FRIENDS of CANCER RESEARCH

PARTNERSHIPS SCIENCE SOLUTIONS

2016 ANNUAL REPORT

15 16 17

FRIENDS OF CANCER RESEARCH DRIVES

COLLABORATION AMONG PARTNERS FROM EVERY HEALTHCARE SECTOR TO POWER ADVANCES IN SCIENCE, POLICY, AND REGULATION THAT SPEED

LIFE-SAVING TREATMENTS TO PATIENTS.

2015
2016
2017

“I am absolutely alive today because of breakthrough therapies. Absolutely.”

STEPHANIE HANEY
Stage IV Lung Cancer Patient

2016 Friends of Cancer Research Annual Meeting Brings Together Global Leaders, Examines Potential Upcoming Regulatory Issues

On November 16, 2016, Friends of Cancer Research hosted this year's Annual Meeting, addressing critical issues in the development of new oncology drugs. The event kicked off with a keynote conversation between Doug Lowy, Acting Director of the U.S. National Cancer Institute, and Robert Califf, Commissioner of the U.S. Food and Drug Administration (FDA), and was moderated by Ellen Sigal, *Friends* Chairperson & Founder. The opening keynote conversation discussed the importance of funding and the need for improved clinical trials in great detail, with both Califf and Lowy agreeing that establishing effective data sharing practices requires further collaboration. Two panels comprised of experts from federal health and regulatory agencies, academic research, the private sector, and patient advocates followed the morning keynote. The first panel discussed how eligibility criteria, which are necessary to define the patient population under study, can be appropriately expanded in clinical trials. Building on the topic, panel two focused on collecting real-world evidence in the post-market setting and using it to support regulatory decisions. This year's lunch conversation took place between Richard Pazdur, FDA; Francesco Pignatti, European Medicines Agency; and Prudence Scott, Medex Consulting in Australia, who discussed issues in global harmonization for drug development. The meeting's third and final panel discussed methods to evaluate unexpectedly large improvements in overall survival in early phase randomized studies for regulatory decisions.

Friends and Alexandria Explore Utility of Real World Evidence for Breakthrough Therapies

This year marked the 5th Annual Alexandria Summit and Friends of Cancer Research Blueprint Forum. This year's topic focused on the potential utility of Real World Evidence (RWE) to support clinical trials. RWE is a collection of data that captures patients' clinical experiences and may serve as a source of information to support regulatory decision-making. Three panels explored RWE in great detail with an opening keynote by Robert Califf, Commissioner of the FDA. A multi-stakeholder working group developed a whitepaper that was distributed to conference attendees in advance of the event. The paper helps identify a path to using RWE by addressing three areas: identifying relevant disease and drug candidates in oncology as potential use cases; developing regulatory strategies for optimal use of RWE in oncology; and outlining potential opportunities to pilot studies in oncology to be used for clinical evidence generation and support regulatory decisions.

Unleashing Immunotherapy and Optimizing Its Potential

Friends and the Parker Institute for Cancer Immunotherapy convened a roundtable discussion on October 5, 2016, to discuss strategies to optimize the use of immunotherapy in cancer. Leading experts in the field shared their perspective on the state of the science and strategies to address current research questions and evidence gaps. The roundtable was divided into three sessions: standardizing toxicity management and communication; developing a non-comparative collaborative trial to test multiple PD-1 inhibitors using a common control; and exploring combinatorial approaches. After the meeting, a group was established to further discuss and develop a streamlined toxicity management guide for PD-(L)1 agents.

Friends and Duke Look to the Future of the U.S. Biosimilar Market

On October 18, 2016, Friends of Cancer Research and the Duke-Margolis Center for Health Policy held a forum on biosimilars in the U.S. market. The half-day forum brought together clinicians, originator and biosimilar drug sponsors, advocates, regulators, and payers to tackle remaining uncertainty surrounding the future of the U.S. biosimilars market. The forum was divided into two sessions with the first focusing on the current landscape of biosimilars development, regulatory review, and stakeholder education. The second addressed challenges in clinical decision-making, coverage and reimbursement, and postmarket evidence development. Ahead of the meeting, multi-stakeholder working groups developed two whitepapers providing a landscape analysis of biosimilar development, education, and utilization in the U.S.

