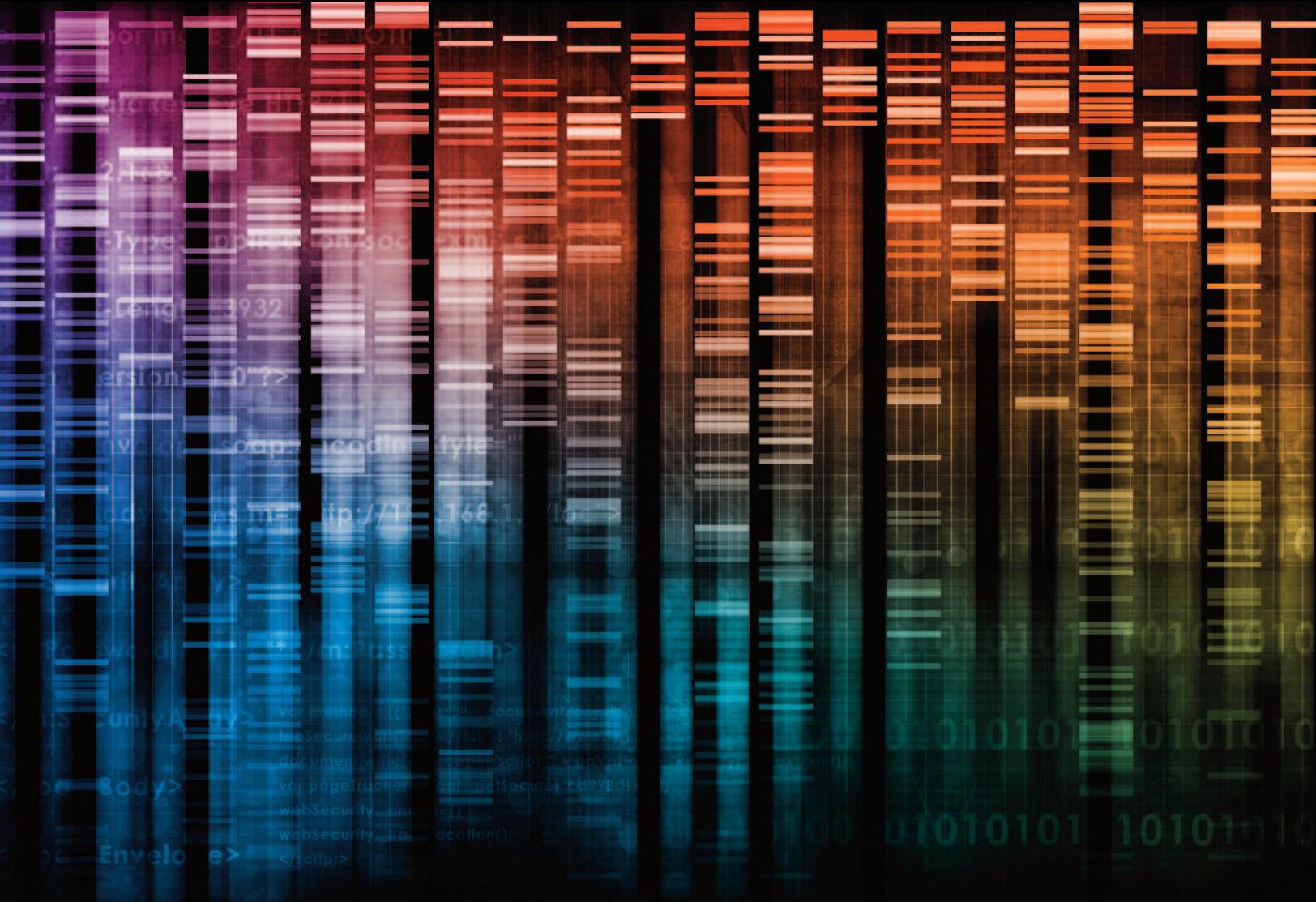




2014 ANNUAL REPORT

Accelerating Science and Technology



2014 Highlights

March 7, 2014

Friends hosts forum on exploring new disease settings for the groundbreaking master protocol clinical trial design

April 30, 2014

Friends of Cancer Research leadership chosen for prominent roles in House Energy and Commerce Committee's "21st Century Cures" Initiative

May 6, 2014

Friends hosts Congressional Briefing: "Science and Progress at the FDA" on the Progress of the FDA's Breakthrough Therapies Program

May 8, 2014

Friends co-hosts annual Congressional Advocacy Day on Capitol Hill

June 16, 2014

Lung-MAP clinical trial launches across the country

September 23, 2014

Friends hosts 18th Annual Cancer Leadership Awards Reception honoring Dr. Margaret Foti, Governor Deval Patrick & Governor Bobby Jindal

September 18, 2014

Friends announces partnership with the Aspen Institute Health, Medicine and Society Program

October 17, 2014

Aspen Institute and Friends of Cancer Research: "Curing Cancer: How Close Are We?"

November 21, 2014

7th Annual Friends of Cancer Research-Brookings Institution Conference on Clinical Cancer Research

September 30, 2014

Friends hosts forum with Alexandria: "A Blueprint for Drug/Diagnostic Co-development: Next Generation Sequencing (NGS)"



A Unique Model to Advance Biomedical Research

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 the discoveries that 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.

Lung-MAP Clinical Trial Launches Across the Country

On June 16, 2014, Friends of Cancer Research announced the launch of the Lung Cancer Master Protocol (Lung-MAP) clinical trial. Lung-MAP is the product of years of collaboration with government, research institutions, patient advocacy groups, and industry, and it is our hope that it will provide a model for more efficient, cooperative trials in the future.

Lung-MAP was developed through a unique public-private collaboration among the National Cancer Institute (NCI), part of the National Institutes of Health; SWOG Cancer Research; Friends of Cancer Research (*Friends*); the Foundation for the National Institutes of Health (FNIH); the US Food and Drug Administration (FDA); five pharmaceutical companies (Amgen, Genentech, Pfizer, AstraZeneca; and AstraZeneca's global biologics R&D arm, MedImmune); and Foundation Medicine.

Lung-MAP is a multi-drug, multi-arm, biomarker-driven clinical trial for patients with advanced squamous cell lung cancer. Squamous cell carcinoma represents about a quarter of all lung cancer diagnoses, but there are currently few treatment options beyond surgery for the disease. The trial uses genomic profiling to match patients to one of several different investigational

treatments that are designed to target the genomic alterations found to be driving the growth of their cancer. This innovative approach to clinical testing should both improve access to promising drugs for patients and ease the significant recruitment and infrastructure burdens on researchers involved in traditional clinical trials.

The trial was first proposed at the 2012 Conference on Clinical Cancer Research hosted by *Friends* and the Engelberg Center for Health Care Reform at the Brookings Institution. *Friends*, in conjunction with its government and industry partners, then developed a clinical trial design in a series of workshops, forums, and working groups. The final design was announced at the 2013 Conference on Clinical Cancer Research, along with the first five drugs to be tested.

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. We would like to thank our partners, our supporters, and all of the volunteers who have chosen to enroll in Lung-MAP for their continued work on behalf of this innovative new trial and the patients it serves.

