



FRIENDS OF CANCER RESEARCH  
2013

ANNUAL REPORT



ACCELERATING SCIENCE AND TECHNOLOGY

# 2013 Highlights

## **FEBRUARY 6, 2013**

Friends of Cancer Research Capitol Hill Briefing:  
The Blueprint of Medical Research: How New  
Medicines Get from the Lab to the Patient

## **MARCH 12, 2013**

Workshop: Design of a Lung Cancer Master  
Protocol

## **MAY 16, 2013**

Friends co-hosts annual Congressional Advocacy  
Day on Capitol Hill

## **JULY 24, 2013**

Friends of Cancer Research Congressional  
Briefing: Answering a Compelling Need:  
Expediting Life-Saving Treatments to Patients

## **SEPTEMBER 6, 2013**

Forum: A Blueprint for Future Drug/Diagnostic  
Co-Development: Breakthrough Therapies

## **SEPTEMBER 23, 2013**

Friends of Cancer Research Leadership Awards  
Reception

## **NOVEMBER 7, 2013**

Friends of Cancer Research and the Engelberg  
Center for Healthcare Reform at Brookings  
Conference on Clinical Cancer Research  
➤ The Lung Cancer Master Protocol is officially  
announced



Because patients deserve better options

Because a father, a sister, a child or friend  
deserve the best treatment possible

Because cancer won't wait

**Friends of Cancer Research** is our country's leading voice in advocating for policies and solutions that will get treatments to patients in the safest and quickest way possible.

Friends of Cancer Research (*Friends*) develops groundbreaking partnerships and creates a more open dialogue among both public and private sectors and tears down the barriers that stand in the way of conquering cancer. By collaborating with premier academic research centers, professional societies and other advocacy organizations, Friends is able to accelerate innovation.

We work closely with government agencies (FDA, NCI, NIH, HHS) and Congressional leadership to create educational, policy, and scientific approaches to improve health outcomes and cancer care. As a respected independent think tank and advocacy organization, Friends is able to cut through bureaucratic red tape, put aside partisan politics and engage all stakeholders, producing real results.

- We have made great strides in the fight against cancer but challenges still exist
- 79 percent of cancer research grants go unfunded each year
- It still takes more than 12 years for newly discovered treatments to get from the research bench to the patient's bedside

Imagine what discoveries could be made and the lives that could be saved if more grants were funded, if the barriers between discovering new treatments and getting them to a patient's bedside were overcome.

***We are working every day to make new treatments a reality for patients everywhere.***

To learn more please visit: [www.focr.org](http://www.focr.org) or 202.944.6700

# Friends of Cancer Research Spearheads a Groundbreaking Clinical Trial Model

Taking a new drug from the initial discovery stage through clinical testing and regulatory review is complicated, expensive, and often inefficient. Trials are difficult to initiate, subject to lengthy regulatory review, infrastructure-intensive, and reliant on the enrollment of volunteers. These challenges are compounded by the fact that trials for new drugs are almost always conducted separately, even when multiple drugs are being developed to treat the same condition.

In 2013, Friends brought together a multi-stakeholder group of academics, government officials, and members of industry to develop a new and more efficient clinical trial protocol that will begin guiding a multi-drug lung cancer trial in Spring 2014.

## About Lung-MAP

**A groundbreaking clinical trial model that uses a multi-drug, targeted screening approach to match patients with promising new treatments based on their unique tumor profiles.**

- **Lung-MAP** is an unprecedented public-private collaboration among the National Cancer Institute, SWOG Cancer Research, Friends of Cancer Research, the Foundation for the National Institutes of Health, five pharmaceutical companies (Amgen, Genentech, Pfizer, AstraZeneca, and AstraZeneca's global biologics R&D arm, MedImmune), Foundation Medicine and several lung cancer advocacy organizations.
- **Lung-MAP** is a multi-drug, multi-arm, biomarker-driven squamous cell lung cancer clinical trial that uses cutting-edge genomic profiling to match patients to investigational treatments that may target the genomic alterations, or mutations, found to be driving the growth of their cancer.

- Instead of having to undergo multiple diagnostic tests to determine eligibility for many different studies, enrollees are tested just once according to a "master protocol" and assigned to one of five different trial arms, each testing a different drug from a different developer.
- That means shared information and infrastructure, better access for patients to promising drugs, better access for researchers to relevant enrollees based on their genomic profiles, and less time and money needed before new drugs can be tested.

## HOW IT WORKS:

- Patients are screened using a comprehensive genomic profiling platform that looks at over 200 cancer-related genes for genomic alterations.
- Based on the results of this screening, patients are assigned to whichever one of up to five sub-studies testing different investigational regimens best suits their genomic profile.
- This innovative approach improves a patient's likelihood of receiving a drug that will work for them while allowing for new therapies in development to be added as the trial progresses.

## OVERCOMING CURRENT HURDLES IN CLINICAL TRIALS:

- While the rise of precision medicine has improved many aspects of patient care, it has also exacerbated the challenges of running a clinical trial. Smaller, targeted patient populations have made it more difficult to recruit eligible patients. And reliance on multiple, single-gene diagnostic tests can increase infrastructure costs, complexity, and patient burden.

## Lung-MAP Timeline

### November 2012

A working group at the **2012 Conference on Clinical Cancer Research**, hosted by Friends of Cancer Research and the Engelberg Center for Health Care Reform at the Brookings Institution, proposed an alternative clinical trial design: a multi-arm, multi-marker/drug trial in a specific disease setting.

### March 2013

Friends hosted a working session on the **Design of a Lung Cancer Master Protocol** to begin to develop a clinical protocol for the trial. Leaders from federal health and regulatory agencies, academic research centers, patient advocacy organizations and the private sector came together to reach consensus on the design of a biomarker-driven, multi-drug, multi-arm phase two/three registration trial in lung cancer.

The trial would also serve as a model that can be used for other diseases. It represents a new opportunity for patients in a setting where few clinical trials may be available or accessible, especially for those patients with very rare mutations.

### November 2013

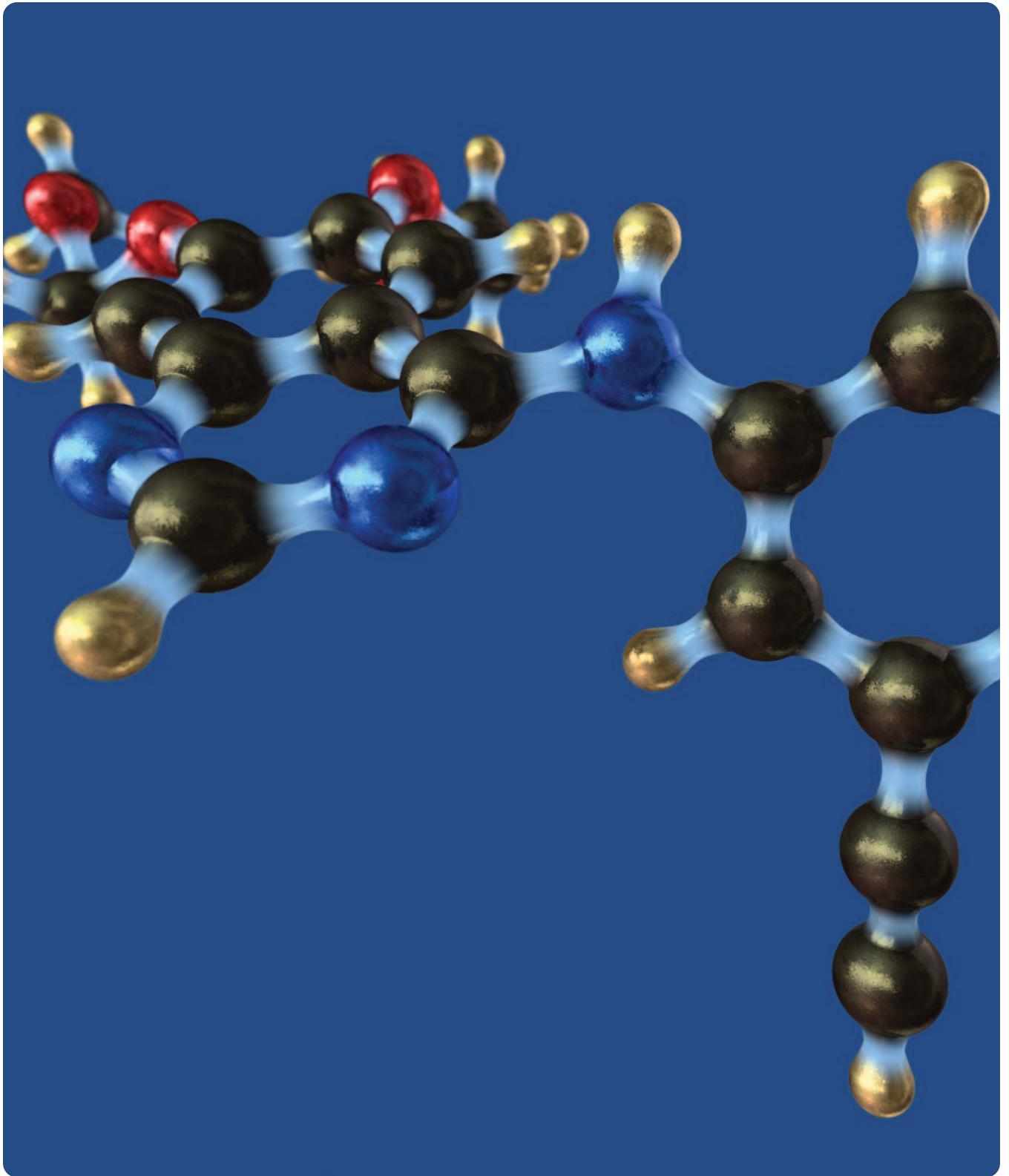
The final design of the Lung Cancer Master Protocol was announced to the public at the **6th Annual Friends/Brookings Conference on Clinical Cancer Research**, along with the first five participating drugs.

