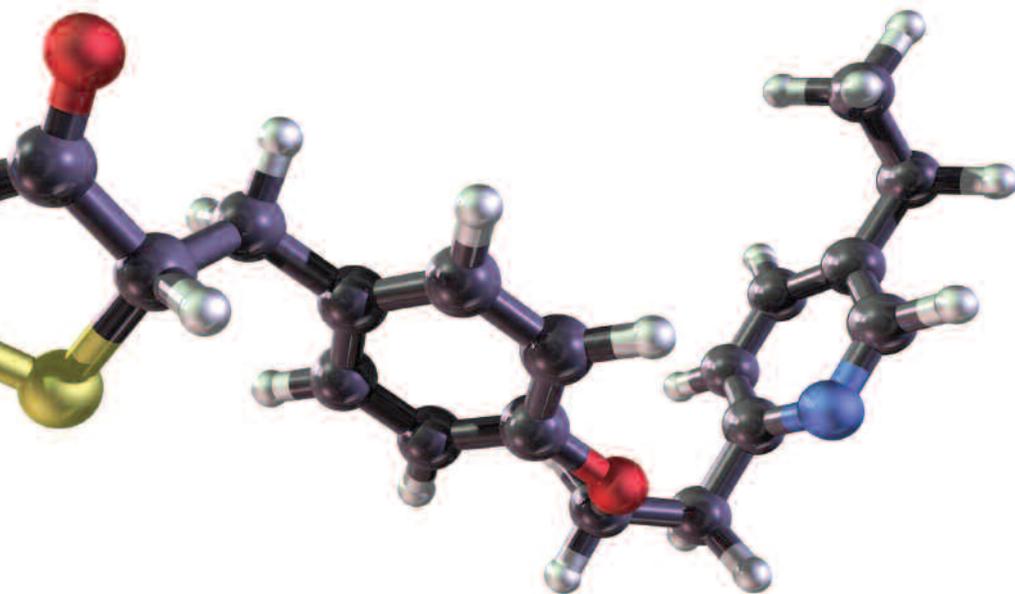


FRIENDS OF CANCER RESEARCH
Progress in Science & Innovation

ANNUAL REPORT 2010





- 79% of cancer research grants for promising new discoveries go unfunded each year.
- It takes more than 12 years for a newly discovered cancer treatment to get from the research bench to the patient.

We have made great strides in the fight against cancer. But imagine the discoveries that could be made—and the lives that could be saved—if those grants were funded, and if the scientific, regulatory, and clinical barriers between promising discovery and an approved treatment were overcome.

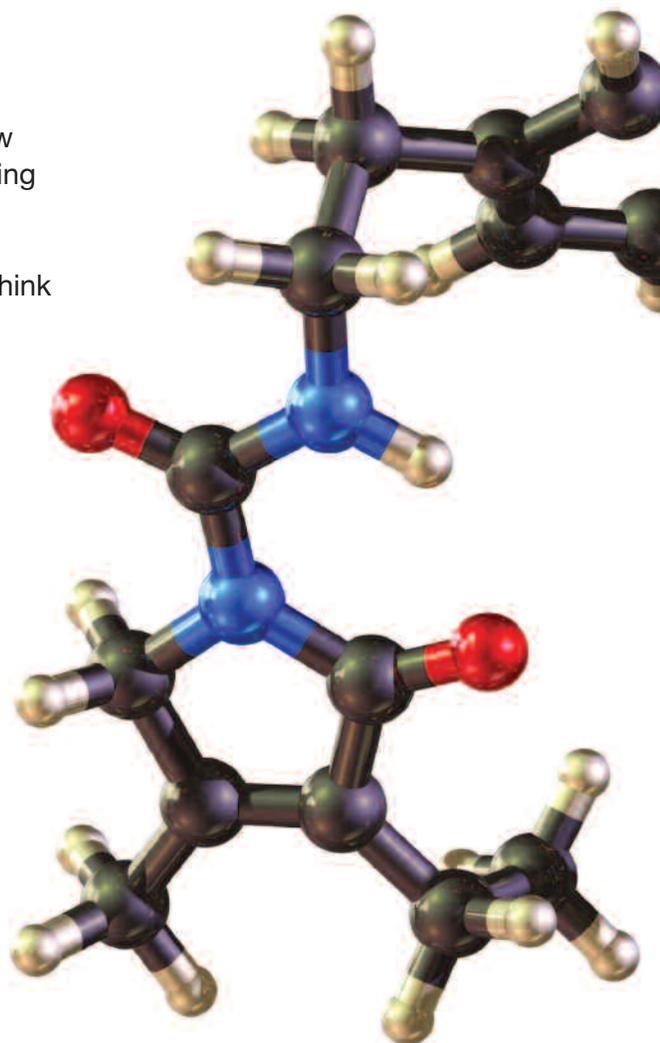
YOU CAN BE PART OF THE SOLUTION.