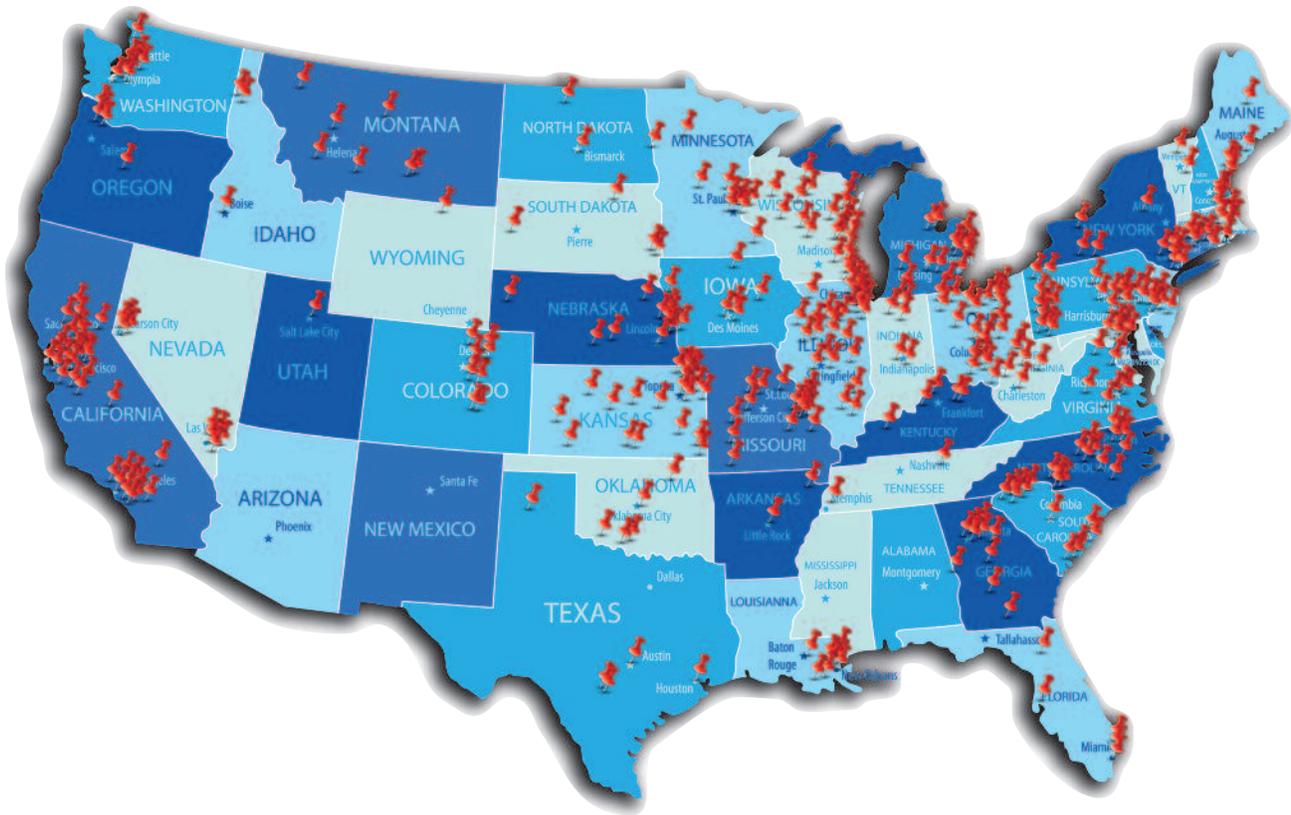
How Lung-MAP Works

- **Genomic Profile Screening:** Patients are screened using a comprehensive genomic profiling platform that looks at over 200 cancer-related genes for genomic alterations.
- **Sub-study Assignment:** Based on the results of this screening, patients are assigned to whichever one of the sub-studies testing different investigational treatments best suits their genomic profile.
- **Innovative Approach:** This innovative approach improves a patient's likelihood of receiving a drug targeted at the genetic profile of their particular tumor while allowing for new therapies in development to be added as the trial progresses.

609 Activated Trial Sites

212 Patients Enrolled

45 States Across the Country



Lung-MAP Key Points

OVERCOMING CURRENT HURDLES IN CLINICAL TRIALS

- Taking an investigational drug from the initial discovery stage through clinical testing and regulatory review is complicated, expensive, and inefficient.
- Trials are difficult to initiate, infrastructure-intensive, subject to lengthy regulatory review, and reliant on the enrollment of volunteers—all challenges compounded by the fact that investigational drugs are almost always tested in separate research studies, even when multiple drugs are being developed to treat the same condition.
- 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. Additionally, reliance on multiple, single-gene diagnostic tests can increase infrastructure costs, complexity, and patient burden.
- The Lung-MAP trial has the potential to change and accelerate the way investigational biomarker-defined therapies are tested and approved for lung cancer, and eventually for many other diseases.



Lung-MAP Study Principal Investigators

- **David Gandara**, Director, Thoracic Oncology Program, UC Davis Comprehensive Cancer Center
- **Roy Herbst**, Chief of Medical Oncology, Yale Cancer Center
- **Vali Papadimitrakopoulou**, Professor, Department of Thoracic/Head and Neck Medical Oncology, MD Anderson Cancer Center

Lung Cancer Master Protocol Timeline

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.

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 in November of 2013, along with the first five participating drugs.

The resulting trial is Lung-MAP (Lung Cancer Master Protocol): a Phase 2/3 trial which tests multiple new therapies simultaneously in separate randomized treatment arms for patients with advanced stage refractory squamous cell NSCLC.

NOVEMBER 2012

MARCH 2013

NOVEMBER 2013

JUNE 15, 2014

JULY 15, 2014

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 2/3 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.

Lung-MAP officially launched, activating at cancer centers and clinics nationwide. News stories about the trial were carried by over 550 outlets and the trial's website, Lung-MAP.org, received over 2000 visits in its first week.

The trial had already opened at over 250 locations across the United States.

***Friends* Hosts Forum on Exploring New Disease Settings for the Groundbreaking Master Protocol Clinical Trial Design**

Building on our success in pioneering a biomarker-driven master protocol for squamous cell non-small cell lung cancer (Lung-MAP), *Friends* has been working with experts in multiple sectors to initiate a similar master protocol Phase 2 screening trial in metastatic colorectal cancer. Colon cancer is the second leading cause of cancer death in the United States, with approximately 141,210 new cases diagnosed per year. The Cancer Genome Atlas (TCGA) revealed colorectal cancer to be comprised of several distinct tumor types, many of which may be defined by the mutational profile of the tumor and may potentially be targeted by novel therapeutics. Therefore, an approach in which multiple genomic aberrations are screened at once and then patients are assigned to biomarker-specific sub-studies within an umbrella trial may be appropriate in this disease setting. To develop consensus on the optimal design of a master screening trial, identify interested industry parties, and develop the overall governance structure, a working group consisting of experts from FDA, NCI, FNIH, academia, advocacy and industry was convened on March 7, 2014 in Washington, DC.

Dr. Roy Herbst, Chief of Medical Oncology, Yale Cancer Center, delivered an introductory presentation to meeting participants, describing lessons learned in the development of the Lung-MAP trial. He emphasized the need to have strong governance and project management, for participants to recognize that the trial design must provide value for all stakeholders, and above all to remember that the goal is to help patients. He also discussed the need to plan carefully: drug selection is critical, as is selection of endpoint and screening platform. Finally, he said that for a master protocol to be successful, it must be flexible and ready to adapt to new science.