The resulting trial called Lung-MAP; a phase two/three trial which tests multiple new therapies simultaneously in sparse randomized substudies for patients with advanced stage refractory squamous cell NSCLC.

### Spring 2014

Trial enrollment is expected to begin in Spring 2014.

**This trial has the potential to change and accelerate the way new biomarker-defined therapies are tested and approved for lung cancer, and eventually for many other diseases.**



A model of a molecule of Erlotinib, also known as Tarceva. It is used in cancer treatment, especially for forms of lung cancer and pancreatic cancer.

MARCH 12, 2013

## WORKSHOP

# Design of a Lung Cancer Master Protocol

On March 12, 2013, Friends of Cancer Research hosted a workshop on the **Design of a Lung Cancer Master Protocol**. At this workshop, a multi-stakeholder group of experts came together to reach consensus on the design of a biomarker-driven, multi-drug, multi-arm Phase 2/3 registration trial in lung cancer. This trial, which was first proposed at the 2012 Conference on Clinical Cancer Research hosted by Friends of Cancer Research and the Engelberg Center for Health Care Reform at the Brookings Institution, has the potential to revolutionize and accelerate the way new biomarker-defined therapies are tested for lung cancer as well as other diseases. It also represents a new opportunity for patients in a setting where few clinical trials may be available or accessible, especially for those patients with very rare mutations.

Participants came to agreement on the overall trial design (including endpoints, patient population, biomarker screening, controls, and statistical analysis). This trial will utilize a broad, multi-marker screening platform. The ability to screen for a large number of alterations from the same sample is a key feature of this trial. This feature maximizes the information potential of patient tissue and maximizes the likelihood that any particular patient will be successfully matched to a biomarker-defined arm. This also reduces the burden on drug companies of having to find a diagnostic partner to continue the development of

a biomarker-defined therapy. Another key feature is that all of the NCI cooperative groups and their research institutions would be involved to enable rapid patient accrual. The master protocol could be amended for individual study arms depending on the features of a specific investigational agent at IRBs around the country rather than having new trials starting- this would greatly increase efficiency. The trial would be designed with the ultimate goal of FDA approval for those drugs that meet pre-specified efficacy criteria.

The workshop also led to consensus on the roles of different groups in the conduct of the trial. After approval by the NCI Thoracic Malignancy Steering Committee, the master IND would be held by the Foundation for the National Institutes of Health (FNIH), who would hold contracts with all of the entities including trial sites, labs, CROs, and companies. The trial would utilize a drug selection committee consisting of independent experts to determine what biomarkers and drugs would be studied in the trial. A Global Trial Oversight committee, consisting of members of FDA, NIH, FNIH, patient foundations and industry would also evaluate site management and data collection. The NCI SWOG cooperative group would be responsible for operational monitoring, quality control, tissue and data flow.

NOVEMBER 7, 2013

## Friends of Cancer Research and the Engelberg Center for Health Care Reform at Brookings Conference on Clinical Cancer Research

Each year, **Friends of Cancer Research** (*Friends*) and the **Engelberg Center for Health Care Reform at Brookings** convene the **Conference on Clinical Cancer Research** (CCCR). This annual conference is the culmination of a year spent in collaborative meetings and working groups, writing pre-conference reports and developing patient-driven solutions to the most pressing issues in cancer care. The conference brings together leaders from federal health and regulatory agencies, academic research centers, patient advocacy organizations, and the private sector to address the most critical, cutting-edge issues surrounding the development and regulation of cancer drugs and therapies.

On November 7, 2013, the sixth Friends-Brookings conference was held in Washington, DC, with generous support from the **American Society for Clinical Oncology** and **Susan G. Komen for the Cure**. This year's conference, attended by hundreds of leaders in their fields, featured three sessions that proposed innovative approaches to current challenges in drug development:

### **PANEL ONE: Facilitating Development of Immunotherapies**

### **PANEL TWO: Lung Cancer Master Protocol Activation Announcement**

### **PANEL THREE: Optimizing Dosing of Oncology Drugs**

In welcoming remarks, **Dr. Ellen Sigal, the chair and founder of Friends of Cancer Research**, detailed a wide-ranging list of cancer advances that evolved directly from research presented at the previous five CCCR gatherings—most notably, the "Breakthrough Therapies" designation which today enables the FDA to expedite development of drugs that show substantial promise in early clinical trials. The Breakthrough Therapies pathway originated from a

session at the 2011 meeting; bipartisan legislation was enacted barely nine months later, and has resulted in some extraordinary early successes, Sigal explained. "Each year, we work together towards getting results," she reiterated. At this year's conference, that commitment to results could be seen in the announcement of a new Lung Cancer Master Protocol—an exciting milestone which grew out of a white paper presented at last year's CCCR gathering. **Dr. Mark McClellan, senior fellow and director of the Health Care Innovation and Value Initiative at Brookings**, echoed Sigal's comments. "Through the past five conferences, this community has explored a host of critical topics and helped identify practical next steps to advance regulatory science, clinical research, and policy," he said. This year's gathering would again "encourage an emphasis on actionable and practical recommendations to improve current clinical cancer research and practices," McClellan promised.

**Dr. Margaret Hamburg, Commissioner of the U.S. Food and Drug Administration**, offered keynote remarks. "In a field that's too often limited by silos, the *Friends-Brookings* conference is one of the best venues for open, public collaboration on behalf of patients everywhere," she said, adding that the FDA relies extensively on the conference findings every year for invaluable "input, problem-solving and guidance." By convening international experts from so many sectors, she said, the CCCR ensures that "ideas that originate [here] don't end up lost in transcripts and white papers—they go on to profoundly impact the way we approach cancer treatment and care."

The commissioner commended two recent advances, in particular: The Breakthrough Therapies designation to expedite development and review of new, life-saving drugs, and the Lung Cancer Master Protocol. Less than two years after the idea was proposed at this conference,





Panel Discussion One at CCCR 2013: (L-R) Mary Redman, Jeff Abrams, Vince Miller, Ann Ashby, Vali Papadimitrakopoulou, David Gandara, Janet Woodcock, Roy Herbst, Jeff Allen

we have already seen 30 drugs designated as Breakthrough Therapies, she noted. And regarding the lung cancer protocol, she said, it had "blossomed from an innovative idea born here just last year into a tangible and truly revolutionary new structure designed to take advantage of the opportunities in personalized medicine."

The new protocol "vastly increases the chance that we will find more effective treatments, and does so in a creative and more cost-effective way," she said, adding that its promise extends far beyond lung cancer drugs to clinical research on a wide range of diseases and the search for cures.



Richard Pazdur discusses oncology dosing



Ellen Sigal gives welcoming remarks

**PANEL ONE:****Facilitating the Development of Immunotherapies: Intermediate Endpoints for Immune Checkpoint Modulators**

Immunotherapies hold the potential for long-term antitumor activity; however, the response to immunotherapies is often delayed. The pre-conference white paper that was put forth sought to identify new tools for measuring the activity of immunotherapies, and proposed several endpoints that could account for the potential of delayed treatment effect.

Moderated by **James Allison**, Immunology Chair, The University of Texas MD Anderson Cancer Center.

**Panelists:**

**Mark Gorman**, Survivor and Patient Advocate

**Ramy Ibrahim**, Senior Medical Director, Clinical Development, Oncology, MedImmune

**Axel Hoos**, Vice President of Oncology R&D, GlaxoSmithKline

**Tai-Tsang Chen**, Lead Statistician, Bristol-Meyers Squibb

**Steven Rosenberg**, Head of the Tumor Immunology Section, NCI

**Amy McKee**, Lead Medical Officer, Center for Drug Evaluation and Research, FDA

**Celia Witten**, Director, Office of Cellular, Tissue, and Gene Therapies, Center for Biologics Evaluation and Research, FDA

The first session explored the promise and potential for a certain class of drugs, called check point modulators, to regulate a patient's immune system to kill cancer cells. One notable challenge associated with this exciting field of oncology: A patient's response to some immunotherapies may take longer to be observed than response to traditional anti-cancer drugs. As a result, conventional measurements of 'success' may underestimate the efficacy of these drugs and may even be an inappropriate way to evaluate them. The presenters discussed the need for alternative methods to evaluate clinical cancer trials that account for the potential of a delayed treatment effect. A better understanding of appropriate endpoints could reduce the chance that trials of promising new drugs would be prematurely halted, and could thereby encourage the development of effective, life-saving treatments in cancer care.

Dr. James Allison discussed the "durable response" that various immunotherapies have been shown to deliver in

clinical research on patients with lung, brain, and prostate cancers, and noted that the approach has been shown to work in virtually all types of cancer. Dr. Tai-Tsang Chen further explored the rationale for agreeing on a new set of intermediate endpoints or tools to measure the activity of immune checkpoint modulators. He proposed an alternate approach to evaluating survival, dubbed the "milestone survival analysis," which measures the proportion of survivors at a pre-specified time, and detailed the pros and cons of this approach compared to those of the conventional approach of measuring survival. And reflecting on the discussion of alternative endpoints, Dr. Amy McKee reminded the group that "overall survival as an endpoint is still the gold standard" in any effort to measure the efficacy of a cancer therapy.