Friends Celebrates 20 Innovative Years Advancing Treatments for Patients

On September 21, 2016, Friends of Cancer Research celebrated its 20th anniversary with a dinner held at the United States Institute of Peace in Washington, D.C. Leaders from the government, advocacy, industry, and science sectors attended the celebration to mark two decades of partnership and innovation on behalf of patients. The event honored Janet Woodcock, FDA; Eric Lander, The Broad Institute; and Sean Parker, The Parker Institute for Cancer Immunotherapy. In addition to the awards program with remarks from each honoree, *Friends* showcased four videos on each honoree and the organization during the 20th anniversary celebration, and Francis Collins, NIH, surprised the crowd with an original song dedicated to *Friends* for 20 years of hard work.

Sustained Leadership for Patient-Centered Innovation

As Friends of Cancer Research celebrated 20 years of dedicated work for patients and looked ahead to the future, the organization took steps to evolve and grow to continue to be a leading voice in cancer research and innovation. The organizational changes included Jeff Allen being named *Friends*' first President & CEO, and Ryan Hohman was named the first Vice President. Allen, who joined the organization in 2006, previously served as Executive Director, is a molecular biologist, and thought leader on many issues related to the FDA, regulatory strategy, and healthcare policy. Hohman, who joined the organization in 2008, served previously as Managing Director, is an attorney, and leading public affairs advocate with a diverse career in health policy and strategy.

Charting the Future of Breakthrough Therapies

The FDA began granting the breakthrough therapy designation in 2012 to medical products for serious and life-threatening diseases that showed promising early clinical evidence of improvement over available therapy. Since that time, FDA has granted more than 150 designations and approved more than 50 products under the program. *Friends* hosted a briefing on April 12, 2016, exploring multiple stakeholders' experiences with the breakthrough therapy designation, as well as preliminary evidence of the designation's impact on oncology development programs. The event coincided with a paper *Friends* published in the March issue of *Nature Reviews Drug Discovery* titled "Regulatory Watch: Impact of breakthrough therapy designation on cancer drug development."

Examining the Current Landscape in Genomic Testing and Its Future in Precision Medicine & Patient Care

On October 13, 2016, Friends of Cancer Research and the Deerfield Institute convened a briefing on Capitol Hill to discuss current and future regulatory issues related to genomic testing. Representatives from the multiple industries involved in genomic testing served on the panel, as well as individuals from patient advocacy and the FDA. The panel, comprised of Jonathan Leff, Deerfield Management; Andrea Ferris, LUNGevity; Jeff Allen, Friends of Cancer Research; John Iafrate, Massachusetts General Hospital; Mike Pellini, Foundation Medicine; and Jeff Shuren, FDA, and was moderated by Kate Rawson Powell, Prevision Policy, discussed ways to facilitate the continued innovation in precision medicine while also maintaining high standards for the accuracy and reliability of genomic tests. The meeting was optimistic regarding what genomic tests can do for patients and for future research into the biological underpinnings of disease.

Testifying Before the Senate HELP Committee on "Laboratory Testing in the Era of Precision Medicine"

On September 20, 2016, the Senate Health, Education, Labor and Pensions Committee convened a hearing to discuss laboratory testing in the era of precision medicine. The hearing took place amid an ongoing debate over the proper regulatory framework for laboratory-developed tests (LDTs). The regulation of LDTs was the topic of a draft FDA guidance document proposing that FDA enhance its oversight of these tests to promote greater accuracy in precision medicine. Four witnesses were invited to participate in the hearing, including *Friends*' President & CEO, Jeff Allen, who argued that the current divided system of oversight puts patients at risk and does not guarantee that tests measuring the same markers produce equivalent results.

The 2016 Presidential Election and Its Potential Impact on Biopharma

On November 4, 2016, Friends of Cancer Research and Prevision Policy convened the second annual BioPharma Congress. The event focused on the impact of the presidential election on biopharma in 2017 and featured speakers such as Robert Califf, FDA, and Andy Slavitt, Centers for Medicare and Medicaid Services. The event brought together regulators, industry, patient advocacy groups, and congressional staff, as well as payment and reimbursement organizations.