Dr. Peter O'Dwyer, Director, Developmental Therapeutics Program, Abramson Cancer Center presented the origin and design of the proposed colon cancer master protocol trial ("ASSIGN"). He described how the NCI Colon Task Force sub-committee was formed and charged with

implementing genomic advances into clinical research. Through iterative discussions with members of NCI's Cancer Therapy Evaluation Program and others in cooperative groups and academic institutions, a consensus outline was developed. In the proposed trial, patient biopsies would be screened up-front using next-generation sequencing and immunohistochemistry. Biomarkers to be screened include KRAS/NRAS mutation, BRAF mutation, PTEN expression or mutation, AKT1 mutation, EGFR expression, HER2 amplification, c-MET amplification, and IGF2 expression. Following first-line standard of care treatment, patients would be assigned to molecularly defined subgroups based on screening results. These sub-studies would test the addition or substitution of a pathway-appropriate targeted therapy to the maintenance chemotherapy regimen. The primary objective would be to determine if there is an improvement in progression-free survival with any of these targeted agents. Because this trial would be conducted under the aegis of the Colon Task Force and within the National Clinical Trials Network, it is expected that it would be able to accrue approximately 1,000 patients per year.

Through the course of the workshop, participants focused on choice of disease setting, chemotherapy backbone, source of biopsy, target effect size, screening platform, and biomarker and drug selection. All of these factors were discussed in the context of the path to registration: ultimately the goal of the trial is to advance effective drugs through the pipeline so that they can gain FDA approval and become available for patients in need. This collaborative, biomarker-driven approach to clinical trial design will lead to improved knowledge, better opportunities for trial participants and, ultimately, new hope for patients.

Congress Launches Initiative to Accelerate the Pace of Cures and Medical Breakthroughs

Friends of Cancer Research Leadership Chosen for Prominent Roles in House Energy and Commerce Committee’s “21st Century Cures” Initiative

Friends continues to play a central role in developing and championing legislation intended to improve the development of innovative therapies. On April 30, 2014 Congresswoman **Diana DeGette (D-CO)** and Congressman **Fred Upton (R-MI)** announced the launch of the "21st Century Cures Initiative," a public discussion regarding the state of biomedical innovation in the United States and new ways to accelerate the process of bringing new treatments and cures to patients. The initiative would look at the full arc of medical innovation - from discovery to development to delivery - to determine what steps could be taken to best make use of the advances the United States has made in science and technology. The process would culminate in new legislation.

On May 6, 2014, *Friends* Chair & Founder, **Dr. Ellen Sigal** joined NIH Director **Dr. Francis Collins** and FDA's **Dr. Janet Woodcock** as a participant on the House Energy and Commerce Committee's kickoff event for the "21st Century Cures Initiative." For that roundtable, spearheaded by Representatives DeGette and Upton, the committee hosted thought leaders in the first public discussion regarding the state of biomedical innovation in the United States. **Jonathan Leff**, Partner at Deerfield Management and a *Friends* Board Member, was also a participant.

At the second event around this initiative, on May 20, 2014, *Friends'* Executive Director **Dr. Jeff Allen** testified before the House Energy and Commerce Subcommittee on Health in a hearing entitled, "21st Century Cures: The President's Council of Advisors on Science and Technology (PCAST) Report on Drug Innovation."



Ellen Sigal at the 21st Century Cures Roundtable



Jeff Allen testifies before the House Energy and Commerce Subcommittee on Health

Friends of Cancer Research Announces Partnership with the Aspen Institute Health, Medicine and Society Program

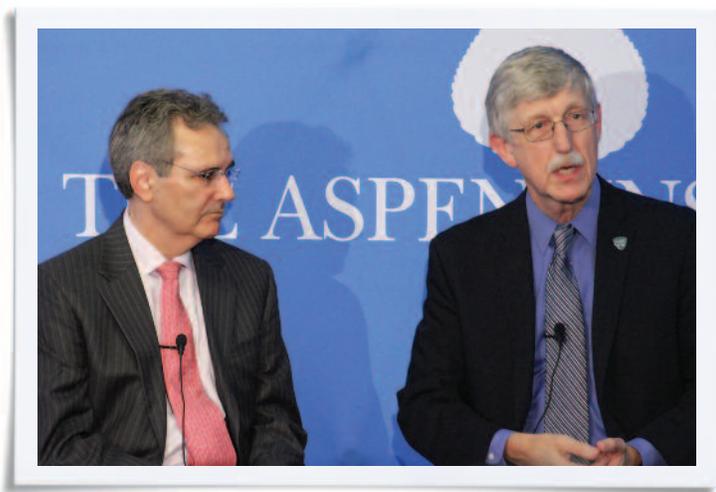
On September 18, 2014, The Aspen Institute Health, Medicine and Society Program and Friends of Cancer Research (*Friends*) announced a new partnership, which launched with a series of policy briefings on *Cancer Research in the 21st Century*. The briefings will explore the history and evolution of cancer care, highlighting the progress made — how far we have actually come in our knowledge and understanding of cancer and what the future holds. The briefings will also showcase the medical research successes achieved and preview exciting new scientific advances in cancer care. The goal of the program is to provide an understanding of the evolution of cancer treatment, including drug research and development, as well as a view toward the future in combatting the second leading cause of death in United States today.

“The treatment for cancer is the scientific quest of our age. With this partnership with *Friends*, we hope to advance the conversation on this most important of issues,” said Elliot Gerson, executive vice president of policy and public programs at the Aspen Institute.

“This partnership will bring together true leaders of innovation for a unique dialogue on the progress, opportunities and realities of biomedical research and the battle against cancer,” said **Dr. Ellen Sigal**, Chair & Founder of *Friends*. “The platform that this partnership with Aspen creates will allow this conversation to be heard by an incredible and diverse audience.”

The inaugural event, “Curing Cancer: How Close Are We?”, featured **Dr. Francis Collins**, Director of the National Institutes of Health, and **Dr. Ronald DePinho**, President of the University of Texas MD Anderson Cancer Center, and was moderated by **Susan Page**, Washington Bureau Chief at *USA Today*. The briefing took place on October 14, 2014, in the Aspen Institute’s DC office. Its subject was the ongoing fight against cancer, the progress that has been made so far, and the challenges that remained.

Dr. DePinho described the state of the field: “One of the greatest proven success stories in the history of the nation has been our investment in fundamental research and now translational research that has converted knowledge—basic insights—into things that matter for patients ... At this point, we have a very clear line of sight in making an impact on the cancer problem worldwide through prevention, through screening, and through therapeutic advances that are truly game-changing.”



(L-R) Ronald DePinho, Francis Collins

Breakthrough by the Numbers

Exceptional Early Progress with Breakthrough Therapies

Working with our partners in all sectors, *Friends* took 'Breakthrough' from concept, to scientific whitepaper, to bipartisan legislative solution, to a tool in full use by FDA to expedite the approval of multiple drugs in 13 months.

What is a Breakthrough Therapy?

A new drug may be designated as a breakthrough therapy by the Food and Drug Administration (FDA) if it is intended to treat a serious or life-threatening disease and preliminary clinical evidence suggests it provides a substantial improvement over existing therapies. Once the breakthrough therapy designation is requested by the drug sponsor and granted by the FDA, the FDA and sponsor work together to determine the most efficient path forward.