Join **Friends of Cancer Research** as we strive to bring new research, treatment, and hope to patients and families battling cancer.

Friends of Cancer Research (*Friends*) is a cancer research think tank based in Washington, D.C.

Friends is a leader in advocating for policies and funding that will get treatments and therapies to patients in the safest and quickest way possible as well as developing unique partnerships among the public and private sectors aimed at creating a more open dialogue.

- During our 15-year history, *Friends* has progressively increased our scope of work to create educational, policy, and scientific approaches to improve health outcomes and cancer care.
- *Friends* works with government agencies (Food & Drug Administration, National Institutes of Health, National Cancer Institute, Health and Human Services), Congressional leadership, academic research centers and private sector industry, producing real results.



Celebrating 15 Years

Friends of Cancer Research (Friends) is a non-profit cancer research think tank and advocacy organization dedicated to accelerating the nation's progress toward prevention and treatment of cancer.

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 Senators and Congressmen and women, but create new champions for biomedical research.

Now, 15 years later, Friends of Cancer Research is a leader in advocating for policies and funding that will get treatments and therapies to patients in the safest and quickest way possible. 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.



Top photo: Marlene Malek, Ellen Sigal, Sen. Kennedy, Sherry Lansing
Bottom photo: FDA Commissioner Hamburg



Top left photo: Marlene Malek, Sen. Specter, Ellen Sigal

Top right photo: Advocates from across the country, including; Ellen Stovall, Olivia Newton-John, Ellen Sigal, Sally Field, Sherry Lansing and Jack Klugman

Middle left photo: Sen. Gregg and Marlene Malek

Middle right photo: Donald Miller, Fmr. FDA Commissioner Von Eschenbach

Bottom left photo: Rep. Myrick

Bottom right photo: Minority Leader Pelosi, Marlene Malek, Sherry Lansing, Ellen Sigal



Top left photo: Fmr. NIH Director Zerhouni
Top right photo: Rep. Pallone and Ellen Sigal
Middle left photo: Reese Witherspoon (at podium) and Ellen Sigal
Middle center photo: Sen. Hatch
Middle right photo: Fran Drescher and Ellen Sigal
Bottom left photo: Sen. Kennedy
Bottom right photo: Ellen Sigal, Sen. Collins, Rep. Dingell, Sen. Feinstein, Marlene Malek, Dr. Von Eschenbach



Top left photo: Rep. Hoyer

Top center photo: Sen. Feinstein

Top right photo: Ellen Sigal and Vice President Al Gore

Middle left photo: Rep. DeLauro, Dr. Von Eschenbach, Edward Chee

Middle right photo: Sen. Stevens, Sen. Mikulski, Dr. Von Eschenbach, Ellen Sigal, Marlene Malek, Dr. Zerhouni, Sen. Cochran, Sen. Kennedy

Bottom left photo: Ellen Sigal

Bottom center photo: Fmr. Majority Leader Gephardt, FDA Commissioner Hamburg, NIH Director Collins

Bottom right photo: Rep. Dingell

Hill Briefing – Science and Progress at the FDA: Realizing the Return on Investment from Biomedical Research

Speakers at a Congressional briefing on March 24, 2010, said that in order to realize the full return on investment from biomedical research, the U.S. Food and Drug Administration (FDA) needs to be in a position to take the baton of innovation from the research community and turn it into medical products that are safe, effective, and accessible for patients who need it most. The briefing, *Science and Progress at the FDA*, was convened by Friends of Cancer Research and FasterCures/The Center for Accelerating Medical Solutions.