This year's BioPharma Congress was split into eight sessions with *Friends*' President & CEO, Jeff Allen, participating in a session that discussed PDUFA VI, new drug approvals, and what's next for breakthrough therapies. *Friends* Chairperson & Founder, Ellen Sigal, participated in a conversation with Richard Pazdur, FDA, and Greg Simon, Office of the Vice President on the Cancer Moonshot. Other sessions included: Medicare Part B and coverage issues for injectable drugs, innovation and the need for big ideas in the biopharma space, election impact on health policy, and a discussion with FDA center directors. Throughout the day issues regarding drug pricing, the Cancer Moonshot, the 21st Century Cures Act, and the future of the FDA were discussed.

Ellen Sigal Appointed to Distinguished Blue Ribbon Panel

On April 4, 2016, Friends of Cancer Research Chairperson & Founder, Ellen Sigal, was appointed to the National Cancer Institute's (NCI) Blue Ribbon Panel. The panel advised the National Cancer Advisory Board (NCAB) on the Vice President's National Cancer Moonshot Initiative. The Blue Ribbon Panel was comprised of scientific experts to advise the NCAB on different scientific opportunities to accelerate progress against cancer. In addition, it aimed to evaluate potential new investments in cancer research in order to energize national efforts to combat cancer.

Cancer Moonshot Blue Ribbon Panel Releases Report, Critical Recommendations to Advance Cancer Research

On September 7, 2016, the Cancer Moonshot Blue Ribbon Panel, comprised of 28 world-class leaders from science, research, and patient advocacy presented their recommendations for accelerating cancer research to the President's National Cancer Advisory Board. The Blue Ribbon Panel authored a report at the request of the Vice President, which outlined areas of urgent action to accelerate the field of cancer research.

The Blue Ribbon Panel recommendations were accepted by the NCI, and on October 17, 2016, Vice President Biden hosted an event at the White House on the Blue Ribbon Panel Report. *Friends* Chairperson & Founder, Ellen Sigal, delivered the opening remarks.

Revolutionary 21st Century Cures Act Signed Into Law

Friends of Cancer Research played a key role in the crafting and passage of the *21st Century Cures Act*. The Act passed the House and Senate with historic bipartisan majorities (392-26 and 94-5 respectively), shortly before President Obama signed it into law on December 13, 2016. This was the result of months of deliberation, compromise, and a productive national debate about the priorities and opportunities to enhance biomedical research and development of safe and effective new products. The *21st Century Cures Act* accomplishes this through key provisions including investment in critical science & research, building a framework to better involve and incorporate patients in the drug development process, and streamlining clinical trials, improving efficiency, and laying the groundwork for the future that will positively impact patients.

Senate Briefing: Proposing A Disease-Oriented Approach to Better Streamline FDA

On February 24, 2016, *Friends* hosted a Senate briefing discussing the proposal for an FDA Oncology Center of Excellence and why it is necessary for FDA to implement. The existing regulatory framework had been defined by a "divide and conquer" approach to oversight; separate centers within FDA regulate three major categories of medical products: drugs, devices, and biologics.

In order to take advantage of today's advancements in science, drug development, and patient treatment, the FDA's structure needs reorganization to focus its resources and ensure the best outcomes for patients. *Friends* proposed enhancing coordination at the FDA based on specific diseases to reflect 21st century science and modern medical care.

Streamlining FDA Review Through the Oncology Center of Excellence

After spending several months analyzing the current regulatory environment and examining opportunities for potential improvement in the review process of new cancer treatments, *Friends* proposed to establish Centers of Excellence at FDA. As a first step, Friends of Cancer Research suggested a pilot in oncology, which could be followed by Centers of Excellence in other major disease settings. On June 29, 2016, the Vice President announced that an FDA Oncology Center of Excellence (OCE) will be created as part of the Cancer Moonshot. The same day, Robert Califf, FDA, issued an announcement naming Richard Pazdur, FDA, the acting director of the OCE. The OCE will be further realized through the implementation of the *21st Century Cures Act*.

Note from the President & CEO

On behalf of Friends of Cancer Research, I would like to thank all of our supporters and collaborators for your continued and steadfast commitment to our mission. We've made great strides in developing new models to enhance science and innovation.