Emotional testimony from Mark Gorman, a long-term survivor of metastatic melanoma and an eloquent patient advocate, added a powerful first-hand perspective on immunotherapy. Reflecting on the deaths of one 'cancer buddy' after another over the last 15 years, he expressed relief that his current buddy is finally responding well to a new combination of immunotherapeutic treatments. Calling himself an 'outlier', he shared his hope that cases like his would become the rule instead of the exception in the successful treatment of cancer. "Durability is a good thing," he concluded.



Panel Two: (L-R) Tai-Tsang Chen, Axel Hoos, Ramy Ibrahim, Mark Gorman

**PANEL TWO:****Announcement of a Lung Cancer Master Protocol**

Moderated by **Jeff Allen**, Executive Director, Friends of Cancer Research

**Panelists:**

**Roy Herbst**, Chief of Medical Oncology, Yale Cancer Center

**Janet Woodcock**, Director, Center for Drug Evaluation and Research, FDA

**David Gandara**, Director, Thoracic Oncology Program, UC Davis

**Vali Papadimitrakopoulou**, Professor, Dept. of Thoracic/Head and Neck Medical Oncology, MD Anderson Cancer Center

**Ann Ashby**, Deputy Executive Director, Foundation for the National Institutes of Health

**Vince Miller**, Chief Medical Officer, Clinical Development, Foundation Medicine

**Jeff Abrams**, Chief, Clinical Investigations Branch, National Cancer Institute

**Mary Redman**, Statistician, Fred Hutchinson Cancer Center

This session centered on the official announcement of a Lung Cancer Master Protocol, a first-of-its-kind clinical trial design that has generated much excitement in the broader cancer research community. The new protocol is designed to address a fundamental challenge in cancer research and care: Developing a potential therapy from the initial discovery stage through clinical testing and regulatory review is a complicated, expensive and often inefficient process that can take up to 15 years.

The new master protocol aims to cut through the typical administrative and regulatory hurdles associated with large clinical trials by testing several drugs at once in hundreds of clinics across the United States. Rather than undergoing multiple time-consuming tests, patients can be screened for various biomarkers with a single test, and be "matched" to the treatment regimen and medicine most likely to help slow their cancer as soon as possible. Researchers will study patients with advanced-stage squamous cell lung cancer. When the study gets underway in the next few months, it will represent the first biomarker-defined, multi-arm, multi-drug trial designed to evaluate the effectiveness of five different therapies for the disease.

Dr. Roy Herbst detailed the unique public-private partnership behind the master protocol lung cancer trial. The collaboration brings together Friends of Cancer Research with the FDA, the National Cancer Institute, the Foundation for the National Institutes of Health (FNIH) and the Southwest Oncology Group (SWOG), as well as leaders in academia and in the pharmaceutical industry. The FNIH and SWOG will manage the trial, while Foundation Medicine, a cancer genetics research company, will provide the common platform used to test molecular alterations in each patient's lung tissue. Herbst also announced the five cancer therapies that will be included initially, as selected by an academic review panel:

MEDI4736, from MedImmune, an anti-PD-L1 monoclonal antibody

AZD4547, from AstraZeneca, a FGFR tyrosine kinase inhibitor

Rilotumumab, from Amgen, a hepatocyte growth factor receptor c-MET inhibitor

Not identified, from Genentech/Roche, a PI3 kinase inhibitor, and

Palbociclib, from Pfizer, a CDK4/6 kinase inhibitor

Dr. Vali Papadimitrakopoulou, the study chairman, described plans to screen up to 1,250 potentially eligible patients and enroll 500 to 1,000 of them in the first year. With guarded optimism, she told conference attendees she expected to launch the first phase of trials in spring 2014, and hoped to share preliminary results by the time the CCCR convenes again next November.

"We need to turn the clinical trial paradigm on its head," urged Dr. Janet Woodcock, noting that the lung cancer protocol has the potential to do just that. "This is an ethical imperative," she added, calling the new approach "the best hope for patients" whose survival depends on faster answers and research. And Dr. David Gandara echoed these thoughts when he described the potential of the Lung Cancer Master Protocol to revolutionize cancer trials in general. If we can screen 1,000 patients a year for specific, targeted biomarkers, "we will have changed the entire paradigm for drug development" and cancer care. "The world is watching this group," he added, noting that members have already received inquiries from researchers in Europe about launching master protocols based on the model.

### PANEL THREE: Optimizing Dosing of Oncology Drugs

The current approach to oncology drug development provides a suboptimal understanding of the relationship between dose and clinical outcome. The pre-conference white paper put forth sought to determine how dosing can be improved for cancer patients, and proposed a novel approach for using expanded access protocols to perform comparative dosing studies.

Moderated by **Richard Schilsky**, Chief Medical Officer, American Society of Clinical Oncology

#### Panelists:

**Atiqur Rahman**, Director, Division of Clinical Pharmacology V, CDER, FDA

**Daniel Auclair**, Research Director, Multiple Myeloma Research Foundation

**Lori Minasian**, Deputy Director, Division of Cancer Prevention, National Cancer Institute

**Oliver Rosen**, Vice President and Head of Global Medical Affairs, Millennium: The Takeda Oncology Company

**Richard Pazdur**, Director, Office of Hematology and Oncology Products, CDER, FDA

The final session of the conference considered important questions around the optimal dose of oncology drugs for patients with varying needs. Cancer treatment has changed over the years, evolving from the use of highly toxic chemotherapy focused largely on treatment at the "maximum tolerated dose" (MTD) to the use of targeted therapies that may achieve optimal efficacy at a dose lower than the maximum a patient can tolerate. Identifying the right balance—the dose at which efficacy is maximized but toxic side effects are minimized—is important to provide longer survival and the best quality of life for patients. Panel members proposed alternative approaches to determining dosing for oncology drugs that seek to optimize dose selection as well as enable a more complete understanding of the relationship between drug exposure and clinical outcomes. They also explored how this can be achieved without delaying the entry of promising new drugs to the market.

Dr. Richard Schilsky outlined the key factors that lead to variable drug responses among different cancer patients-



Margaret Hamburg delivers her keynote remarks



(L-R) Roy Herbst and Jeff Allen



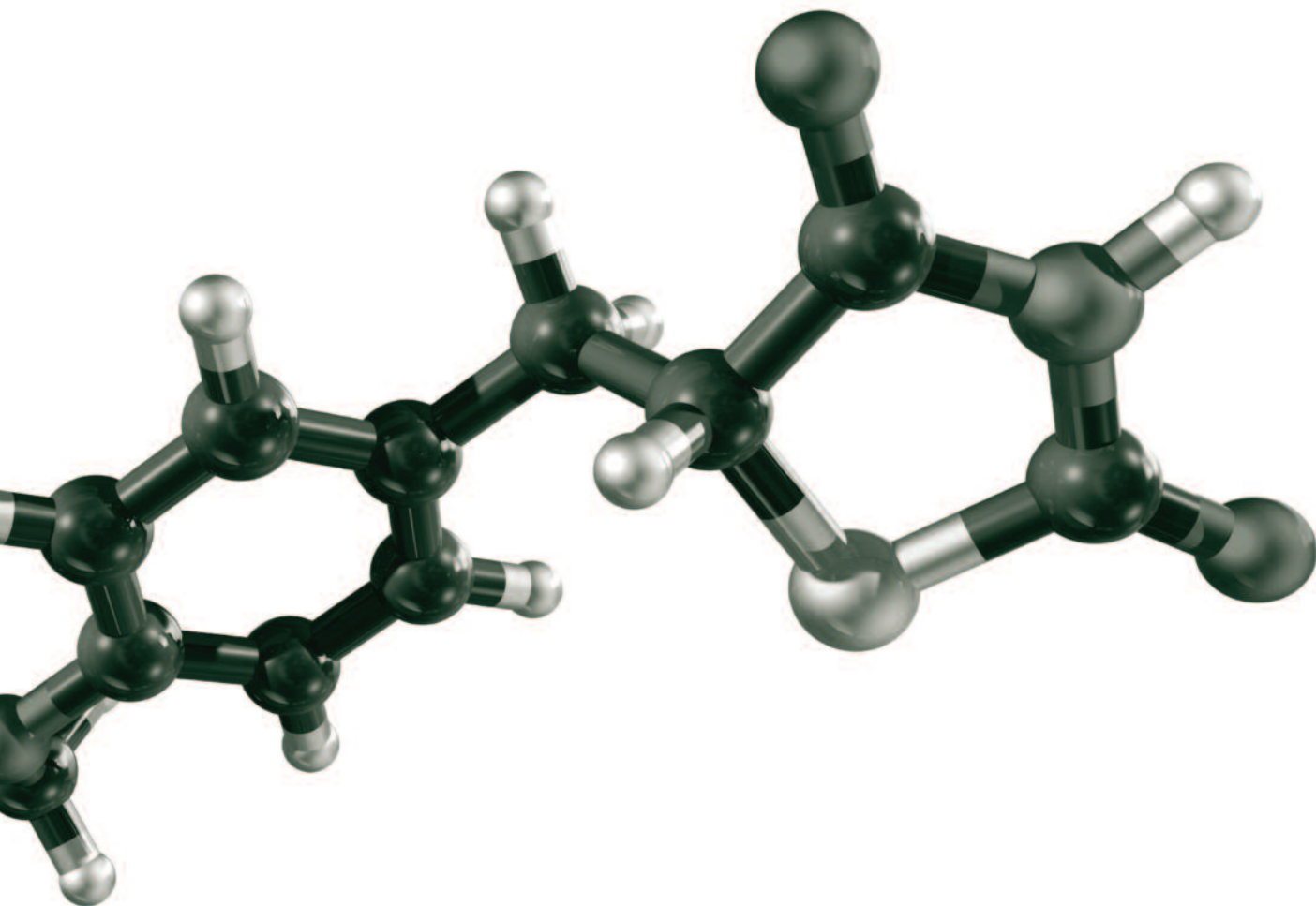
Panel Three: (L-R) Richard Pazdur, Oliver Rosen, Lori Minasian, Daniel Auclair, Antiquir Rahman, Richard Schilsky