285 Applications for Breakthrough Designation

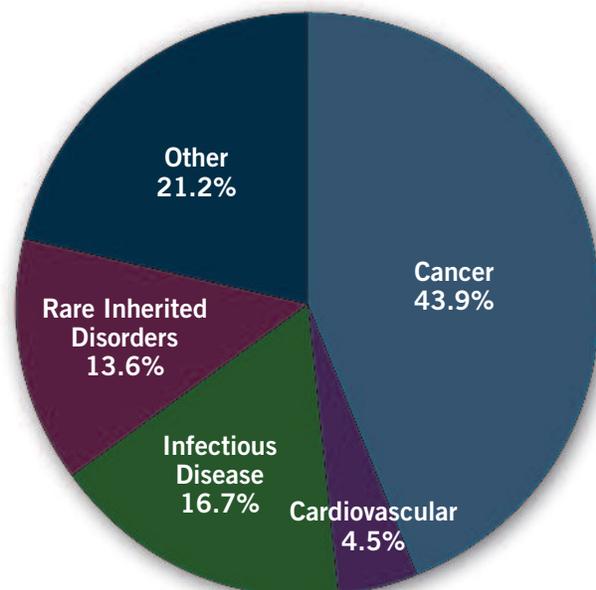
82 Designations Granted by the FDA

29 Designations for Cancer Drugs

22 FDA Approvals of Breakthrough Drugs

11 FDA Approvals of Breakthrough Cancer Drugs

Breakthrough Designations by Therapeutic Categories



Numbers are as of March, 2015
Chart represents designations that have been announced by their sponsor

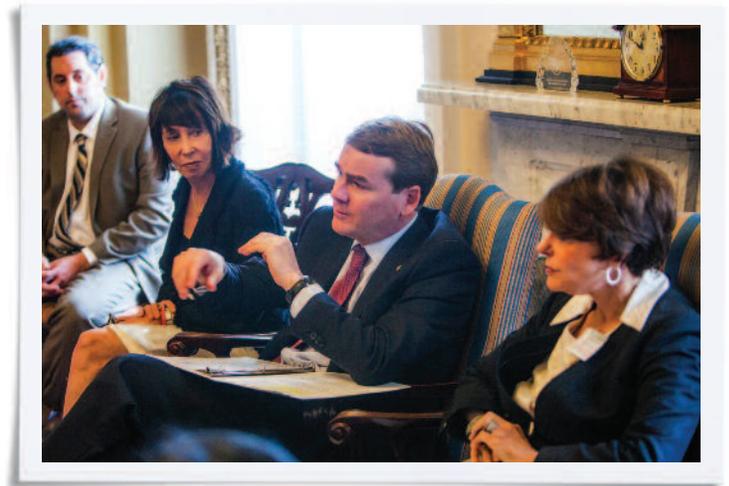
Science and Progress at the FDA: A Friends of Cancer Research Congressional Briefing on the Progress of the FDA's Breakthrough Therapies Program

On May 6, 2014, Friends of Cancer Research (*Friends*) hosted a congressional briefing entitled, "Science and Progress at the FDA: A Friends of Cancer Research Congressional Briefing on the Progress of the FDA's Breakthrough Therapy Program." The event was sponsored by Senators **Michael Bennet** (D-CO), **Orrin Hatch** (R-UT), and **Richard Burr** (R-NC), who together introduced the initial legislative proposal for this program, the "Advancing Breakthrough Therapies for Patients Act," in March of 2012.

The goal of the briefing was to discuss how the Breakthrough Therapy program has performed in the two years since it was established in July of 2012. Representatives from FDA, industry, and patient advocacy groups were invited to share their views. The panel discussion was moderated by **Kate Rawson**, Senior Editor of The RPM Report.

All the panelists agreed that the Breakthrough program has been far more popular than expected. **Dr. Janet Woodcock**, Director of FDA's Center for Drug Evaluation and Research, said the FDA has been inundated with requests for Breakthrough designation, and **Dr. Ellen Sigal**, Chair and Founder of *Friends*, added that when she was first promoting the new pathway, she thought that just two drugs a year would be granted Breakthrough designation.

The panelists were asked to share what they thought could still be improved. Dr. Woodcock noted that FDA still



(L-R) Ellen Sigal, Sen. Michael Bennet (D-CO), Marlene Malek

needed to release final guidance on the distinction between drugs that constitute incremental improvements and those that demonstrate substantial, game-changing effects. She added that manufacturing processes needed to be streamlined for Breakthrough products, since development and review times are substantially shortened. **Dr. Sandra Horning**, Chief Medical Officer at Genentech, echoed Dr. Woodcock's comments, adding that she would still like to see more clarification on how drugs and diagnostic development could best be aligned when Breakthrough status has been granted.

Dr. Urte Gayko, Senior VP of Global Regulatory Affairs at Pharmacyclics, had two suggestions for improving discussions between sponsors and FDA. First, she asked that FDA provide informal target dates for reviews to provide sponsors with a clearer idea of when they need to have manufacturing concerns resolved. Second, she said that PDUFA V now requires more meetings between sponsors and the FDA that often prove to be unnecessary for Breakthrough products, where substantial informal communication takes place.



Kate Rawson opens the panel discussion



(L-R) Chip Kennett, Ellen Sigal, Janet Woodcock, Kate Rawson, Sandra Horning, Urte Gayko



Janet Woodcock



Sandra Horning

The panelists also shared their views about the program's successes. Dr. Gayko estimated that receiving Breakthrough designation shaved an entire year off the development time for Imbruvica, which was approved for mantle cell lymphoma in late 2013. Dr. Horning said the greatest success of the program was the change in mindset it brought about within the agency, referring to a spirit of collaboration that now exists between sponsors and the FDA in the approval of Breakthrough products.

During Q&A, audience members asked about the high cost of many Breakthrough products. Both Dr. Sigal and Dr. Woodcock agreed that the price tags are high, but that the therapies are worth it. Dr. Sigal noted that some Breakthrough products are curative, which changes the entire treatment landscape. Dr. Woodcock added that the research community is driving towards cures, that enormous progress has been made, and that we need to continue to push for more innovation. The high prices of recent Breakthrough products, in her view, represent a down-payment that will produce long-term cost savings.

HIGHLIGHTS:

Sandra Horning on FDA-industry collaboration:

"FDA and industry march to the beat of the same drummer during the development and review of Breakthrough products."

Urte Gayko on Breakthrough time savings:

"Receiving Breakthrough Designation allowed Imbruvica to be approved a year earlier than was expected."

Janet Woodcock on the promise of Breakthrough:

*"Getting drugs to patients who need them is why many people choose to work at the FDA."
"We are driving towards cures."*

Chip Kennett on being treated with a Breakthrough product:

"One of the biggest benefits of receiving a Breakthrough drug was improved quality of life, time with my wife and children"

Ellen Sigal on supporting the FDA:

"Breakthrough has changed the culture at FDA. They need more resources to implement all of these programs that are vital to patients, it's a big issue, and it needs to be resolved before PDUFA VI."

7th Annual Friends of Cancer Research-Brookings Institution Conference on Clinical Cancer Research

On November 21, 2014, the seventh-annual Conference on Clinical Cancer Research was convened by Friends of Cancer Research and the Engelberg Center for Healthcare Reform at the Brookings Institution, with the support of Susan G. Komen and the American Society of Clinical Oncology (ASCO).

Each year, this conference brings together a diverse group of experts in cancer drug development from academic and clinical research centers, federal health and regulatory agencies, patient advocacy organizations, and the private sector to develop practical, consensus-driven solutions to critical challenges in the development of drugs for cancer.