As we embark on the upcoming year, we look forward to continuing partnerships, built on cutting-edge science, to create solutions that will change the cancer landscape.

Your support over the last 20 years has been vital to our success and will be imperative in the future as we continue to advance innovation for patients.

With great appreciation,

Jeff Allen, PhD

PRESIDENT & CEO
FRIENDS OF CANCER RESEARCH

FRIENDS of CANCER RESEARCH

ProgressForPatients.org

ADVOCACY ALLIANCE & EDUCATION

New Program Designed By Patient Advocates, For Patient Advocates

BRINGING TOGETHER THOSE WHO SEEK SOLUTIONS TO ADVANCE SCIENCE THROUGH ENGAGING, EDUCATING, AND EMPOWERING PATIENTS.

Patient advocates devote time, energy, and resources to help patients suffering every day by advocating on their behalf. To make the greatest impact, advocates need the knowledge and understanding of the laws and regulations affecting the process of new treatments for patients, as well as the people and institutions that are involved in that process.

Friends has developed a training program for patients and patient advocates who wish to bring their insight to the regulatory process. This program consists of an online curriculum series, developing into additional in-person learning and networking opportunities. Through this training program, *Friends* provides advocates with the tools they need to effectively communicate with researchers, developers, regulators, and policy makers. This program will enable them to make the connections necessary to be fully active as an effective patient advocate, as well as engage with the FDA and drug companies.

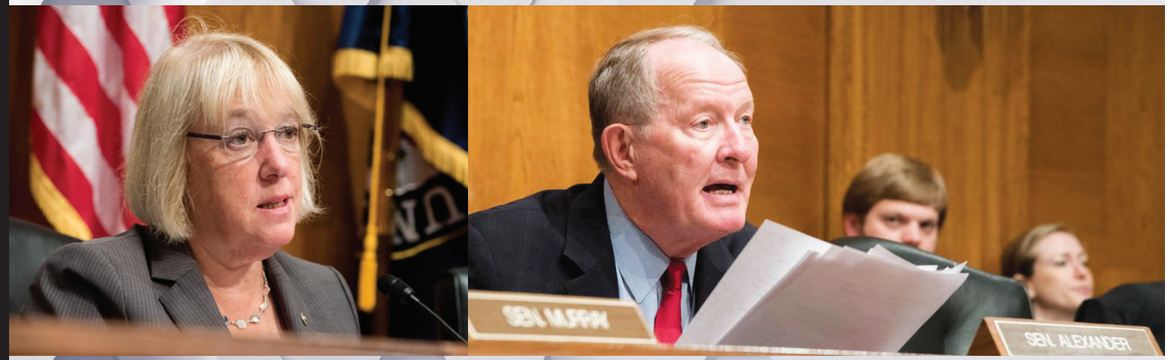
After completion, advocates will be a resource for organizations seeking to involve the patient perspective and input into various aspects of their operations. Our goal is that by completing this program, advocates will learn when and where to add their voice, and be better equipped to advocate for patients and the treatments they need.

VISIT PROGRESSFORPATIENTS.ORG AND FIND OUT MORE!

Engaging Innovation

Cultivating A New Online Community

Engaging Innovation is more than just the Friends of Cancer Research blog, it is an online community based on collaboration and a desire to streamline the process for getting new treatments to patients. With a refreshed look, the blog will continue to focus on leaders in science, policy, and advocacy while broadening its outlook with more frequent features and posts, spotlighting unique perspectives and relationships in cancer innovation and research. Our goal is to provide an engaging, forward-looking resource for those interested in regulatory science.



Friends Priorities for Reauthorizing the Prescription Drug User Fee Act

In 2017, the Prescription Drug User Fee Act (PDUFA) will undergo a reauthorization process through Congress. *Friends* achieved the passage of the Breakthrough Therapy Designation during PDUFA's reauthorization in 2012, and the initiatives highlighted below will all remain a part of our policy work as *Friends* looks toward this year's reauthorization process and helping patients.