which include different rates of metabolism, and a patient's age, weight, and diet. The result, he explained, is that "any dose we put in the marketplace will inevitably be too high for some patients and too low for others." Dr. Lori Minasian discussed the importance of patient-reported outcomes of adverse events during cancer treatment, and how they often differ considerably from reported side effects by clinicians. And Dr. Oliver Rosen led a discussion about the optimal timing for conducting dosing comparison research. The panel discussed optimal timing for such studies, concluding that while the most appropriate time is during phase two studies before initiation of registration trials, the time period after completion of registration trials but prior to marketing approval by the FDA presents an additional window of opportunity. The panel proposed that dosing studies could be performed through expanded access protocols, an approach that may facilitate patient access as well as improve understanding of the drug without delaying development.

Brookings' Mark McClellan closed the conference, commending the panels and attendees for "moving us closer to consensus on how to approach some of the most critical issues in clinical cancer research today. And turning that consensus into action—as exemplified by the Lung Cancer Master Protocol unveiled earlier in the day—is what it will take to make a real difference in the lives of people directly affected by cancer," he concluded.



Mark McClellan



## Breakthrough Therapies Advancing to Patients

**The past year was momentous for Friends and its many allies in the cancer community. Working with partners in advocacy, regulation, drug development, and Congress, Friends took Breakthrough Therapy from innovative concept, to legislative solution, to federal law in just 13 months.**

When new drugs for undertreated conditions show unprecedented effect in preclinical or early clinical testing, patients shouldn't have to wait. The Breakthrough Therapy Designation is a new FDA tool that ensures that patients have access to revolutionary drugs as quickly as possible, in clinical trials and beyond.

On November 10, 2011, a panel at the Friends-Brookings Conference on Clinical Cancer Research proposed strategies to expedite FDA approval of exceptional drugs without sacrificing safety and efficacy standards. On March 22, 2012, Friends held a Congressional briefing to encourage the creation of a "Breakthrough Therapy" designation.

In Spring 2012, Senators Michael Bennet (D-CO), Orrin Hatch (R-UT), and Richard Burr (R-NC) and Representatives Diana DeGette (D-CO) and Brian Bilbray (R-CA) introduced the bipartisan Advancing Breakthrough Therapies for Patients Act. On July 9, 2012, Breakthrough Therapy was passed into law as part of PDUFA V, The Food and Drug Administration Safety and Innovation Act (FDASIA). *Friends* released a white paper and held a panel discussion in November 2012 to encourage developers and regulators to use the new pathway and to inform FDA guidance for Breakthrough developers. In July 2013, Friends hosted "**Answering a Compelling Need: Expediting Life-Saving Treatments to Patients**", a Congressional briefing to discuss the pathway's use and future one year into its existence.

The Breakthrough Therapy Designation has already seen substantial use.

### Breakthrough by The Numbers

■ Applications for Breakthrough Designation:	<b>178</b>
■ Designations Granted by the FDA:	<b>44</b>
■ Designations for Cancer Drugs:	<b>14</b>
■ FDA Approvals of Breakthrough Drugs:	<b>6</b>
■ FDA Approvals of Breakthrough Cancer Drugs:	<b>4</b>

JULY 24, 2013

## Friends of Cancer Research Congressional Briefing – Answering a Compelling Need: Expediting Life-Saving Treatments to Patients

On July 24, 2013, Friends of Cancer Research (Friends) hosted a Congressional briefing titled "**Answering a Compelling Need: Expediting Life-Saving Treatments to Patients**" to discuss the Food and Drug Administration's (FDA's) Breakthrough Therapy designation.

Between January and this panel, twenty-five drugs were granted Breakthrough status. Panelists from FDA, the pharmaceutical industry, and patient advocacy groups gathered to discuss the successes, the implications, and the ongoing challenges of expedited drug development and approval.

Long-time medical research advocate **Senator Michael Bennet** (D-CO) gave opening remarks, praising the bipartisan nature of the discussion, the work of its participants, and the rapid impact of Breakthrough Therapy. He thanked the panelists for their ongoing efforts in regulatory reform, highlighting the humanitarian and economic value of bringing safe, effective drugs to market as efficiently as possible.

**Dr. Janet Woodcock**, Director of the FDA Center for Drug Evaluation and Research (CDER), began the panel discussion by outlining the purpose and promise of the Breakthrough Therapy designation. As new drugs become increasingly targeted, developers and regulators see unprecedented clinical impact as early as Phase One trials. "If the drug is so much better than anything out there for that disease, we don't want to wait seven years while it's tested in Phase One, Two, and Three," she said.

Dr. Woodcock noted that FDA involvement must be cross-disciplinary and timely, as expedited drug development could leave manufacturing requirements, an aspect of drug development that is often addressed toward the end of the process, as the rate-limiting step in bringing new drugs to market.

**Dr. Jeffrey Leiden**, CEO of Vertex Pharmaceuticals, the developer of the Breakthrough-designated cystic fibrosis drug Kalydeco, described communication with FDA under the new program: "It's a different kind of conversation. It's iterative. It's continuous. It's pick up the phone if you have a problem. It's collaborative. Everything is on the table: the trial size, the trial design, the statistical analysis, the manufacturing process. And that makes the progression of the trial and the progression of the development immeasurably smoother and easier."

**Dr. Jay Siegel**, Head of Global Regulatory Affairs for Johnson & Johnson, sponsor of Breakthrough-designated cancer drugs ibrutinib and daratumumab, echoed the sentiment, stating that ibrutinib's expected approval date has been accelerated by up to two years. "It's not just that we can pick up the phone and call, but they'll pick up the phone and call us and say 'Here's a way you might do this faster.'"

**William Elder, Jr.**, a cystic fibrosis patient taking Kalydeco and speaking on behalf of the Cystic Fibrosis Foundation, discussed his experience with an expedited drug. "This Breakthrough designation changes everything. It changes the way we see our future."

In response to a question by **Moderator Kate Rawson**, Senior Editor for The RPM Report, Director Woodcock described the number of Breakthrough-designated drugs as the result of a fundamental scientific shift. "We're really seeing a new kind of drug development that's driven by understanding the basic science of the disease and intervening on those pathways. There's a tremendous recognition by the clinical staff that this is different." Though she acknowledged that it is still vital to review all drugs efficiently, Breakthrough drugs present such an enormous opportunity that "We can't afford not to do this."





Sen. Michael Bennet (D-CO) delivers keynote remarks

**Dr. Ellen Sigal**, Founder and Chairperson of *Friends*, pointed out that active FDA involvement with Breakthrough-designated drugs is resource intensive and expressed concern for the impact of the federal budget sequester on already tight budgets. "This is about a collaborative scientific process. None of these Breakthroughs came out of the air. They're a result of the hard work of the NIH, academic researchers, patients, industry and FDA coming together to improve people's lives." To cut research and regulatory funding is short-sighted, said Sigal. "If we treat the right population with the right drugs, we will save money, in addition to a lot of pain and suffering."

Dr. Leiden agreed. "The next generation of these remarkable precision medicines won't be there if we cut away at the beginning of the process at the NIH and at the FDA."

Dr. Siegel expressed concern for mismatched expectations between FDA and foreign regulators, particularly regarding Breakthrough-designated drugs. Where FDA attempts to move patients from control groups to effective medicines when possible, European regulators may require longer-term monitoring before any trial adjustments could be made. "Our hope is that foreign regulators will catch up," Dr. Siegel said.

Even after initial approval, panelists anticipated challenges regarding how insurance providers would



(L-R) Jeffrey Leiden, Ellen Sigal, William Elder



Ellen Sigal



(L-R) Jay Siegel, Janet Woodcock, Jeffery Leiden

determine reimbursement for drugs that went through the Breakthrough program. Because of their accelerated development process, Breakthrough drugs can demonstrate the safety and efficacy required to reach the market prior to developing long-term measures on outcomes sought for cost-benefit analysis both overseas and, increasingly, in the United States.

Further discussion touched on the role companion diagnostics play in drug development. Though they will be required for many Breakthrough drugs, their development and manufacturing are not directly subject to the benefits of the Breakthrough Therapy designation.

Dr. Sigal noted *Friends'* ongoing work with CDER and the FDA Center for Devices and Radiological Health (CDRH) to explore strategies to expedite the co-development of diagnostic tools with drugs that have received a Breakthrough Therapy designation. Additional topics of discussion included a desire by both FDA and industry to see the manufacturing process improved, the benefits of publicly-funded research to drug development, and the possibility of large-scale clinical trial networks to ease the trial process for drugs treating rare conditions, where patient volunteers are often scarce.