In her opening remarks, Rep. Rosa L. DeLauro (D-CT) said that the FDA must work in new ways to enhance science at the agency, recruit more top minds to formulate regulatory regimens to promote safety without stifling innovation, and adapt a multidisciplinary approach. She noted that for the FDA to be the thriving center of scientific knowledge, it requires more than resources alone; its culture must change from within.

Jesse L. Goodman, MD, MPH, Chief Scientist and Deputy Commissioner for Science and Public Health (Acting) at the FDA defined the premise of the FDA's focus on regulatory science. He said that the science done by academia - often supported by NIH - gives us tremendous insight into basic mechanisms of disease while the work done by the bio-pharmaceutical industry focuses on getting products on the market. Regulatory science is applied science that aims to develop, assess and provide new, validated tools and approaches to better evaluate the utility of new medical products.

"At our very best, FDA can join with partners to develop tools that make the evaluation process sounder," Goodman said. "The model of going it alone doesn't work. The world of science and amount of information out there is just too huge. What we need to do is collaborate and engage with the scientific community."

Echoing DeLauro's point, he said that some of it is about resources, but some of it is about doing things in a more innovative way. He noted the need to reduce risk while maintaining patient benefit.

"Along with medical and scientific risks, we need to consider financial risk as well," said Max Wallace, CEO of Accelerated Brain Cancer Cure. Wallace, who has also founded a number of biotechnology companies, said that capital is the rocket fuel that propels the process from discovery to product.

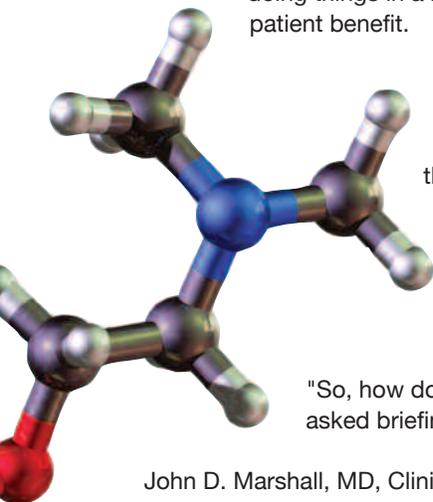
"Capital is rocket fuel without soul - it hates risk. If risk is doubled by time delays or failure to understand science or opaque non-collaborative environment, the capital will seek its level in other places," he noted.

"So, how do these discussions about regulatory science translate into patient benefit?" asked briefing moderator Margaret Anderson, Executive Director of FasterCures.

John D. Marshall, MD, Clinical Director of Oncology for Georgetown-Lombardi Comprehensive Cancer Center, and principal investigator of over 150 clinical trials, provided real-world context. He said that the



John Marshall, Margaret Anderson, Ellen Sigal, Jesse Goodman, Max Wallace





Rep. DeLauro delivers keynote at Hill briefing

FDA sets the goal, the rules, the parameters that we are to aim for in clinical research: safety and efficacy.

Marshall said that by having the scientific capacity to determine the best models to measure safety and work and collaborate with researchers to design appropriate trials, the FDA can make the process more effective, and efficient. He noted that having the ability to tailor trials to a subset of patients that are known to respond to specific medical products paves the way to a better development system and realizes the promise of personalized medicine.

Ellen Sigal, PhD, Chair and Founder of Friends of Cancer Research, said that patients have different risks and profiles. But consistent among all patients is the need for more information. Sigal said that "we can evaluate risk with the right data. We want to be able to make choices based on the data."

Marshall later noted the importance of "building a system that allows buy-in from the patients."

Regulatory science is the backbone that supports all other FDA activities. Improvements in regulatory science will support better assessment of drug and device safety, and create efficiencies in the development process.

