serving as a forum for patients and caregivers to connect with one another in

a supportive environment. This year, clinics were offered by the physician groups from associate institutions, and patient stories became a part of the event.

GDOLs would not be possible without the generous support of sponsors such as Bayer, Blueprint Medicines, Novartis, and Pfizer.

We presented five webcasts, with topics ranging from Gene Fusions in GIST to information on clinical trials for DCC-2618 and BLU-285.

Newsletters, providing timely topics including a Clinical Trials focused issue, continue to be published. Each issue included member stories, updates on patient-powered science themes, and stories with global reach.

The LRG continues to be a strong voice for GIST patients, advocating for access to GIST specialists, clinical trials, and mutational testing.

We continued to maintain membership in national and local organizations such as NORD (National Organization for Rare Disorders) and OVAC (One Voice Against Cancer), participating in advocacy campaigns through both our email community and social media.

The LRG joined the efforts of the Marketplace Access Project (MAP), a group of leading national patient advocacy organizations dedicated to protecting non-profit insurance premium and cost-sharing assistance to individuals with chronic and life-threatening illnesses. Currently, the project continues to urge the Centers for Medicare and Medicaid Services (CMS) to modify a federal rule that allows insurance companies to deny coverage to patients who receive premium or cost-sharing assistance from non-profit charities. Support for the Access to marketplace Insurance Act (H.R.3976) continues to be part of this campaign. The bill was introduced on October 5, 2017, and is in the first stage of the legislative process.



Hannah Marcus, Karen Meyers, Teena Petersohn, and Marisa Bolognese at OVAC Lobby Day in D.C.

On a very local basis, we encouraged our New Jersey members to take action in support of Bill A2337 in the General Assembly, which requires health insurers to limit patient cost-sharing and provide an appeal process concerning certain prescription drug coverage. We participated in local networking opportunities, along with lobby visits at the statehouse.

LRG Members Teena Petersohn, Hannah Marcus and Karen Meyers participated in OVAC's Lobby Day in Washington, D.C., visiting their legislators to lobby for the preservation of funding for rare disease research.



Outreach and engagement also expanded in 2017. Working with state leaders and volunteers, we have been able to reach more patients, advocacy groups and medical professionals than ever. In conjunction with the Patient Registry department, brief surveys were conducted each month on a variety of topics to help promote our programs.

Our community continues to be a valuable source of support, with 30 leaders representing 25 states, and international representatives from 61 different countries.

Our fourth annual GIST Awareness Day was a weekend affair in Southern California, and included fun activities around our H.E.R.O. campaign, as well as a GDOL, a clinic day, and a tour at Moores Cancer Center. From coast-to-coast, members raised awareness from state fairs to local meetings. The international community pulled out all stops with their billboard style artwork.



LRG Members and Staff at our GIST Awareness Day in La Jolla, CA

The 14th Annual Night to Fight Cancer event hosted by Board President Jerry Cudzil along with Matt Knopman, was once again a success, raising close to \$200,000 for research while providing a night of good food and fun for participants.

Global GIST advocates from 15 countries convened in October for the New Horizons GIST Meeting at the LRG's Wayne, New Jersey office. This international meeting focused on how disease advocacy organizations can utilize real world evidence from patient health records to advance GIST research. A key issue of concern was obtaining access to diagnostic testing and cutting-edge treatments. The seventh annual meeting of Alianza GIST took place concurrently.

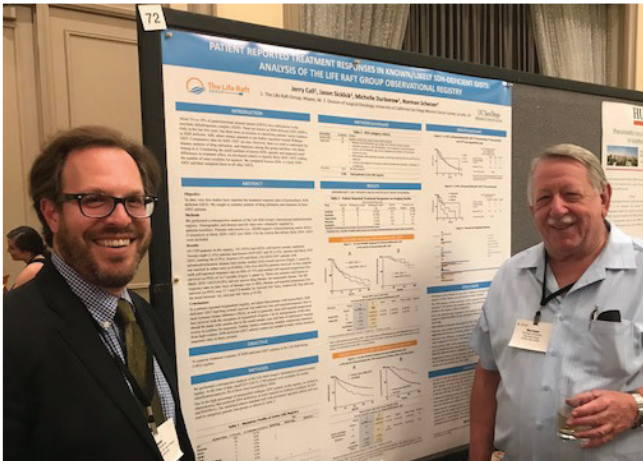
Efforts in Latin America continued to focus on raising awareness and providing access to treatments. Fundación GIST Chile expanded collaborative efforts with other Chilean advocacy organizations in a newly formed Association of Cancer Groups in Chile called ACHAGO, combining efforts to be able to achieve public policies that enable patients to have access to the proper and accurate diagnosis as well as the treatments patients need to survive.