STRENGTHEN precision medicine by creating a regulatory framework for diagnostic tests

TRANSFORM the way new treatments are evaluated through the development and use of patient experience data

BOOST FDA hiring and retention processes to attract and retain top talent

ENHANCE accelerated approval through the qualification of surrogate endpoints and other drug development tools

Thank You to Our 2016 Donors

INNOVATORS

Abbvie
Alexandria Real Estate Equities, Inc.
Amgen
Astellas US LLC
AstraZeneca
Battelle
Bayer
Bristol-Myers Squibb Company
Celgene
Eli Lilly & Company
Foundation Medicine
Genentech
Kenneth and Ginny Grunley
The Hands of Love Foundation
Janssen Pharmaceutical Companies of Johnson & Johnson
Marlene and Fred Malek
Joel Marcus
Merck & Co. Inc.
Novartis Pharmaceuticals Corporation
Pfizer Inc
PhRMA
Ellen and Gerry Sigal
Takeda Oncology

CHAMPIONS

The Anne and Ronald Abramson Family Foundation
Gary & Pennie Abramson
The Rona and Jeffrey Abramson Foundation
American Association for Cancer Research
American Cancer Society
American Society of Clinical Oncology
Amgen
Ambassador Yousef Al Otaiba
Boehringer Ingelheim Pharmaceuticals, Inc.
Gilead Sciences
The Kovler Foundation Judy and Peter Kovler
Jed Manocherian
Constance Milstein
Pharmacyclics Inc
Resnick Family Foundation
Sandoz
Sanofi

PARTNERS

ADP Foundation Inc.
AdvaMed
Association of American Cancer Institutes
Harold Beznos
Biotechnology Innovation Organization
Marcia and Frank Carlucci
Dr. and Mrs. James Cavanaugh
Dana-Farber Cancer Institute
Deerfield
Duke Cancer Institute
EMD Serono, Inc.
Epstein Family Foundation, Inc.
Daria Foster and Eric Wallach
Rob and Shari Friedman
Greenleaf Health, LLC
Sam and Peggy Grossman Family Foundation
Herbert Family Foundation
Incyte Corporation
Jonsson Comprehensive Cancer Center, UCLA
Kite Pharma
Susan G. Komen
Sherry Lansing
Annette M. and Theodore N. Lerner Family Foundation
The Meltzer Group
Miller & Long Company Inc.
David and Nicole Mitchell
Moffitt Cancer Center & Research Institute
David and Barbara Moser
National Patient Advocate Foundation
Otsuka America Pharmaceuticals
Suzy and Bob Pence
Peregrine Pharmaceuticals
The Abe and Kathryn Selsky Foundation
Larry and Klara Silverstein
Robert H. Smith Family Foundation
Stand Up to Cancer & The Entertainment Industry Foundation
Vertex Pharmaceuticals
George Washington Cancer Institute

ADVOCATES

AdvaMedDx
Miriam and Sandy Ain
Carolyn Aldige
Jeff Allen and Kevin Kenney
Richard and Donna Ben-Veniste
Dr. Philip and Faith Bobrow
Katherine and David Bradley
Rachel Brem
The Eli and Edythe Broad Foundation
CBS Corporation
William and Janet Cohen
Jackie Newmyer Deal
The Eacho Family Foundation
Edelman
Dan and Marina Ein
Joel and Barbara Finkelstein
Lucy Fisher and Doug Wick
Peter Grossman
Gerard and Pamela Heiber
Wilhelmina Holladay
Alphonso and Marcia Jackson
Michael and Elaine Jaffe
University of Kansas Cancer Center
Annette Kirshner
Mark Krueger & Associates
Edward Lenkin and Roselin Atzwanger
The Leukemia and Lymphoma Society
Fred and Britlan Malek
University of Maryland–Greenebaum Cancer Center
Joel and Debbie Meisel
Alan and Amy Meltzer
Douglas and Ellen Moeller
Shelley Mulitz
National Pharmaceutical Council
Arthur Newmyer
A.G. Newmyer III
Michelle and Chris Olson
Catherine Oppenheimer
William and Renay Regardie
Mace and Joyce Rothenberg
Eric and Kimberly Rubin
Matthew and Lynne Shank
Maggie Shannon
Mr. and Mrs. Albert H. Small
Jan Smith
Catherine Stevens
Leslie and Michael Tive
Annie Totah
Andy and Madelyn Von Eschenbach