Additional Presentations of Note:

Dr. Jeff Allen

Opportunities for the Advancement of Regulatory Science. Institutes of Medicine Drug Forum, April 29, 2010

Perspectives on Drug Information: Advocacy Organizations in the USA Effectively Communicating New Information about Drugs. Drug Information Association 46th Annual meeting, June 16, 2010

Harnessing Innovations in Molecular and Regulatory Sciences to accelerate Cancer Cures. Massachusetts Institute of Technology NEW Drug Development ParadIGms Initiative (NEWDIGS), September 1, 2010

Dr. Ellen Sigal

Accelerating Cures by Building a More Efficient FDA. Milken Institute Global Conference, April 27, 2010, Los Angeles, CA

Reauthorization of the Prescription Drug User Fee Act. FDA Public Hearing, April 12, 2010, Rockville, MD

Implication of Health Care Reform for Products Safety and the Pharmaceutical Industry. Drug Information Association 46th Annual Meeting, June 16, 2010, Washington, DC

CER Forum – Improving Medical Decisions Using Comparative Effectiveness Research

On June 18, Friends of Cancer Research hosted a forum on the future of comparative effectiveness research (CER). The meeting featured leaders from all sectors discussing priorities for expanding the conduct of CER in the United States.

The forum began with a discussion between Dr. Francis Collins (Director, NIH) and Dr. Carolyn Clancy (Director, AHRQ) moderated by Ramsey Baghdadi of The RPM Report. Dr. Collins and Dr. Clancy were the first designated members of the twenty-one-member Board of Governors of the Patient Centered Outcomes Research Institute (PCORI). This institute, established as a part of health care reform legislation, will play a pioneering role in the future of CER.

During the discussion it was noted that the PCORI Board needs to promote transparency, credibility and inclusiveness in the processes of identifying the best choices for patients.

Dr. Clancy conveyed her optimism about the opportunity to focus research on a longitudinal basis, as well as the legislation's recognition of the need for additional methods of development. Both speakers agreed that achieving meaningful use out of today's wealth of data is a major priority. This included the importance of establishing a robust infrastructure to support ongoing CER. Specifically, Dr. Collins suggested tapping into patient data gathered in HMOs.

The discussion concluded with a conversation regarding the compatibility of CER and personalized medicine. Both agency directors suggested that the two are complimentary and voiced that CER and personalized medicine ought to be considered "hand in glove."

Panel 1: Coordination and Use of CER Studies by Federal Health Agencies

Richard Hodes, Director of the National Institute on Aging, (NIH)

Jesse Goodman, Chief Scientist & Deputy Comm. for Science & Public Health (Acting), (FDA)

Louis Jacques, Director, Coverage & Analysis Group (CMS)

Jean Slutsky, Director, Center for Outcomes and Evidence, (AHRQ)

Stephen Cha, Professional Staff, House Committee on Energy & Commerce

Andrew Hu, Professional Staff, Senate Committee on Finance

The first discussion panel provided the perspective of several key agencies and representatives from Capitol Hill about the future direction for CER.

The panel opened with enthusiasm for CER and the new Institute, as well as the inter-agency collaborations that are already taking place. Dr. Hodes noted those recently established partnerships between the FDA and NIH. The panelists discussed the need for public literacy and transparency in terms of CER, and the importance of filtering CER information through decision makers to improve patient outcomes.

Congressional staff members were asked to discuss the approach they took in developing the health care reform legislation. The two panelists acknowledged that the House and Senate had different visions of the role of CER, although both felt that the language in the bill was structured appropriately. Both panelists made clear that separating CER and coverage decisions was a priority. The staffers also agreed that the makeup of advisory committees would be one of the most important factors determining the Board's success, as well as patient input and participation.

The panelists continued their discussion by suggesting hypothetical CER projects they would like to see undertaken. Dr. Jacques suggested addressing elderly care, and whether particular interventions were as effective if they were administered in-home. Dr. Slutsky spoke of the importance of the CER community to keep an open mind about factors such



Jeff Allen and Bill Dalton



Ramsey Baghdadi, AHRQ Director Clancy, NIH Director Collins

as different populations, interventions, and the spectrum of care. The panelists also discussed clinical trials in terms of CER, and the challenge of encouraging patients to participate when they frequently would prefer existing treatment options, or in some cases experimental treatment.