New Horizons Meeting Participants at the 2017 meeting held in Wayne, NJ

Individual patients were aided by the Life Raft Group in obtaining treatment in countries from Croatia to Australia.

With 30 state representatives and 61 international representatives, the voice of the LRG can and will continue to be heard.



*Norman Scherzer and Dr. Jason Sicklick
presenting a poster at CTOS*

In 2017, our approach to research emphasized “Patient Powered Science for Life”, a paradigm based on one of the core premises of the Life Raft Group – real-world evidence provided by and for patients.

This approach is not new. Since the earliest days of the LRG we have believed that patient data can provide us with critical insight into survival strategies in a timely and accurate manner, bypassing the lethal time lag that is part of traditional clinical trials. The combination of the traditional research studies we support combined with patient data from our platforms fill a critical gap.

With Project InterGR, a comprehensive suite of research tools including our Patient Registry, SideEQ and GIST Collaborative Tissue Bank, we carved out a niche in GIST research, laying

the groundwork for exciting new studies, including a global surveillance project with key GIST specialists, providing timely real-world evidence to increase patient survival and enhance quality of life.

Our flagship Patient Registry was enhanced with the release of GIST/PRIME, an interactive component of the registry that allows patients to be involved in research directly.

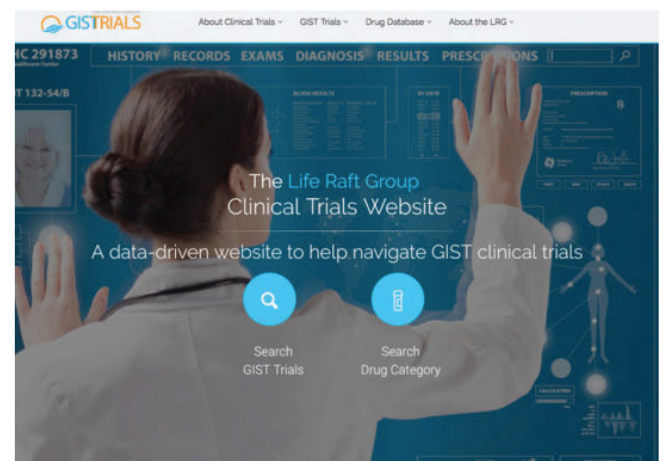
SideEQ, our online side effects management tool, was opened to three additional sarcoma patient organizations. The system was enhanced to track side effects over time.

Our redesigned and optimized GIST Clinical Trials database now provides a more user-friendly and intuitive search of trials for patients and physicians.

Continuing our collaboration with Columbia University Medical Center NewYork Presbyterian, we have provided 150 tissue samples. Dependent upon funding, the next phase will be a clinical trial. With Blueprint Medicines, We participated in a data project to aid in trial design and to analyze progress and remaining medical need in a GIST patient’s journey based on patient-reported real-world experience.

Continuing to encourage patient-powered science globally, we supported data sharing in Latin America through the Salud con Datos project. The goal of the project is to expand the Latin America GIST Registry, to close the information gap and aid in understanding the needs of GIST patients in Latin America, and to translate data to health authorities and medical professionals with the goal of improving patient outcomes.