PATRONS

John Adelman
AmazonSmile Foundation
Margaret Anderson
Seki Atsushi
David Beier
Catherine J. Blair
Steven Bloom
Susan and Dixon Butler

Thank You to Our 2016 Donors (con't)

Heather Chaney
Susan Hill Clark
Benjamin Cole
The Community Foundation for Northern Virginia
Christine Daley
Anita Dayton
Chip Diggins
Michael Faletto
Stuart Geller
Joseph and Alma Gildenhorn
Leslie Gitlin
GlaxoSmithKline
Janet Goss
David Grossman and Suzanne LaFleur
Roy Herbst
Ryan Hohman and Bradford Rathgeber
Robert Jesse
JustGive
Dianne Kay
Joanne Kemp
Marguerite Kenner
Sheila Kennett
Debbi Kirshner
Susie Kirshner and Liora Moriel
Paul Lewis
Olga Marchenko
Roger Meyer
Ted and Jean Miksinski
Tom and Bonnie Miller
Marie Mitoff
Amy Muhlberg
Melanie and Larry Nussdorf
David Ochsman
Linda Ostermann
Vassiliki Papadimitrakopoulou
Joe Patterson
Personalized Medicine Coalition
Geraldine Fogel Pilzer
Terri Sanker
Michael Shea
Lisa and Ricky Shofnos
Elaine Simpson
Mark Stewart
Berrett Stradford
Courtney Straus
Craig B. Thompson
Francine & Stephen Trachtenberg
Lita and Ed Treacy
Alexi Turbow
Stacy and Alberto Valner
Geoff Wahl
Ann and Daniel Von Hoff
Jeannette Jackson Walker
Robert Watson
Hal and Marilyn Weiner
Sharon Weiss
Courtney Wise
Myles Wittenstein Charitable Foundation Inc.
Daniel Wright Junior High School
Robert Young

IN-KIND

Alston + Bird
GCS, Inc.
SIGAL Construction

Friends of Cancer Research Board of Directors

EXECUTIVE COMMITTEE

Ellen V. Sigal, PhD
Chairperson & Founder, Friends of Cancer Research

Marlene Malek, RN
Vice Chair, Friends of Cancer Research

Jeff Allen, PhD
President and CEO, Friends of Cancer Research

Barbara Duffy Stewart, MPH
Secretary, Executive Director, Association of American Cancer Institutes

Sudip S. Parikh, PhD
Treasurer, Senior Vice President and Managing Director, Americas, Drug Information Association

Jonathan Leff
Partner, Deerfield Management

Margaret Foti, PhD, MD (h.c.)
Chief Executive Officer, American Association for Cancer Research

David Mitchell
President and Founder, Patients for Affordable Drugs

Joel S. Marcus
Chairman, Chief Executive, and Founder, Alexandria Real Estate Equities, Inc. Alexandria Venture Investments

Richard Schilsky, MD
Chief Medical Officer, American Society of Clinical Oncology

BOARD OF DIRECTORS

Pennie Abramson, PhD (h.c.)
President's Circle, The Weizmann Institute of Science

Carolyn "Bo" Aldige
President & Founder, Prevent Cancer Foundation

Anna Barker, PhD
Professor & Director, Transformative Healthcare Networks, Arizona State University

Sherry Lansing
Chief Executive Officer, The Sherry Lansing Foundation

Christopher W. Hansen
President, American Cancer Society Cancer Action Network

Mary Woolley
President, Research!America

Robert C. Young, MD
President, RCYMedicine

Special Thanks

Thank you to all of the *Friends* staff and collaborators who contribute to our work.

Jeff Allen, PhD
President and CEO

Ryan Hohman, JD
Vice President, Public Affairs

Heather Chaney
Managing Director, Program Administration

Berrett Stradford
Director, External Affairs

Mark Stewart, PhD
Science Policy Analyst

Michael Shea
Policy Research Associate

Joe Patterson
Public Affairs Associate

Alexi Turbow
Public Affairs Associate

Courtney Wise
Executive Assistant

Linda Ostermann
Science Policy Research Assistant