Overall, panelists were supportive of the Breakthrough Therapy designation and optimistic for the future of drug development and review. So long as the collaboration, the innovation, and the funding that made Kalydeco possible continue, the future of medicine is bright. Said Dr. Leiden, "I think we're standing at the threshold of the most exciting era in biomedicine that we've ever seen. And that's partly scientific, it's partly regulatory. That's what Breakthrough is about." Dr. Sigal wrapped up the conversation emphasizing that all parties do their part and "keep this going, because ultimately this collaboration is going to help patients."

## PRESENTATIONS OF NOTE

APRIL 9, 2013

**AACR Annual Meeting: “Regulatory Science and Policy Session: ‘Breakthrough Therapy’ Designation and Its Implications for Accelerating Oncology Drug Development”****Ellen Sigal** (Moderator), **Jeff Allen** (Panelist)

To potentially mitigate delays in delivering advanced therapies to patients, the Advancing Breakthrough Therapies for Patients Act was introduced and included as a component of the 2012 reauthorization of the Prescription Drug User Fee Act to expedite development of new, potential “Breakthrough” therapies. This legislation specifies that a new drug may be designated as a “Breakthrough Therapy” if it is intended to treat a serious or life-threatening disease and preliminary clinical evidence suggests that it provides a substantial improvement over existing therapies.

This session discussed the following:

- Key components of “Breakthrough Therapy” designation

- How to identify a potential Breakthrough therapy
- How to balance the need to provide sick patients with expedited access to Breakthroughs and the need to protect patients from potentially ineffective or unsafe drugs.

**Panelists:****Jeff Allen**, Friends of Cancer Research**Sandra J. Horning**, Genentech, Inc.**Jonathan Leff**, Deerfield Institute**Howard Scher**, Memorial Sloan-Kettering Cancer Center**Rachel Sherman**, U.S. Food and Drug Administration**Moderator: Ellen V. Sigal**, Friends of Cancer Research

APRIL 17, 2013

**Arnold & Porter / Univ. of Maryland Conference: “Emerging Issues in Food & Drug Law”**

This conference covered a number of important topics within food and drug law, with a particular focus on the then-recently-enacted Food and Drug Administration Safety and Innovation Act (FDASIA).

The timing of this conference was just over nine months after the enactment of FDASIA and followed a more recent spotlight on FDA involvement in drug shortages and its regulation of pharmacy compounding and FDA’s announcement of its FDASIA timeline.

**Panelists:****Leslie Kux**, JD, Assistant Commissioner for Policy, FDA**Patric Frey**, MPP, Office of Planning and Analysis, FDA’s Center for Drug Evaluation and Research (CDER)**Ryan Hohman**, JD, MPA Managing Director, Friends of Cancer Research

Ryan Hohman at the University of Maryland School of Law Conference

**Laura Broch**, PhD, RN, Director, Human Research Protections Office, U.S. Army Medical Research and Materiel Command**Elizabeth Jungman**, JD, MPH, Senior Health Policy Advisory, U.S. Senate HELP Committee

## PRESENTATIONS OF NOTE

APRIL 22, 2013

**BIO Annual Meeting: “Impact of PDUFA and New Expedited Pathway Provisions on the Work of Prescription Drug Regulatory Professionals”****Jeff Allen** (Panelist)

FDA Safety and Innovation Act (FDASIA) passed on July 9, 2012 and implementation started on October 1, 2012. Embedded within FDASIA is the Prescription Drug User Fee Act (PDUFA) Commitment Letter between industry and FDA, which is intended to restore FDA's review performance by strengthening the scientific dialogue and transparency between FDA and the Sponsor.

In addition to PDUFA, FDASIA also includes provisions that intend to accelerate the drug development and review process, improve regulatory procedures, and increase patient access to critical medications—namely, the Breakthrough Therapy and Enhanced Accelerated Approval provisions.

This session addressed the need for regulatory professionals to:

- identify the specific terms of the provisions included in FDASIA,
- effectively assess FDA's progress in carrying out these provisions, and
- discuss the impact of the provisions on the overall drug and device development and review process, regulatory efficiency, and patient access.

APRIL 2, 2013

**NCCN Policy Summit: “Biosimilars and Molecular Testing”****Jeff Allen** (Panelist)

The *NCCN Oncology Policy Summit: Evolving Policy Issues in Oncology – Revisiting Biosimilars and Molecular Testing* was held at the National Press Club in Washington, DC. Stakeholders gathered to examine how biosimilars and molecular testing in oncology have changed since they were addressed by NCCN Work Groups and at the NCCN Policy Summits in 2011. The Summit's agenda included discussion of the current status of these areas, review of the newest guidance documents and regulatory requirements, examination of payer viewpoints and practices, and discussion about where the oncology community is headed on these two important issues. The program, moderated by Clifford Goodman, PhD, of The Lewin Group, consisted of two short presentations and two roundtable discussions with vigorous discussion and audience participation.

This introductory presentation was followed by a roundtable discussion moderated by Dr. Goodman. The panelists included Jeff Allen, PhD, Friends of Cancer Research; Stan Bukofzer, MB, BCh, MMed, Hospira; Leah Christl, PhD, US Food and Drug Administration (FDA); James Hoffman, PharmD, St. Jude Children's Research Hospital; Richard Markus, MD, PhD, Amgen; Lee Newcomer, MD, MHA, UnitedHealthcare; Marjorie Shapiro, PhD, FDA; and Andrew Zelenetz, MD, PhD, Memorial Sloan-Kettering Cancer Center. The discussion revolved around a variety of issues surrounding biosimilars development and their clinical use, including, but not limited to, interchangeability standards, formularies, and substitution practices.

**JUNE 25, 2013**

## **Friends Plays a Leading Role at the DIA Annual Meeting**

### **DIA Annual Meeting: “Breakthrough Therapy: One Candle on the Birthday Cake – Are Innovators Enjoying Sweet Success or Is the Pathway Not Baked Yet?”**

What was been accomplished in the first year of Breakthrough Therapy? This forum explored experiences, lessons learned and how these lessons could inform further regulatory changes intended to spur innovation.

#### **Presentations and Speakers:**

Chairperson

**Nancy Bradish Myers, Esq, JD**, President, Catalyst Healthcare Consulting, Inc

*“Patient Advocacy Perspective on Breakthrough Therapy at One Year”*

**Jeff Allen, PhD**, Executive Director, Friends of Cancer Research

*“Industry/Manufacturing Perspective on Breakthrough Therapy at One Year”*

**Earl S. Dye, III, PhD**, Director, Technical Regulatory Policy, Genentech, A Member of the Roche Group

*“Industry Perspective on Breakthrough Therapy at One Year”*

**Urte Gayko, PhD**, Senior Vice President, Global Regulatory Affairs, Pharmacyclics

---

### **DIA Annual Meeting: “Expediting Drug Development and Review for Serious Conditions”**

This forum provided clarity about FDA's expedited drug development and review programs and ways in which the European Medicines Agency (EMA) enables drug development. It emphasized the importance of expediting drug development to address the critical need for new therapies to treat serious or life-threatening diseases that lack therapeutic alternatives.

#### **Learning Objective(s):**

Panelists discussed FDA's and EMA's processes and programs for expediting drug development. They identified the importance of these processes and programs to speed

the development and review of drugs for serious or life-threatening diseases for patients who lack alternative therapies.

#### **Presentations and Speakers:**

Chairperson

**Robert J. Temple, MD**, Deputy Center Director for Clinical Science, CDER, FDA

*“From Gatekeepers to Enablers: How Drug Regulators Respond to a Challenging and Changing Environment”*

**Hans-Georg Eichler, MD, MSc**, Senior Medical Officer, European Medicines Agency, European Union

*“A Pharma Company Perspective on Expedited Development Pathways”*

**Robert Metcalf, PhD**, Vice President, Global Regulatory Affairs - US, Eli Lilly and Company

Panelist

**Jeff Allen, PhD**, Executive Director, Friends of Cancer Research

---

### **DIA Annual Meeting: “Co-development of Targeted Therapies and Companion Diagnostics: Identifying Regulatory Strategies to Overcome Challenges”**

FDA released a draft guidance in July 2011 that outlined the basics on developing targeted therapies and companion diagnostics, but it left many questions unanswered. This session discussed potential approaches to address these questions.

#### **Panelists and Speakers:**

Chairperson:

**Janet Jenkins-Showalter**, Senior Regulatory Group Director, Regulatory Policy and Intelligence, Genentech, A Member of the Roche Group

**Jeff Allen, PhD**, Executive Director, Friends of Cancer Research

**Erling Thor Donnelly, PhD, RAC**, Manager, Worldwide Regulatory Strategy, Pfizer Inc

**Shayesteh Fuerst-Ladani, MBA, MS**, Director, SFL Regulatory Affairs & Scientific Communication, Switzerland

SEPTEMBER 6, 2013

## Friends of Cancer Research Forum – A Blueprint for Drug/Diagnostic Co-development: Breakthrough Therapies

On September 6, 2013, Friends of Cancer Research and Alexandria Real Estate Equities, Inc. held their second forum on the co-development of drugs and companion diagnostics. The forum brought together researchers, sponsors, advocates, and regulators to discuss possible approaches to the expedited development of a companion diagnostic device (co-Dx) that is intended for use with a Breakthrough therapy, as well as other issues that will arise from the increased use of co-Dx.

Many of the "Breakthrough" drugs designated over the past year are targeted agents that act upon precise molecular structures. Because these targeted therapies only show activity against specific molecules, an IVD co-Dx is required to identify patients and conditions likely to respond to treatment. But although the development and review of Breakthrough drugs is expedited, the regulatory requirements for their diagnostics remain unchanged, prompting concern that slow diagnostic review might hinder the approval and use of Breakthrough therapies. This forum discussed potential modifications to diagnostic development that could be used for co-Dx to therapies that have received Breakthrough designation.