The conference emphasizes outcomes and many of its panels have led to material improvements to cancer care, including: the 2012 passage of the Advancing Breakthrough Therapies for Patients Act, which created a pathway to expedite the development of exceptional new drugs for patients in need; the development of Lung-MAP, an innovative multi-arm, biomarker-driven clinical trial that uses a targeted screening method to match patients with investigational new treatments; and the creation of FDA Guidances such as the 2010 Guidance describing how to test novel drugs in combination.

Dr. Ellen V. Sigal, Chairperson and Founder of *Friends*, welcomed the attendees and introduced the morning keynote speaker, **Congressman Fred Upton** (R-MI), Chairman of the House Energy and Commerce Committee. Rep. Upton, together with **Congresswoman Diana DeGette** (D-CO), has spearheaded the 21st Century Cures Initiative, a bipartisan effort which aims to accelerate the development and approval of new drugs and devices. "This initiative will profoundly change the way we approach innovation and do business in drug development," said Dr. Sigal in her introduction.

Rep. Upton stated that we only have cures for approximately 500 of the 7,000 known diseases. He explained that the current economic and regulatory climate in the United States has driven more than half of venture capitalists overseas. "If we want to save more lives and keep the United States the leader in medical innovation," he said, "we have to make sure that there is not a major gap between the science of cures and the way we regulate those therapies."

To that end, the 21st Century Cures Initiative is working with and seeking ideas from all stakeholders on how to accomplish the following 5 goals: 1) keep patients at the center of the decision-making process, 2) modernize clinical trials, 3) foster 21st century digital medicine by facilitating data-sharing and the use of medical apps, 4) encourage young scientists to enter medical research, and 5) incentivize the development of drugs and devices for unmet medical need. The initiative has held 4-5



Ellen Sigal

roundtables in Washington DC, dozens of roundtables across the United States, and 8 committee hearings.

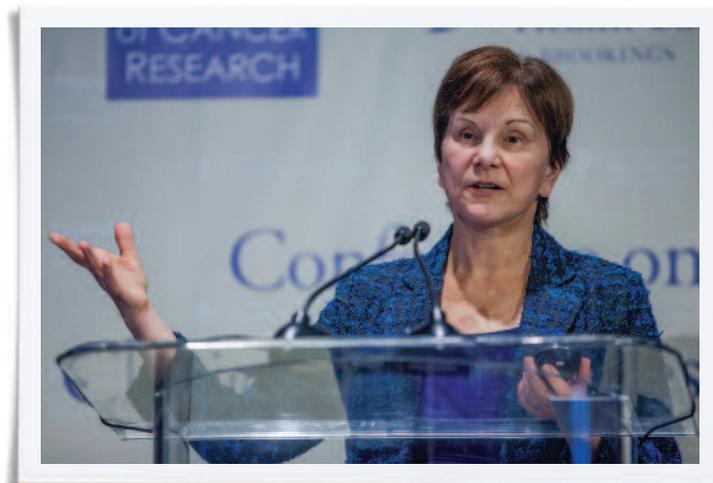
As of this writing, a discussion draft has been released based on ideas that have been generated through this process. Congressman Upton announced that Friends of Cancer Research will be holding a roundtable for advocacy organizations to provide feedback on this discussion draft in late January. Once this feedback has been incorporated, the initiative aims to introduce a bill and move it through the Energy and Commerce Committee in March and to have it on the floor by Memorial Day. Said Congressman Upton, "Together we can achieve the common goal of accelerating the pace of cures."

Dr. Janet Woodcock, Director of the Center for Drug Evaluation and Research at the FDA, delivered a keynote address on the state of the Breakthrough Therapy designation. The FDA has received far more requests and granted more designations than had been thought possible when the program was originally conceived. To date, CDER and CBER have received 243 requests for Breakthrough designation and 69 designations have been granted. According to Dr. Woodcock, there are many drugs currently on the market that would not yet be available in the absence of the Breakthrough Therapy program.

Dr. Woodcock stressed that the success of the program is not due to lower standards for the approval process—standards have remained rigid and appropriate for each specific disease setting—and that Breakthrough designation does not directly translate into drug approval. Instead, the program allows for increased attention and consultation between developers and regulators. For instance, owing to efforts within the newly formed Office of Pharmaceutical Quality, some Breakthrough drugs that would have been delayed getting to market due to the compressed manufacturing timeline were made accessible earlier, "drugs that were really needed by patients."



Rep. Fred Upton (R-MI)



Janet Woodcock

Dr. Woodcock noted that the robustness of the program has led the FDA to consider streamlining the workload as the Medical Policy Council continues to evaluate the Breakthrough program. They are also working on ways to make the process more standardized and to educate industry partners as to how submissions can be improved. FDA recognizes that "Not all of these are going to pan out, but for people with the most need, people with serious and life-threatening diseases, there are actions we can take to make a difference."

PANEL ONE

The Role of Non-Randomized Trials for the Evaluation of Oncology Drugs

Deborah Armstrong, Professor of Oncology, Johns Hopkins Kimmel Cancer Center

Gideon Blumenthal, Clinical Team Leader, FDA

Mace Rothenberg, Senior Vice President, Clinical Development & Medical Affairs & Chief Medical Officer, Pfizer, Inc.

Richard Simon, Chief, Biometric Research Branch, National Cancer Institute

Josh Sommer, Executive Director, Chordoma Foundation

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

The first panel discussed how single-arm trials might be used to support full approval of "transformational" oncology drugs. Although randomized trials provide the most reliable evidence of a drug's safety and efficacy, there are situations where randomized trials are not possible or ethical. **Dr. Richard Pazdur** explained that drugs now exist that demonstrate unprecedented response rates and duration. The question the office must often consider is not whether a drug should be approved, but rather how quickly can it be approved. Trials should be designed in such a way that patients want to enroll. He emphasized that there should always be equipoise—if there is not, randomization does not make sense. **Dr. Mace Rothenberg** described criteria for single-arm trials to support full approval: 1) an unprecedented effect on overall response rate (ORR) is observed in a setting of high unmet medical need; 2) clinical trial patients have been well characterized, enabling a target population to be clearly defined; 3) experience exists in a sufficient number of patients to allow adequate assessment of the risk-benefit relationship; and 4) a proper historical context can be provided for analysis. **Dr. Richard Simon** emphasized that prospective selection of historical controls as well as a statistical analysis plan are needed, and the panel called for an FDA Guidance or best practices document to provide guidelines to facilitate the use of rigorous single-arm trials. **Josh Sommer** added that for some rare or molecularly defined cancers, historical controls may not exist but patient organizations are well suited to develop these.

The panel also discussed the potential use of response rate as an endpoint sufficient to support full approval.



Richard Pazdur



Panel Two

Dr. Deborah Armstrong discussed the history of response rate as an endpoint for clinical trials and as a tool for managing patient care. **Dr. Gideon Blumenthal** described how response rates might be considered predictive of long-term outcomes or, in certain contexts, clinically meaningful in and of themselves. Josh Sommer added to this, saying that for many patients, "Survival is not the only goal that matters. They often would rather have more good years than years in total. We cannot let perfect be the enemy of the good." Dr. Pazdur said, "We have been demeaning response rate as an endpoint. It is meaningful."