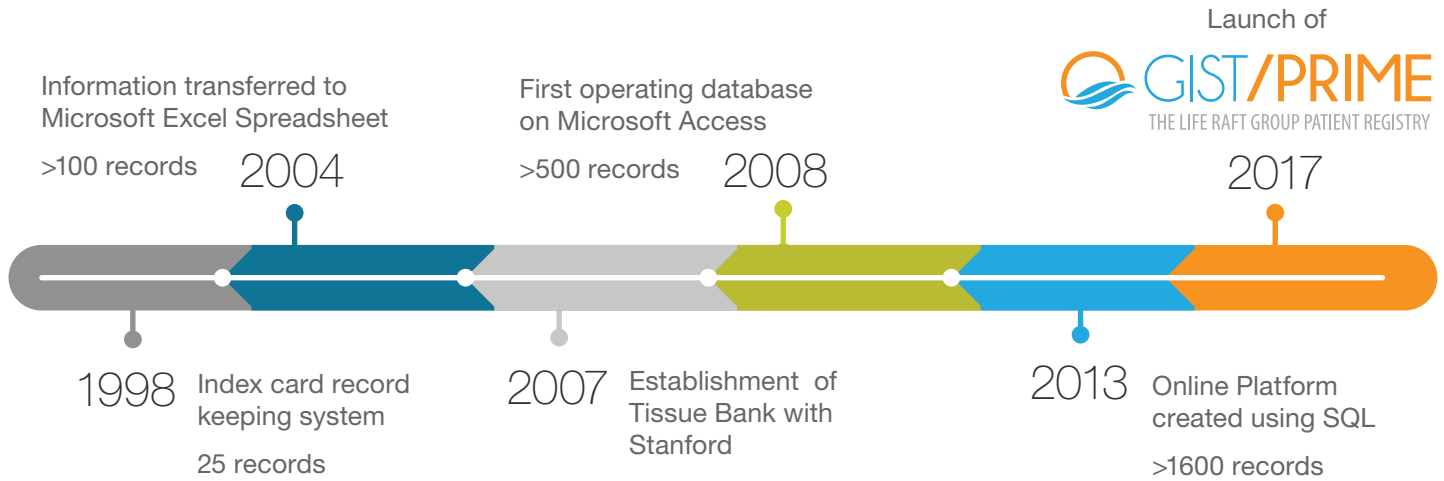
The LRG continued to attend and present at meetings such as the FDA/World Orphan Drug Congress, ASCO, CTTI and others. Norman Scherzer co-presented a poster with Dr. Jason Sicklick of Moores Cancer Center, University of Southern California, San Diego, at the Connective Tissue Oncology Society (CTOS) meeting in Hawaii. Data was drawn from the LRG Patient Registry on the response of SDH-deficient GIST to drugs.



Launching our GIST Clinical Trials database



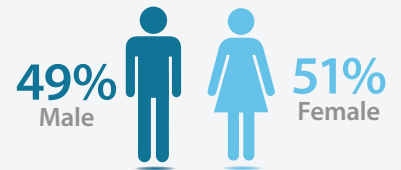
Initial Development of the Patient Registry