The first panel, **"Development Strategies for Breakthrough Therapy Diagnostics,"** presented **"A Risk-based Approach for In Vitro Companion Diagnostics Device FDA Approval Process Associated with Therapies that have Breakthrough Designation" (September 2013)**, a consensus document developed by a large working group prior to the forum. The report highlighted optimal processes and proposed novel risk-based approaches to drug/diagnostic co-development that would allow diagnostic development to remain on pace with the expedited development of the companion Breakthrough therapy.

**Barbara Conley**, Associate Director of the Cancer Diagnosis Program at the NCI Division of Cancer Treatment and Diagnosis, explained that the risk-based approach is not designed to lower overall standards but to focus on those

activities that prevent or mitigate risks related to the diagnostic product, an idea that was reiterated throughout the forum.

Moderator **Howard Scher**, Chief of the Genitourinary Oncology Service at Memorial Sloan-Kettering Cancer Center, presented two proposals: first, that co-Dx designed for a drug with the breakthrough designation be automatically eligible for priority review and second, that highly coordinated administrative processes and management commitments, similar to those offered with the breakthrough designation for drugs, be used for the review of IVD co-Dx associated with breakthrough therapies.

**Tracy Bush**, Director of Companion Diagnostic Regulatory Affairs for Roche Diagnostics, presented two proposals involving the application of a risk-based approach. The first uses the approach to determine the co-Dx analytical studies required, dependent on assay type, for PMA filing, and the second looks at the requirements for quality systems, manufacturing processes, and software testing and documentation.

**Christine Gathers**, Senior Director of Regulatory Affairs for Diagnostics at Eli Lilly and Company, discussed the panel's final proposal, a "Continued Access" supplement IDE that will allow labs that do not participate in a trial to get experience with a new diagnostic system earlier and faster, allowing for broader commercial availability.

Two panelists representing FDA, **Mike Pacanowski**, Associate Director of Genomics and Targeted Therapy at the Center for Drug Evaluation and Research's (CDER's) Office of Clinical Pharmacology, and **Elizabeth Mansfield**, Director of the Personalized Medicine Staff at the Center for Devices and Radiological Health (CDRH), responded to these proposals.

Dr. Pacanowski commended the proposals, stressing the importance of efficient planning in diagnostic development



(L-R) Howard Scher, Barbara Conley, Tracy Bush, Christine Gathers, Mike Pacanowski, and Elizabeth Mansfield

and increased communication between sponsors and FDA. Dr. Mansfield mentioned that CDRH does prioritize co-Dx, and they do receive priority review. She stated that the administrative suggestions seem quite doable, and she liked the risk-based approach and suggestions towards identifying potential improvements to co-Dx development efficiency. Dr. Mansfield also mentioned that the biggest hurdle in the process may be Quality Systems Regulations.

After the presentation, Dr. Mansfield also highlighted that FDA understands how co-Dx are affecting drug development and regulation and that CDRH and CDER are working extremely closely to discuss new and innovative approaches. **Keith Flaherty**, Director of the Henri and Belinda Termeer Center for Targeted Therapies at the Massachusetts General Hospital Cancer Center pointed out that one concern about the Breakthrough Designation is the potential strain on FDA caused by an "all hands on deck" philosophy towards those products, especially given the large number of applications that have been received. Jeff Shuren, the Director of CDRH, acknowledged that limited resources present a challenge.

The second panel, "**Policy and Practices to Facilitate Personalized Medicine**," moderated by Senior *Nature Medicine News* Editor **Elie Dolgin**, responded to Panel One's proposals and provided perspectives regarding the

opportunities and challenges that exist around co-Dx development and the implementation of "precision medicine." Highlighted topics included the growing role of molecular diagnostics in medicine, how reimbursement for co-Dx is being approached by insurance companies, and the role databases and registries may play in improving drug development.

**Jeff Shuren**, Director of CDRH, stated that CDRH is working on formalizing a policy shifting certain pre-market requirements to post-market, much like the accelerated approval process for drug development. He also reiterated FDA's support for the suggestions proposed by Panel One, stating, "What is coming out of the recommendations today from the white paper are considerations we would look to potentially fold into [a] draft policy." When asked about the need for additional legislation, Dr. Shuren said that although it could be helpful, there's a lot the CDRH can do with its current authority.

**Janet Woodcock**, Director of CDER, mentioned her hope that Breakthrough would expand from targeted, genetic diseases, like cancers, to resistant bacterial infections. **Bill Chin**, PhRMA's Executive Vice President for Science and Regulatory Affairs, commented that someday all drugs should be breakthroughs, but this has not yet been possible because our current understanding of disease is so limited.

Dr. Flaherty brought up the role of payers in a landscape where many diagnostic markers are still exploratory. **Matt Zubiller**, Vice President of Decision Management for McKesson Health Solutions, explained McKesson's attempts to develop a new coding system for insurance forms that will determine the value of different diagnostic tests being used and identify the clinical utility of the tests. Diagnostic tests are not currently reimbursed based on value, but pharmacy benefit and medical benefit should be important considerations in diagnostic reimbursement.

A discussion ensued about the decision-making process in determining treatment and the role that cost plays in those decisions. **Michael Kolodziej**, National Medical Director of Oncology Solutions at Aetna, pointed out that as more choices of drugs and treatments are available at multiple prices, coverage decisions will have to be made based on that. Kolodziej stressed the need for more effective treatment as well as the benefits of co-Dx, stating, "We have to find the way to get the right drug to the right patient."

Dr. Flaherty mentioned the inefficiency of unused data collected in clinical trials. Although the current weaknesses in databases and registries was acknowledged, it was pointed out that diagnostic and drug development will benefit from shared investment and academics and industry will have to compromise to share data and improve trial designs. Dr. Chin stressed the importance of balancing rigor with innovative science.



Janet Woodcock



Elizabeth Mansfield

#### PANEL ONE:

#### **Development Strategies for Breakthrough Therapy Diagnostics**

##### *Panelists*

**Elizabeth Mansfield**, Director, Personalized Medicine Staff, Center for Devices and Radiological Health, FDA

**Mike Pacanowski**, Associate Director for Genomics and Targeted Therapy, Office of Clinical Pharmacology, FDA

**Christine Gathers**, Senior Director of Regulatory Affairs for Diagnostics, Eli Lilly and Company

**Barbara Conley**, Associate Director of the Cancer Diagnosis Program, Division of Cancer Treatment and Diagnosis, National Cancer Institute

**Tracy Bush**, Director of Companion Diagnostic Regulatory Affairs, Roche Diagnostics

*Moderator:* **Howard Scher**, Chief of the Genitourinary Oncology Service, Memorial Sloan-Kettering Cancer Center

#### PANEL TWO:

#### **Policies and Practices to Facilitate Personalized Medicine**

##### *Panelists*

**Jeff Shuren**, Director, Center for Devices and Radiological Health, FDA

**Janet Woodcock**, Director, Center for Drug Evaluation and Research, FDA

**Matt Zubiller**, Vice President, Decision Management, McKesson Health Solutions

**Keith Flaherty**, Director, Henri and Belinda Termeer Center for Targeted Therapies, Massachusetts General Hospital Cancer Center

**Michael Kolodziej**, National Medical Director, Oncology Solutions, Aetna

**William Chin**, Executive Vice President for Science and Regulatory Affairs, PhRMA

*Moderator:* **Elie Dolgin**, Senior News Editor, Nature Medicine



MAY 16, 2013

## ***Friends*** Co-hosts Annual Congressional Advocacy Day on Capitol Hill

On May 16, 2013, Friends of Cancer Research (*Friends*) joined the American Association for Cancer Research, the Association of American Cancer Institutes, and the American Society of Clinical Oncology, to boost congressional support for cancer research and biomedical science.

The five organizations brought more than 100 researchers, clinicians and advocates to Capitol Hill to meet with 150 House and Senate aides and lawmakers, including House and Senate leadership, from 30 states. Advocates called on lawmakers to approve a fiscal year 2014 budget that will fund the NIH and FDA at a level that stakeholders say is necessary to sustain scientific momentum.

While in Washington, the organizations also took time to recognize U.S. Rep. Rosa DeLauro (D-CT) and Sen. Richard Shelby (R-AL) for their tireless work to enhance appropriations for NIH and their commitment to cancer research. The lawmakers were honored with cancer leadership awards during an evening reception.



▲ House Minority Leader  
Nancy Pelosi (D-CA)

▶ (L-R) Ellen Sigal  
Rep. Rosa DeLauro (D-CT)  
Rep. John Larson (D-CT)



(L-R) Barbara Duffy Stewart, Ryan Hohman, Ellen Sigal, Cliff Hudis, and Hayden Rhudy discuss funding for biomedical research

## PRESENTATIONS OF NOTE

FEBRUARY 28, 2013

**Alexandria Summit: “Optimizing Collaborations to Enhance Research Policy”****Ellen Sigal** (Moderator)

The Alexandria Summit™ is an annual, invitation-only gathering of scientific thought leaders that will help shape future directions in life science research and development and regulatory reform. The Alexandria Summit fosters a constructive and interactive platform for discussion and debate among the world's foremost visionaries from the

pharmaceutical and biotechnology industry, medical, academic, financial, philanthropic and advocacy groups, and government, to exchange ideas regarding critical and timely issues, advance knowledge, and tackle the most important global health care challenges.