In the open discussion, **Dr. Renzo Canetta** of Bristol-Myers Squibb pointed out that the response rate



Panel One

endpoint discussed by the panel may only be appropriate for some therapies. He argued that we must develop ways to evaluate quality of life as well as other endpoints, such as milestone survival, that capture the effects of emerging therapeutic modalities such as immunotherapies. **Mark Gorman**, a cancer survivor and patient advocate, asked about the impact of the regulatory and commercial landscape, which favors first-in-class drugs, on the development of second-generation drugs that may be superior to their predecessors. Dr. Blumenthal brought up the example of ALK-inhibitors and how second-generation drugs should now be tested against crizotinib or in crizotinib-refractory patients. Dr. Pazdur added that this is not a static process and that the appropriateness of a single-arm trial also depends on the therapies that are available. If more treatments that are effective are available, then randomized trials are needed.

PANEL TWO
Improving Evidence Developed from Population-Level Experience with Targeted Agents

Mark McClellan, Director, Health Care Innovation and Value Initiative and Senior Fellow, Economic Studies, The Brookings Institution
Richard L. Schilsky, Chief Medical Officer, American Society of Clinical Oncology

Dane Dickson, Director of Clinical Science, MolDX, Palmetto GBA
Samuel Nussbaum, Executive Vice President, Clinical Health Policy and Chief Medical Officer, WellPoint, Inc.
Dietmar Berger, Senior Vice President and Global Head - Clinical Hematology/Oncology, Genentech
Jane Perlmutter, Founder and President, Gemini Group
Vincent Miller, Chief Medical Officer, Foundation Medicine, Inc.
Jeffrey Roche, Medical Officer, Coverage and Analysis Group, Centers for Medicare & Medicaid Services
Richard Pazdur, Director, Office of Hematology and Oncology Products, FDA

Dr. Mark McClellan introduced the second panel, which proposed and discussed frameworks that aim to provide access to drugs while generating evidence of their safety and efficacy in prospective registries. Because it is not feasible to test every drug in every disease setting, off-label use is common in oncology and offers patients a chance to receive a potentially effective therapy that they otherwise would not have access to. However, data providing scientific support for an off-label use may be limited, and the current approach to off-label prescribing does not capture patient outcomes. **Dr. Richard Schilsky** described the TAPUR (Targeted Agent and Profiling Utilization Registry) study being spearheaded by ASCO.

This program, which will be conducted under an IRB-approved protocol and will be launched in mid-2015, aims to create a national facilitated access program and registry of the outcomes of cancer patients receiving off-label treatment with targeted therapies. **Dr. Dane Dickson** described a similar program being developed by Palmetto GBA and MoIDX called MED-C (Molecular Evidence Development Consortium). In this program, patients with a particular malignancy such as non-small cell lung cancer would first receive standardized molecular testing and would then be assigned to a particular "pathway" based on their mutation status. When possible, patients with actionable mutations would be directed to receive an appropriate targeted therapy that has been approved for their disease. If no such therapy has been approved, patients would be directed to either a clinical trial testing a targeted therapy or to receive the agent off-label with data capture.

Dr. Vince Miller praised these efforts and said, "We might already have answers to many of our questions if we had some kind of mechanism to collect outcomes." **Dr. Jane Perlmutter** emphasized the need to learn from patient treatment outside of clinical trials as many patients may be ineligible or otherwise be unable to enroll in clinical trials. **Dr. Jeff Roche** added that cancer is a big burden and someday treatment with targeted therapies should be seen as reasonable and necessary for everyone. **Dr. Richard Pazdur** closed the panel presentation by emphasizing the need for flexibility in the amount and type of data. We are going to have smaller numbers of patients and we have to be realistic that the world of drug development is changing. He said that a regulatory decision requires a uniform, defined patient population, emphasizing the importance of standardized diagnostics to identify patients likely to respond to a specific therapy. He then asked the audience to ponder what the world of oncology would look like today if we had stuck to the 1980s and early 1990s requirements of two randomized controlled trials assessing overall survival. "You probably wouldn't like it," he concluded.

PANEL THREE
Considerations for Summary Review of Supplemental NDA/BLA Submissions in Oncology

Rachel Sherman, Principal, Drug and Biological Drug Products, Greenleaf Health LLC



Vince Miller

- Paul Kluetz**, Acting Deputy Director, Office of Hematology and Oncology Products, FDA
- Tatiana Prowell**, Breast Cancer Scientific Lead, Office of Hematology and Oncology Products, FDA
- Katherine Sugarman**, Senior Director, Global Regulatory Affairs, Oncology, Eli Lilly
- Laurie Strawn**, Senior Director, Worldwide Safety and Regulatory Strategy, Pfizer Inc.
- Kannan Natarajan**, Senior Vice President and Global Head, Oncology Biometrics and Data Management, Novartis
- Raji Sridhara**, Director, Division of Biometrics V, FDA
- Robyn Lim**, Senior Science Advisor, Health Canada
- Andrew Thomson**, Biostatistician, Specialised Scientific Disciplines Department, European Medicines Agency

The final panel discussed the potential use of "summary review" to analyze applications for supplemental indications. **Dr. Paul Kluetz** explained that the oncology office receives many efficacy supplements for new indications. Regardless of the quantity of existing clinical and post-marketing data, clinical reviewers often spend time verifying analyses submitted in the clinical summary from raw and derived datasets. The time spent analyzing primary datasets for efficacy supplements with well-known safety and efficacy in other settings could be better spent. **Dr. Rachel Sherman** added that resources are limited and innovative programs, such as those discussed in the earlier panels, will be resource-intensive, so it is prudent to identify ways to increase efficiency. The FDA, therefore, is proposing to review



Panel Three

clinical study reports rather than primary datasets for carefully selected efficacy supplements. This is similar to the approach used by other regulatory agencies. **Dr. Robyn Lim** explained that Health Canada takes a "top-down" approach in which summaries are evaluated initially, but reviewers may analyze the raw data as needed.

Dr. Tatiana Powell described the initial FDA experience of using a "summary review" approach for the supplemental approval of Avastin to treat cervical cancer. Avastin has a track record of efficacy in a number of settings as well as a significant but well-characterized toxicity profile. It had clear efficacy in the setting of cervical cancer and "absent fraud or a huge error, this was a clear-cut decision" for reviewers, she explained. The application was reviewed without re-analyzing the raw data, allowing the FDA to take regulatory action in only three and a half months. As discussed by **Dr. Katherine Sugarman**, the panel evaluated the US labels of Afinitor, Sutent, Gemzar, and Alimta to determine how safety labeling changed over time as new indications were added. These evaluations found that review of full datasets for each new indication resulted in no significant change to the existing safety profiles described in US labels. In fact, most changes to adverse event labeling over time were attributable to post-

marketing surveillance rather than clinical trials. **Dr. John Jenkins** of the FDA asked the panel about the system of checks and balances, and whether stakeholders could ensure the integrity of the regulatory process. Dr. Kluetz reminded the audience that this program is intended for only very select supplemental applications and added that it would include data auditing and would retain the ability to access raw data as needed. A pilot program would determine exactly what is submitted to and reviewed by the FDA.



Richard Schilsky

A Blueprint for Drug/Diagnostic Co-development: Next-Generation Sequencing (NGS) Forum Co-Hosted with Alexandria Real Estate Equities, Inc.

In the third installment of the *Blueprint for Drug/Diagnostic Co-development*, Friends of Cancer Research and Alexandria Real Estate Equities, Inc. brought together researchers, sponsors, advocates, and regulators to discuss possible approaches to expedite development of Next Generation Sequencing (NGS) as a companion diagnostic. Leading up to the event, a large collaborative group put together a report that proposes mechanisms to optimize NGS development and implementation.