Patient Registry Members

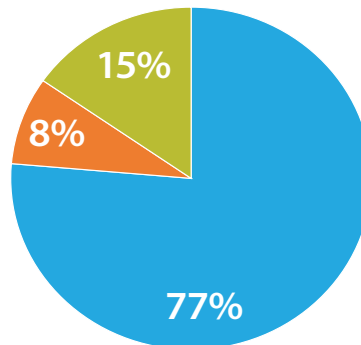
1807
Patient Registry Members

95
New Patient Registry Members



Mutation
44%

of Patient Registry members know their mutation and reported it

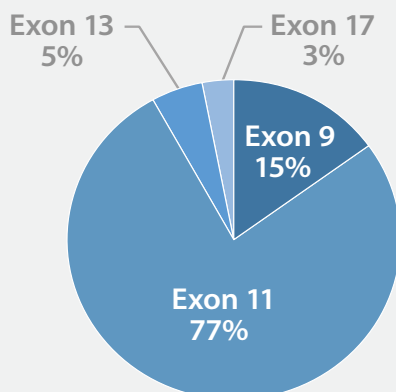


Mutation Breakdown

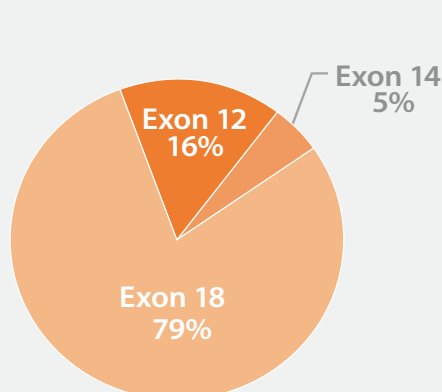
- KIT
- PDGFRA
- Other

Mutation Breakdown

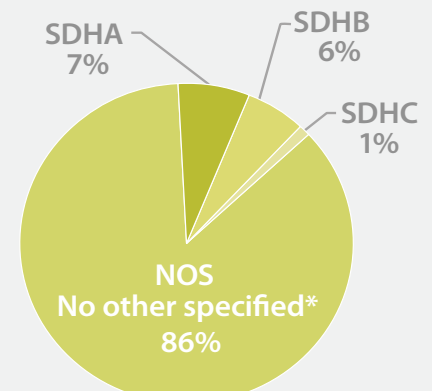
KIT



PDGFRA



OTHER

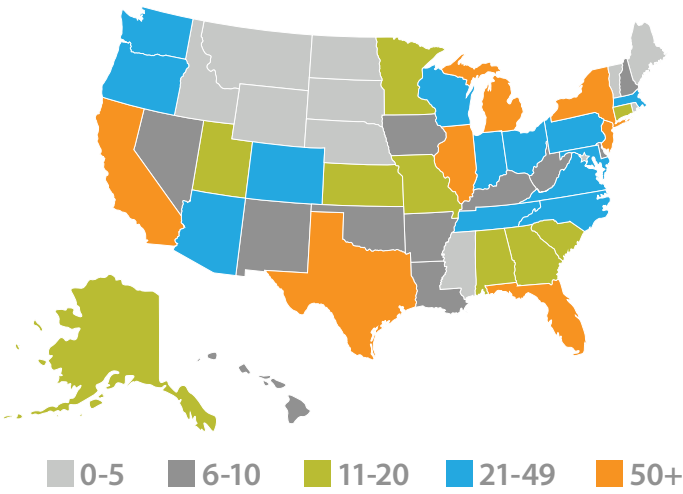


*NOS (No other specified); formerly Wildtype for KIT and PDGFRA



UNITED STATES • CANADA • CROATIA • ITALY
 AUSTRALIA • UNITED KINGDOM • MEXICO •
 COLOMBIA • ARGENTINA • URUGUAY • NET
 FINLAND • BOLIVIA • PERU • GERMANY • C
 BRAZIL • JAPAN • ITALY • VENEZUELA • BULG
 SLOVAKIA • SWITZERLAND • AUSTRIA • SLOV
 PHILIPPINES • SINGAPORE • SOUTH AFRICA
 DENMARK • GERMANY • STRA
 SWITZERLAND • CHINA • MOROCCO • JAP
 THAILAND • CHINA • CEDONIA • TUNISIA
 LITHUANIA • IRELAND • MALAYSIA • BELGIU
 SAMOA • KENYA • SWEDEN • RUSSIAN FEDER
 PAKISTAN • NORWAY • COSTA RICA • SAUDI
 SOUTH KOREA • BERMUDA • TURKEY • POL

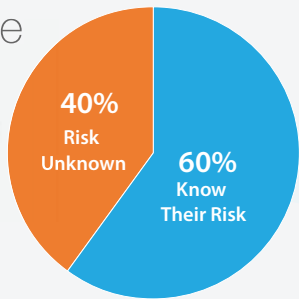
Patient Location



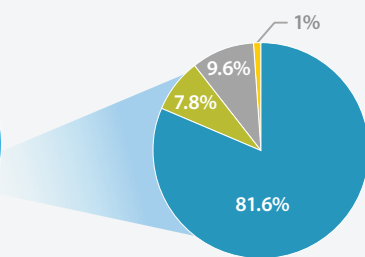
63 countries

Risk of Recurrence

72.7%
Single Tumor at
Diagnosis



27.3%
Mets at Diagnosis



High Risk (Blue), Low Risk (Grey), Intermediate Risk (Light Green), Very Low Risk (Yellow)

There are several different methods used to classify the risk of recurrence in GIST. The Patient Registry uses the Modified NIH Method, which looks at primary tumor size, mitotic count, and location.