MARCH 7, 2013

**Congressional Briefing – Healthcare Leadership Council: “Healthcare Innovation and Progress in a Sunshine Era: Establishing Principles for 21st Century Collaboration”**

The purpose of this briefing was to educate Congressional staff and others on the value of principled collaboration, introduce NDHI's Principles Statement, share initial reactions to the final Sunshine provisions and highlight the potential relationship between federal regulations and future medical innovation.

Panelists:

**David Caraway, MD, PhD**, St. Mary's Medical Center**Guy Chisolm, PhD**, Director, Innovation Management and Conflict of Interest Program, Cleveland Clinic**Ryan M. Hohman, JD, MPA**, Managing Director, Policy & Public Affairs, Friends of Cancer Research**Mary R. Grealy**, President of the Healthcare Leadership Council, moderated the briefing

APRIL 17, 2013

**PMC/BIO Solution Summit: “Evidentiary Standards and Data Requirements for Payer Coverage”****Jeff Allen** (Panelist)

This panel was designed to dive directly into the topic of how payers make coverage decisions and what evidence and standards must be brought to them to support their decision-making process. Dr. Allen noted that in fields like leukemia, greater diagnostic precision over several decades was accompanied all along the way by steady advancements in survival and even cures. The costs of, or the value of, more accurate diagnosis of leukemia doesn't seem to have been questioned. Today, the costs, methods, data, and regulatory standards for improved diagnostics get far more scrutiny, including both the FDA and payer levels. Conway noted that the horizon of molecular tests goes beyond the traditional categories of “diagnosis” and “subtype,” by providing genomic landscapes of each patient's tumors, studying up

to several hundred genes at once. This can have a dramatic impact on cancer care, but an entire system of evidence, payment, and reimbursement needs to be updated and shaped to this kind of transformational testing.

**Jeff Allen, Ph.D.**, Executive Director, Friends of Cancer Research**Jerry Conway**, Vice President of Reimbursement & Payer Strategy, Foundation Medicine**Tamara Syrek Jensen, J.D.**, Deputy Director, Coverage and Analysis Group, CMS**Matthew Zubiller**, Vice President for Decision Management Business, McKesson Corporation**Moderator: Kristin Ciriello Pothier**, Partner, Health Advances

**FEBRUARY 6, 2013**

## Friends of Cancer Research Co-Hosts Capitol-Hill Briefing:

### The Blueprint for Medical Research: How New Medicines Get from the Lab to the Patient

On February 6, 2013 more than 300 policy decision makers, research advocates, key legislative staffers, and members of Congress attended a Congressional briefing "The Blueprint for Medical Research: How New Medicines Get from the Lab to the Patient" co-sponsored by Friends of Cancer Research and FasterCures. At the briefing, medical research leaders representing distinct sectors provided a glimpse into what it takes to turn a scientific discovery into a safe and effective therapy that will improve, and maybe even save, patients' lives.

Panelists included: Francis Collins, Director of the National Institutes of Health (NIH); Margaret Hamburg, Commissioner of the U.S. Food and Drug Administration (FDA); Ellen Sigal, Chair & Founder of Friends of Cancer Research (Friends); Deborah Brooks, Co-Founder of the Michael J. Fox Foundation for Parkinson's Research; Anthony Coles, president and CEO of Onyx Pharmaceuticals; Roy Jensen, Director of the University of Kansas' Cancer Center; Michael Milken, Founder of FasterCures and Chairman of the Milken Institute; and was Moderated by FasterCures Executive Director Margaret Anderson

"There is no one in this room who hasn't been touched by disease, whether it's yourself or a loved one," said Friends of Cancer Research's Chair and Founder, Ellen Sigal, in opening the briefing "We cannot let the FDA or NIH become a victim of political polarization."

Francis Collins, Director of the National Institutes of Health (NIH), said that "while I can tell you there's never been a more exciting time for science, I can also tell you there's never been a more stressful time." He was addressing the issue at hand -tightening fiscal resources threaten the ability of the research and development ecosystem to deliver science's full potential to improve health and well-being.



(L-R) Margaret Anderson, Francis Collins, Roy Jensen, Deborah Brooks, Anthony Coles, Michael Milken, Margaret Hamburg

Improving and accelerating research and development requires all stakeholders in the medical research ecosystem to work together - including federal agencies, industry, academia, and patient groups. Collaboration - existing collaborative efforts, and the need for even more partnerships - was a resounding theme throughout the discussion.

Disease research organizations feel the excitement for an improved system. "This is the most robust pipeline we've seen. Now is not the time to step back from working together, but to do more," said Deborah Brooks, Co-Founder of the Michael J. Fox Foundation for Parkinson's Research.

"The FDA is the final common pathway to translating science to patients," said Margaret Hamburg, Commissioner of the U.S. Food and Drug Administration (FDA). "If the FDA is not fully funded and supported, the ecosystem will not function optimally. When we engage early with the scientific community, we're able to cut five years off of the drug development process."

Roy Jensen, Director of the University of Kansas' Cancer Center, is already seeing how limited resources are impacting research progress. "We're not even funding a fraction of the best science. We're starting to cut to the bone - scientists are having to close labs, they aren't able to train the next generation... It's fundamentally altering our infrastructure," said Jensen.

The importance of supporting the next generation of brilliance and innovation weighs heavily on the minds at the NIH, and researchers are hopeful for the next group of young investigators. "It is very tough right now to be a grad student, or a post-doc. As they look at the landscape of this country, they wonder if there's room for them," Collins said. Coles echoed Collins' concern with a sobering concept about the future of scientific innovation: "What answers won't we have in 10 years if we don't fund this research?"

SEPTEMBER 23, 2013

## 17th Annual Cancer Leadership Awards Reception

### Inauguration of the Ellen V. Sigal Advocacy Leadership Award



On September 23, 2013, **Friends of Cancer Research** welcomed leaders from government, advocacy, industry, and science to its **17th Annual Cancer Leadership Awards Reception** to celebrate another year of partnership and innovation on behalf of patients.

The annual reception is an opportunity to recognize extraordinary work across the fields of cancer research, awareness, treatment, and detection. Past honorees include Senator Edward Kennedy, Secretary Kathleen Sebelius, Senator John McCain, Secretary Michael Levitt, Senator Daniel Inouye, Senator Judd Gregg, Dr. Francis Collins, Senator Arlen Specter, and Congressman John Dingell.

This year, *Friends* established the **Ellen V. Sigal Advocacy Leadership Award** to celebrate the groundbreaking work of the organization's Chair & Founder. For over 17 years, Friends has created unique public-private partnerships and developed innovative approaches to cancer research and treatment.



The inaugural award, which will be given each year to an outstanding advocate for cancer research, was presented to advocate, philanthropist, businesswoman, and legendary former Paramount Pictures CEO **Sherry Lansing**. By founding The Sherry Lansing Foundation and co-founding The Entertainment Industry Foundation and StandUp2Cancer (SU2C), she has inspired innovation, funded dream-teams of scientists, and changed the landscape of cancer research as we know it.

*"As you think about this award being given this year to Sherry Lansing, and in the future to other remarkable leaders, you would want it to have a name attached to it that represents the best of what cancer advocacy can be,"* said **NIH Director Francis Collins** in a special video for Dr. Sigal shown before the award was given.

The video included special messages from many leaders in the community, including **Senator Michael Bennet**, **Congresswoman Diana DeGette**, and **FDA Commissioner Margaret Hamburg**.

Top: Gov. Martin O'Malley

Bottom: (L-R) Ellen Sigal, Janet Woodcock, Gov. Bob McDonnell



**Governor Bob McDonnell** of Virginia and **Governor Martin O'Malley** of Maryland received Friends of Cancer Research Leadership Awards for their extensive support of public and private medical research. One of Governor O'Malley's signature achievements has been the development of BioMaryland 2020, a strategic ten-year investment of \$1.3 billion into Maryland's life sciences research. During his tenure, Governor McDonnell increased private and public biotech investment in his state, including the bolstering of world-class cancer centers at the University of Virginia and Virginia Commonwealth University.

All of us at Friends of Cancer Research would like to thank our Board of Directors and our supporters, colleagues, and collaborators from academia, industry, and advocacy for another incredible year. We are deeply appreciative of all you have done to help continue to grow and work for patients everywhere.



Top: (L-R) Marlene Malek, Sherry Lansing, Ellen Sigal  
Bottom: Margaret Hamburg and Anthony Fauci

# Board of Directors

## EXECUTIVE COMMITTEE:

**Ellen V. Sigal, Ph.D.**

Chair & Founder, *Friends of Cancer Research*

**Marlene Malek, R.N.**

President, *Friends of Cancer Research*

**Barbara Duffy Stewart, M.P.H.**

Executive Director, *Association of American Cancer Institutes, Secretary*

**Sudip S. Parikh, Ph.D.**

Vice President & General Manager, Health & Analytics, *Battelle Memorial Institute, Treasurer*

**Otis Brawley, M.D.**

Chief Medical Officer, *American Cancer Society*

**Margaret Foti, Ph.D., M.D. (h.c.)**

Chief Executive Officer, *American Association for Cancer Research*

**Sherry Lansing**

Chief Executive Officer, *The Sherry Lansing Foundation*

**Richard Schilsky, M.D.**

Chief Medical Officer, *American Society of Clinical Oncology*

## BOARD OF DIRECTORS:

**Pennie Abramson, Ph.D. (h.c.)**

President's Circle, *The Weizmann Institute of Science*

**Carolyn "Bo" Aldige**

President & Founder, *Prevent Cancer Foundation*

**Anna Barker, Ph.D.**

Professor & Director, Transformative Healthcare Networks, *Arizona State University*

**Marguerite D. Baxter, R.N., M.N.**

Health Policy Consultant

**William P. Bro**

CEO, *Kidney Cancer Association*

**Nancy Davenport-Ennis**

CEO, *Patient Advocate Foundation*

**Deborah I. Dingell**

Chair, Manufacturing Initiative, *American Automotive Policy Council*

**Jonathan Leff**

Partner, *Deerfield Management*

**Joel S. Marcus, J.D., CPA**

Chairman, Chief Executive & Founder, *Alexandria Real Estate Equities, Inc.*

**Catherine Ann Stevens, J.D.**

Partner, *Mayer Brown LLP*

**Mary Woolley**

President, *Research!America*

**Robert C. Young, M.D.**

President, *RCYMedicine*

# Supporters

## **INNOVATORS**

Alexandria Real Estate Equities, Inc./ Alexandria Venture Investments

Amgen, Inc.