In oncology, sequencing is on the cusp of transforming from a research tool into a clinical diagnostic tool. The technology has evolved to become affordable, accessible, and routine in many cancer centers. **Karen Gutekunst** (Illumina) explained, "Because of the ability to deeply interrogate a patient's tumor using multiple markers at the same time from a small piece of tissue, it's become a critical tool" that allows us to gain a better understanding of each patient. Between the complexity of cancer itself and the tools being used, many challenges still exist.

The first panel, moderated by **Mya Thomae** (Myraqa/Illumina), addressed what regulatory pathway would best support the clinical paradigm of assessing multiple known actionable and emerging markers as we shift away from the single companion diagnostic linked to a single drug model. **Roman Yelensky** (Foundation Medicine) pointed out that such a path would need flexibility to account for the rapidly changing science and drug development pipeline and stratify based on risk. While companion diagnostics will remain part of the Pre-Market Approval (PMA) pathway, **Elizabeth Mansfield** (FDA) inquired whether "special controls" could be put in place to allow certain markers to avoid the PMA pathway in the absence of sufficient data. **Omar Perez** (Pfizer) described one method of introducing controls by categorizing markers based on available evidence.

In the "wild west" of sequencing technologies, **Mickey Williams** (NCI) pointed out that while "DNA is DNA," standardization and validation of the technology platform and reference material are key. Some areas of the



Panel One

genome are easy to sequence, others are difficult, and while it would be impossible to validate every genomic location, focusing on the most common and the most difficult areas to sequence may be a better approach. It would be invaluable for the community to harmonize and establish minimal reportable information for sequencing to allow standardization and reproducibility of results.

Eli Ketchum (Roche Sequencing) noted that researchers need to identify the appropriate reference method and material to "find sequencing truth" and ensure reproducibility of NGS results. A few efforts are already underway; NIST, Genome in a Bottle, has begun developing standardized reference material. Ultimately, patients should know the quality of evidence they are receiving without being confined to testing in a single lab. Several items remain outstanding: a study to determine which extraction methods could be applied across tumor types; a study to define sensitivity through determination of sample fraction of neoplastic versus normal tissue; a well curated database to allow for information sharing and learning because "eventually there will be enough evidence" that we "can use everybody's data to float everybody's boat," said Elizabeth Mansfield.

The second panel, moderated by **Ramsey Baghdadi** (RPM Report), discussed current reimbursement challenges and NGS use in the healthcare setting. **Keith Flaherty** (Massachusetts General Hospital Cancer Center) said that "accountable care in cancer" requires standardization to pre-identify patients in the community who may benefit from referral to academic centers with large portfolios of clinical trials. In the academic centers, "we see a gradient of opportunity for patients with common actionable genetic alterations versus those whose tests reveal rare events," he noted. "Whose problem is it" Flaherty asked, to validate mutations for rare subgroups of patients: the sponsor, the academic, or the public sector? Professional societies, setting guidelines for best practices, could begin to address some of these outstanding issues.

One mechanism, described by **Janet Woodcock** (FDA), is to promote the use of "standing clinical trial networks that have standardized procedures and processes," including the use of standardized platforms, instead of the current ad-hoc approach, to unfold the complexity of the disease. Lung-MAP is a step in this direction. Additionally, "The data management issues are systemic challenges not just for industry but the entire community" and standards need to be set high enough to ensure reproducibility so as not to waste dollars or patient resources. "What we [industry] want is a diagnostic that can identify patients for the right treatment. What we don't want is the wrong answer," said **Steven Averbuch** (Bristol-Myers Squibb). Through increased "transparency, collaboration and data availability, we'll ultimately get there."

Jeff Shuren (FDA) pointed out the need for greater linkage between the regulatory and reimbursement decisions. "There isn't true patient access until there's active reimbursement," he noted. Unfortunately, "today, we don't know what we're paying for," said **Bryan Loy** (Humana); there is little knowledge about the quality of the test or the intent of use that can be obtained from claims data. Furthermore, payers have limited ability to assess the quality of the informed consent and



(L-R) Bryan Loy, Jeff Shuren, Janet Woodcock



Panel Two

communicate information to ensure patients are informed about their risk. Test-by-test CPT codes, used by insurers to identify diagnostics, to understand the nature of the mutation, the intent of use, or establish eligibility for a clinical trial, would be immensely helpful. NGS technology may lead to long-term cost savings by scaling up from the individual tests but may lead to undesirable clinical consequences.

The forum was a space for collaboration, open communication, and willingness to work together to find the right solutions. The panelists agreed that efforts in this area cannot neglect the patient perspective; improving health literacy should take place ahead of any test. As Jeff Shuren noted, patient preference and tolerance of benefit-risk tradeoffs is an important aspect of device development at FDA.

Friends of Cancer Research Hosts 18th Annual Cancer Leadership Awards Reception Honoring Dr. Margaret Foti, Governor Deval Patrick & Governor Bobby Jindal

On September 23, 2014, **Friends of Cancer Research** (*Friends*) welcomed leaders from government, advocacy, industry, and science to its **18th Annual Cancer Leadership Awards Reception** to celebrate another year of partnership and innovation on the 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, Governor Martin O'Malley, and Congressman John Dingell.

This year, *Friends* honored **Dr. Margaret Foti**, CEO of the American Association for Cancer Research (AACR), with the **Ellen V. Sigal Advocacy Leadership Award** in recognition of her unparalleled advocacy and leadership on behalf of patients, scientists and researchers. Said Ellen Sigal, "Marge is extraordinary. She has very clear goals that are actionable and there's nothing she won't do to help patients."

Governor Deval Patrick of Massachusetts and **Governor Bobby Jindal** of Louisiana received Friends of Cancer Research Leadership Awards for their extensive support of public and private medical research. Governor Patrick

helped to develop the Massachusetts Life Sciences Act, a ten-year, \$1 billion initiative to fund bioresearch and create jobs. Governor Jindal has overseen substantial investment in Louisiana's medical infrastructure, including millions of dollars to LSU Health Sciences Center.

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 do for patients everywhere.



(L-R) Ellen Sigal, Margaret Foti, Marlene Malek



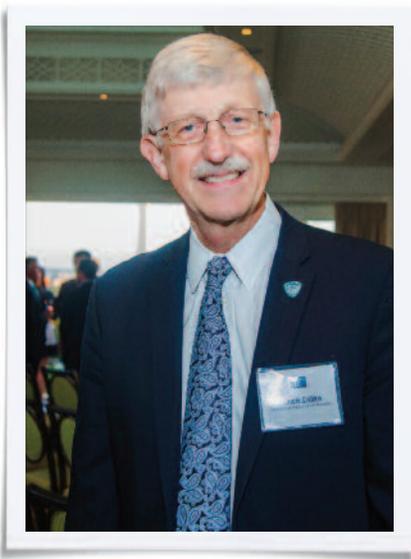
Gov. Bobby Jindal



Gov. Deval Patrick



Marlene Malek and Ellen Sigal congratulate the honorees



Francis Collins



(L-R) Margaret Hamburg, Margaret Foti, Ellen Sigal, Gov. Patrick, Edward Benz

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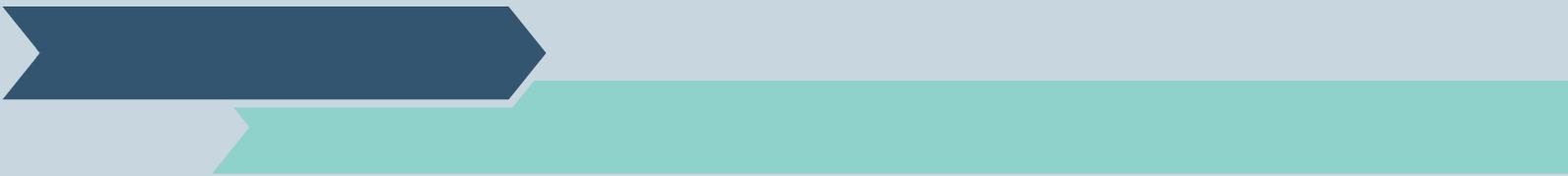
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Presentations of Note

Friends of Cancer Research leadership is engaged in a significant number of conferences, forums, committees and boards of organizations across the country. In 2014, *Friends* played key roles as members of important panels at over 50 events. Below are a few presentations of note.