Tissue Bank

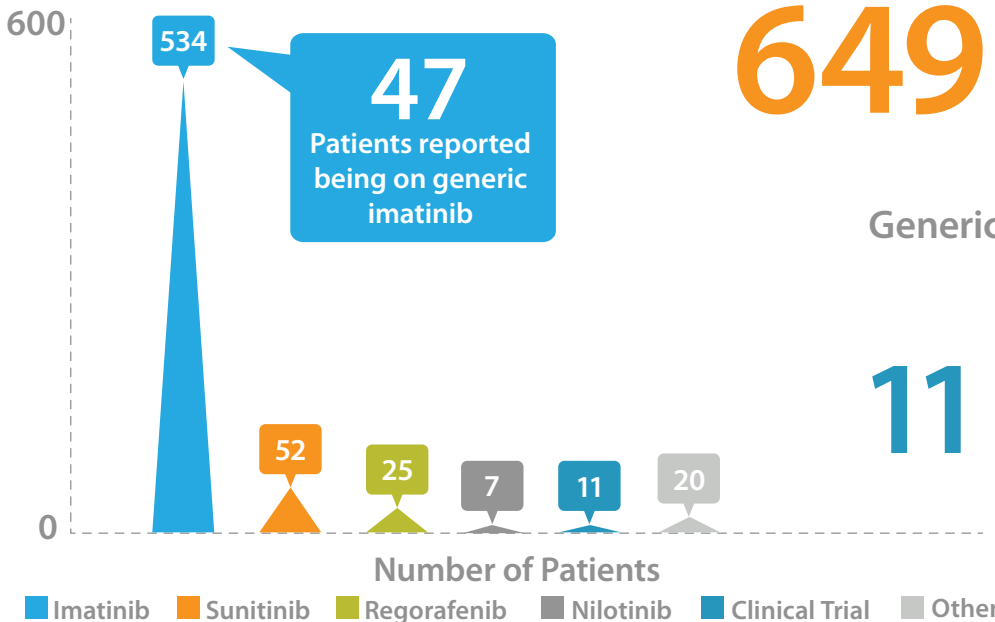
Total Members **414**

Total # of Tissues **778**

Top Three Primary Tumor Location

- 1 Stomach **712**
- 2 Small Intestine **592**
- 3 Rectum/Anus **48**

Medication



649 Number of living patients on medication:

Generics: **47**



11 patients entered a clinical trial this year



JERRY CUDZIL
PRESIDENT



GARY GLASSER
TREASURER



STAN BUNN



JIM HUGHES



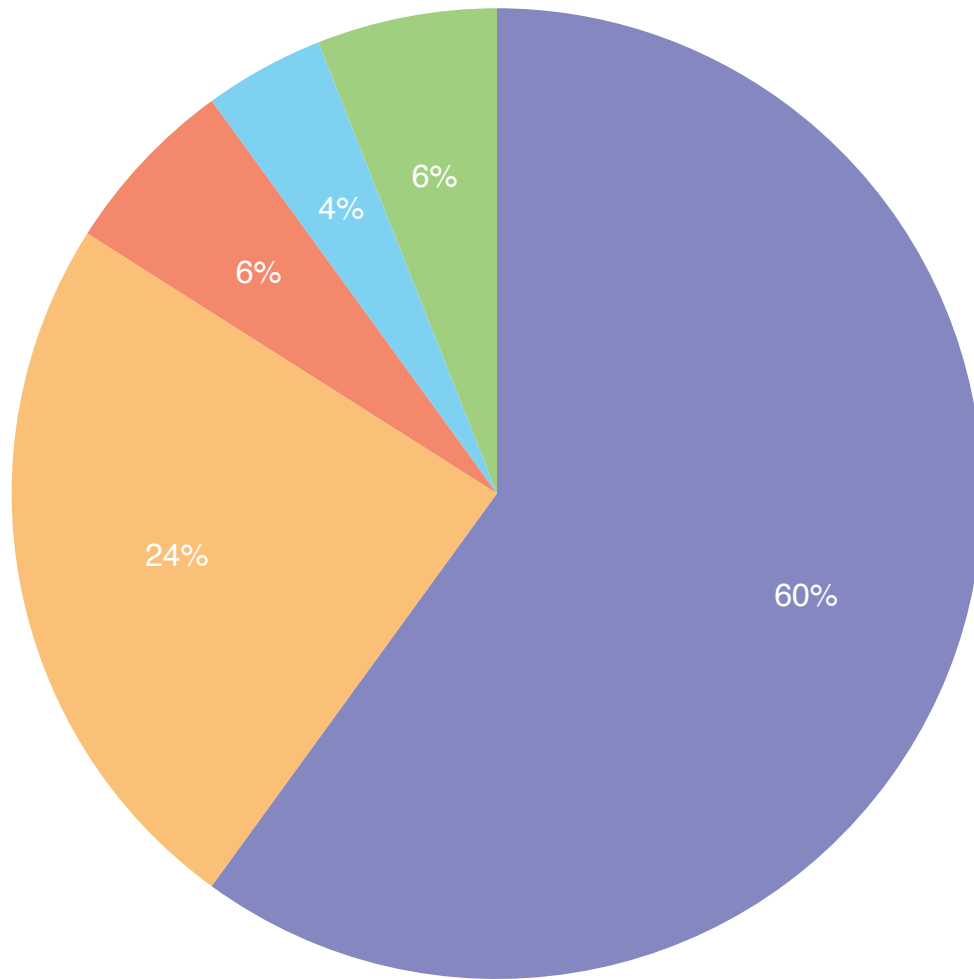
TEENA PETERSOHN



JOHN POSS



RODRIGO SALAS



- Research
- Information, Education, & Advocacy
- Patient Outreach & Support
- Management
- Fundraising

**\$100,000 and Above**

BST Consultants
Novartis Pharmaceuticals
Corp.
Pfizer Global Pharmaceuticals
Stanley Bunn
Carlos Baldor
Jerry Cudzil
Matt Knopman

\$20,000 - \$99,999

Blueprint Medicines Corp.
Bayer HealthCare LLC
Dublins Pub
John Kirk

\$10,000 - \$19,999

Bank of America
Morgan Stanley
Palermo-Ravich Family
Foundation
RBC Capital Markets, LLC
Gerald Knapp
Helen Frey
Jess Ravich
Jim Hughes
Louise Korder
Pat George

\$5,000 - \$9,999

Cantor Fitzgerald Relief Fund
Dan and Stacie Allen
Charitable Fund
Deciphera Pharmaceuticals
Goldman Sachs & Co. LLC
ICE
Investors Savings Bank
Charitable Foundation
JP Morgan Chase
The Knopman Family
Charitable Fund
Tradeweb Markets LLC
Kim & Ron Agypt
Tessie and Arthur Epstein

\$1,000 - \$4,999

Brightfunds.org
CBRE
Arrow Steel, Inc
Deutsche Bank AG
Guggenheim Securities, LLC
Jefferies LLC
Microsoft Matching Gifts
Program
Natixis North America LLC
Nomura
Price Philanthropies
SFC Security & Intelligence