Celgene Corporation

Eli Lilly and Company

Genentech

GlaxoSmithKline

Marlene and Fred Malek

Millennium: The Takeda Oncology Company

Novartis Pharmaceuticals Corporation

Pfizer Inc

Pharmaceutical Research and Manufacturers of America (PhRMA)

Ellen and Gerry Sigal

## **CHAMPIONS**

AbbVie

American Association for Cancer Research (AACR)

American Society of Clinical Oncology (ASCO)

Bayer HealthCare

Bristol-Myers Squibb Company

CJM Foundation

Entertainment Industry Foundation (EIF)

Janssen R&D

Susan G. Komen for the Cure

The Kovler Fund – Judy and Peter Kovler

Merck

Ogden CAP Associates, LLC

Onyx Pharmaceuticals Inc

# Supporters

## COLLABORATORS

Abramson Family Foundation  
 American Cancer Society (ACS)  
 Association of American Cancer Institutes (AACI)  
 AstraZeneca  
 Battelle  
 Boehringer Ingelheim Pharmaceuticals, Inc.  
 Eli Lilly and Company  
 Foundation Medicine  
 Gilead Sciences  
 Johnson & Johnson  
 The Sherry Lansing Foundation  
 Jonathan Leff  
 Annette M. and Theodore N. Lerner Family Foundation  
 Lionsgate  
 MedImmune  
 Miller & Long  
 National Patient Advocate Foundation (NPAF)  
 National Pharmaceutical Council  
 QIAGEN N.V.  
 Shire  
 Silverstein Properties, Inc.  
 The Truland Foundation

## PARTNERS

The Abe and Kathryn Selsky Foundation  
 Inova Health System  
 AdvaMedDx  
 Biotechnology Industry Organization (BIO)  
 CDW  
 City of Hope National Medical Center and Beckman  
 Research Institute  
 Cleveland Clinic Taussig Cancer Institute  
 Deerfield Management  
 Duke University Medical Center  
 Mr. and Mrs. William H. Goodwin, Jr.  
 Sam and Peggy Grossman Family Foundation

Incyte Corporation  
 Jonsson Comprehensive Cancer Center, UCLA  
 The Leukemia & Lymphoma Society (LLS)  
 Lombardi Comprehensive Cancer Center at Georgetown  
 University  
 The Meltzer Group  
 Moffitt Cancer Center  
 OSU James Cancer Hospital and Solove Research Institute  
 Paramount Pictures  
 Sidney Kimmel Comprehensive Cancer Center at Johns  
 Hopkins University  
 University of Texas MD Anderson Cancer Center

## ADVOCATES

Helen Abel  
 Agios Pharmaceuticals  
 Miriam and Sandy Ain  
 Jeff Allen and Kevin Kenney  
 Charlotte Anker  
 Lawrence Becker  
 Richard and Donna Ben-Veniste  
 BioMaryland  
 Philip Bobrow  
 David Bonderman  
 Susan Butler  
 Bill and Janet Cohen  
 Jerry and Adrienne Cohen  
 The Ryna and Melvin Cohen Family Foundation Inc.  
 Dockser Family Foundation  
 EagleBank  
 Edelman  
 Alvin Einbender  
 Faegre BD Consulting  
 Joel and Barbara Finkelstein  
 Lucy Fisher and Doug Wick  
 Geller Design Group  
 Ira and Judith Gottfried Fund



# Supporters

Joseph Grossman  
Grossman Family Foundation  
GW Medical Faculty Associates  
Michael Mandel and Sherry Haber  
Ted Greenberg and Tammy Haddad  
Mary Haft  
Pamela and Gerard Heiber  
Ryan Hohman  
JP Morgan  
Kidney Cancer Association  
Annette Kirshner  
Edward Lenkin  
Leslie and Michael Tive  
Mirella and Dani Levinas  
Lung Cancer Alliance  
Phyllis and Philip Margolius  
David Mitchell  
David and Barbara Moser  
Shelley and Tommy Mulitz  
National Comprehensive Cancer Network (NCCN)  
National Lung Cancer Partnership  
National Venture Capital Association  
Mary Ann Nordheimer  
Melanie and Larry Nussdorf  
Patient and Partners LLC  
Pfizer Foundation Matching Gifts Program  
Prevent Cancer Foundation  
John and Susan Rathgeber  
William and Renay Regardie  
Terri and Bernie Sanker  
Catherine Stevens  
Francine Trachtenberg  
University of Miami Sylvester Comprehensive Cancer Center  
Vornado/Charles E. Smith  
Myles Wittenstein  
Zydowsky Consultants

## **PATRONS**

Academy Health  
AT&T  
Shalom Baranes  
L. Ann and Jonathan P. Binstock  
The Brooks Group and Associates, Inc  
Chip Diggins  
Deborah Dokken  
Stephen Dolman  
Dan and Marina Ein  
Lois England  
Alvin Fettner  
Richard and Barbara Goldberg  
Jeffrey Kirshner  
Debra Kirshner  
Lasker Foundation  
Roger Meyer  
Tom and Bonnie Miller  
National Business Group on Health  
Pancreatic Cancer Action Network (PanCAN)  
Personalized Medicine Coalition  
Research!America  
Berrett Stradford  
Carol Striler  
Ashleigh Talberth  
Dan and Ann Von Hoff  
Steven Young  
ZIOPHARM Oncology

## **In-Kind**

Alston + Bird  
GCS, Inc.  
SIGAL Construction

## About Us

Friends of Cancer Research (*Friends*) is a cancer research think tank and advocacy organization based in Washington, DC. Friends is a leader in developing partnerships and advocating for policies that will get treatments and therapies to patients in the safest and quickest way possible. Working with federal health agencies, Congressional leadership, academic research centers and private sector industry, *Friends* continues to create innovative education, policy, and scientific approaches to approve health outcomes in cancer care.

Founded to mark the 25th Anniversary of the National Cancer Act, *Friends* set out to organize highly effective public policy forums that bring together researchers, leaders of the FDA, NCI, and NIH, industry, elected officials, and patients to discuss critical issues and develop collaborative strategies to assist in the translation of research to treatments and therapies.

When she founded the organization in 1996, Dr. Ellen Sigal saw a compelling need to increase public awareness and support for cancer research and for increased scientific capacity across all federal health agencies. At

that time, Ellen was a Presidential Appointee to the National Cancer Advisory Board along with Marlene Malek, who joined Ellen in 1996 as President of *Friends*.

*Friends* began tackling its mission by holding educational “town halls” across the nation, bringing leaders from science, industry, and academia to the district or home state of key members of Congress. By doing so, Ellen and Marlene were able to not only educate lawmakers but also create new champions for biomedical research. Now, 18 years later, *Friends* continues to expand upon its expertise in developing unique partnerships and creating a more open dialogue among both public and private sectors.

As a respected independent think tank, *Friends* is able to cut through bureaucratic red tape, put aside partisan politics and engage all stakeholders, producing real results.

Join us as we strive to bring new research, treatment, and hope to patients and families battling cancer.

Friends of Cancer Research is a 501(c)(3) non-profit organization.

## Thank you to all of Friends of Cancer Research staff and collaborators who contributed to our work in 2013 and in 2014.

**A special thanks to:** **Jeff Allen, PhD**, Executive Director  
**Ryan Hohman, JD**, Managing Director, Policy & Public Affairs  
**Heather Chaney**, Director, Programs  
**Berrett Stradford**, Director, External Affairs  
**Samantha Roberts, PhD**, Science Policy Analyst  
**Michael Shea**, Policy Research Associate  
**Ryan Dull**, Communications Associate  
**Marina Kozak, PhD**, Science Policy Analyst  
**Nate Higbie**, Executive Assistant  
**Joe Patterson**, Administrative Coordinator  
**Tyler Coffey**, IT Manager  
**Richard Goldberg**, Executive Vice President and CFO, SIGAL  
**Angela Thompson**, Manager of Employee Benefits & Compensation, SIGAL

APRIL 19, 2013

## New Friends Website Goes Live

On April 19, 2013, *Friends* launched a redesigned website intended to improve navigation, increase visual appeal, and make our work accessible to a broader audience. Our home page and menu have been restructured to more prominently feature important news and more conveniently archive older work. The new site includes a blog, “Engaging Innovation,” that hosts conversations and interviews with experts on critical issues in cancer care and a glossary of cancer and drug development terms to help patients better understand medical research and regulation as it applies to *Friends’* work.





Friends of Cancer Research  
1800 M Street, NW | Suite 1050 South | Washington, DC 20036  
[www.focr.org](http://www.focr.org)