Jeff Allen Presents at Biotechnology Industry Organization (BIO) International Convention in San Diego

On June 24 and 25, 2014, Jeff Allen joined international leaders from advocacy and industry at the BIO Convention, where he presented on two expert panels. Topics included the dramatic scientific innovation taking place in oncology and expedited drug development under FDA.

A New Paradigm in Oncology Treatment

Jeff Allen, Executive Director, Friends of Cancer Research

Ronald A. DePinho, President, The University of Texas MD Anderson Cancer Center

Robert J. Hugin, Chairman and Chief Executive Officer, Celgene Corporation

Bahija Jallal, Executive Vice President, AstraZeneca, Head, MedImmune

Peter Lebowitz, Global Therapeutic Area Head of Oncology, Janssen

Moderator: Susan Schaeffer, Editor, BioCentury, The Bernstein Report on BioBusiness

Chasing Breakthroughs: Expediting Drug Development for Unmet Medical Need

Jeff Allen, Executive Director, Friends of Cancer Research

Todd Gillenwater, President & CEO, California Healthcare Institute

Jonathan Leff, Partner, Deerfield Management

Jay Siegel, Chief Biotechnology Officer and Head, Johnson & Johnson

Moderator: Andrew Emmett, Managing Director, Science & Regulatory Affairs, BIO



(L-R) Susan Schaffer, Ronald DePinho, Peter Lebowitz, Bahija Jallal, Robert Hugin, Jeff Allen (far right)

Ryan Hohman Presents at Royal College of Physicians Vital Transformations Conference in London

On October 21st, 2014, Ryan Hohman joined an international group of experts at the Royal College of Physicians in London to discuss how new clinical regulatory pathways, sources of evidence, and evolving data on real world patients can be used both to expedite the development of drugs and improve their quality and access.



Blazing Trails – New Development Pathways in Action

Sandrine Marreaud, Head of Medical and Pharmacovigilance Department, EORTC
Ryan Hohman, Managing Director, Friends of Cancer Research
Laura Esserman, Breast Cancer Center Director (I-Spy), University of California, San Francisco
James Anderson, Director of EU Industry & External Partnerships, GSK
Moderator: Edward Abrahams, President, Personalized Medicine Coalition

Ellen Sigal Presents at AACR and Stand Up To Cancer Annuals Meetings

On January 28, 2014 during the Stand Up To Cancer (SU2C) Annual Meeting in Pasadena and on April 7, 2014 at the American Association for Cancer Research (AACR) annual meeting, Ellen Sigal presented on how the Breakthrough Therapies designation and the Lung-MAP clinical trial model are changing the landscape of drug development.

SU2C Annual Meeting: Managing and Overcoming Roadblocks in Drug Development and Approval

Margaret A. Hamburg, Commissioner, U.S. Food and Drug Administration
Charles Sawyers, Chair, Human Oncology and Pathogenesis, Memorial Sloan Kettering
Richard Gaynor, Vice President, Oncology, Product Development, Eli Lilly and Company
William Hait, Global Head, Janssen
Ellen Sigal, Chair & Founder, Friends of Cancer Research
Suzanne Topalian, Director, Melanoma Program, Sidney Kimmel Comprehensive Cancer Center

AACR Annual Meeting: Breakthrough Therapies: Case Studies of Successful Applications in Oncology

William Hait, Global Head, Janssen
Paul Kluetz, Acting Deputy Director, Office of Hematology and Oncology Products, CDER/FDA
Peter Lebowitz, Global Therapeutic Area Head of Oncology, Janssen
Ellen Sigal, Chair & Founder, Friends of Cancer Research
Nancy Valente, Vice President, Global Hematology Development, Genentech



Ellen Sigal

Thank you to all of Friends of Cancer Research staff and collaborators who contributed to our work in 2014 and 2015.

A special thanks to:

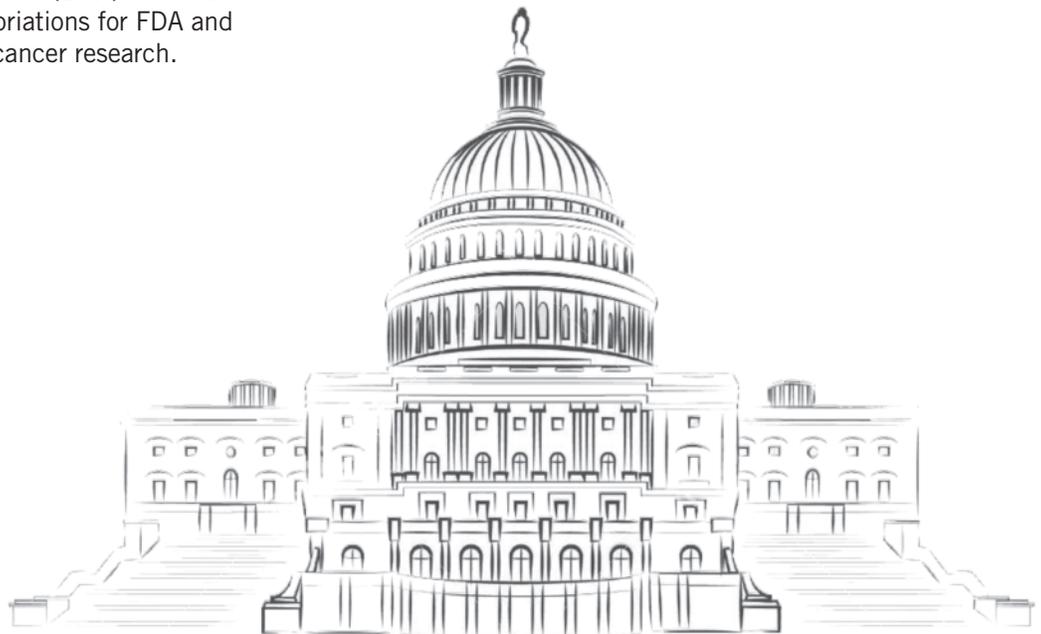
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Friends Co-hosts Annual Congressional Advocacy Day on Capitol Hill

On May 8th, 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 advocate for congressional support for cancer research and biomedical science.

Over 75 researchers, clinicians and advocates met with 109 House and Senate lawmakers and aides, including House and Senate leadership, from 20 states. Advocates called on lawmakers to approve a fiscal year 2015 budget that will fund the FDA and NIH at a level that stakeholders say is necessary to sustain scientific momentum.

At an evening reception on May 7th, the organizations recognized Representative Charlie Dent (R-PA) and Senator Tom Harkin (D-IA) for their tireless work to enhance appropriations for FDA and NIH and their commitment to cancer research.





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 innovating 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 of Cancer Research 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, 19 years later, Friends of Cancer Research 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.



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