LLC
UBS AG Bank
Women of the Greens
David Alexander
Jodi Alexander

\$1,000 - \$4,999 cont'd

Amy Anderson
Graig Badger
Richard Becker
Iris Berke
Georgia Bihl
Christian Bruner
Colleen Carney
Lyon Carter
Benji Cheung
Colman Currie
John Dahne
Michael Disanto
Geoff DuWors
Alicia Eppler
Robert Franz
Brian Gelfand
Gary Glasser
Sandra Glassman
Paul Gordon
Gayle Gow
Matthew Haynes
Richard W. & Sandra L. Hutson
Richard Kinzig
Joy and Doug Knopp
Adam Levy
Claudia & John Link
Ellen MacDonald
Jason Mathai
James and Camille Mayotte
Michael Neumann
Teena Petersohn
David Pichler
Brian Rabin
Marietta Robinson
Raymond Ruhe
Robert Sholiton
Andrew Silverman
Charles Sorrentino
Anne Spar
Robert Spellman
Sheryl Spray
Kay Stolzer
Andrew Tarcia
George & Karen Teren
R. Nicholas Trane
Deborah Wahl
Richard Warren
Richard & Ellen Whiteman
Rosenthal
Dina Wiley
Duane Williams
Leon Wyant
John Young
Daniel Zanty

\$500 - \$999

Arbor Foundation, Inc.
Local Union 254 of UBC&JA
Medtronic Your Cause
Paul and Margaret Renoff
Family Foundation
Plave Koch PLC

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Paul Andiorio
Olof Asbrink
Ryan Atkinson
Christian Bannerman
Yale Baron
Zachariah Barratt
Arie Bedell
Brian Behrens
Richard Bellofatto
Sergey Bessmertnyy
Ettore Bianchi
William Billingsley
Ann Bisno
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Aileen Bruner
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Ray Costa
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Michael Distenfield
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