The panelists all agreed that research priorities and questions should determine how studies are funded. Dr. Goodman suggested that the range from patient intervention to outcomes, and where breakdowns occur in that range, ought to be considered in this context.

Panel 2: Development of a Linked Health Data Network

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

Bob Croyle, Director, *Division of Cancer Control & Population Sciences (NCI)*

Bill Dalton, Director, *Moffitt Cancer Center*

Brett Davis, Senior Director, *Oracle Health Sciences*

Kim Lyerly, Director, *Duke Cancer Center*

Amol Navathe, Medical Officer, *U.S. Department of Health and Human Services*

The final panel discussed the research potential of health data networks and the processes of effectively capturing productive health data.

The panel began by discussing the importance of current data aggregation efforts. Dr. Croyle spoke of the need for balance among several important elements of the aggregation process: infrastructure development, standards development, and grow-your-own development. One of the biggest challenges facing health data aggregation is the amount of unclear evidence that often faces patients. Dr. Navathe described these activities as a priority for government, as well as the balance of long-term and short-term visions and goals.

The panelists agreed that the marriage of different types of data - such as patient-reported outcomes and claims data - was instrumental in making data more useful. The need to encourage active engagement amongst patients, especially in minority communities was highlighted. Coupled with a growing mistrust in the government and the Internet is an increasing trust in physicians. Dr. Navathe felt that government was aware of this issue, and HHS has already begun making investments to address this. The panel concluded with the consensus that the development of an effective data network is the necessary future of health information. Upgrades and investment in Health Information Technology, as well as data collection from a grassroots level, will be required to realize these opportunities.



Bob Croyle, Brett Davis, Kim Lyerly



Stephen Cha, Jesse Goodman

Dr. Ellen Sigal Named to Patient Centered Outcomes Research Institute Board of Governors

Friends of Cancer Research Chair and Founder Dr. Ellen Sigal was appointed to the Patient Centered Outcomes Research Institute (PCORI) Board of Governors. The appointments of the 19 members were announced September 23, 2010 by the Government Accountability Office (GAO). Dr. Sigal fills one of three positions that had been designated for a patient or consumer advocate. The forming of the PCORI board was the first major step for the institute, which was set up as part of the Affordable Care Act passed into law in March of 2010.

Additional Presentations of Note:

Dr. Jeff Allen

The Role of Comparative Effectiveness Research in Improving Cancer Care. Association of Community Cancer Centers 36th Annual National Meeting, March 18, 2010

Impact of Health Reform on Cancer Research and Care. Eastern Cooperative Oncology Group Annual Meeting, June 16, 2010

Comparative Effectiveness Research and the Development of a Linked Data Network. CDISC 2010 Interchange North America, November, 2, 2010

The Patient Protection and Affordable Care Act: The Future of Comparative Effectiveness Research. Food and Drug Administration (FDA) Scientific Rounds, December 8, 2010

Dr. Ellen Sigal

Opportunity and Challenge, Implementation of Comparative Effectiveness Research: The Patient Protection and Affordable Care Act, AACR Annual Meeting, April 20, 2010, Washington, DC

Implementation of The Patient Protection and Affordable Care Act, Comparative Effectiveness Research, Institute of Medicine: National Cancer Policy Summit, October 25, 2010, Washington, DC

Jeff Allen Testifies Before House Energy and Commerce Committee/Subcommittee on Health – NCI Cancer Research: Today’s Progress; Tomorrow’s Challenges

On March 23, 2010, Dr. Jeff Allen, Executive Director of Friends of Cancer Research (*Friends*), testified at a formal hearing of the House Committee on Energy and Commerce’s Subcommittee on Health. Entitled “NCI Cancer Research: Today’s Progress; Tomorrow’s Challenges,” the hearing examined the National Cancer Institute’s (NCI) research efforts and provided recommendations to the path forward for continued progress. Testimony sought to educate Members of Congress and the general public about cancer research conducted and supported by NCI, and to explore the current state of programs at the NCI as well as the direction that the institute, along with the entire cancer community, is headed.

The hearing, held by Subcommittee Chairman Frank Pallone Jr. (D-NJ), opened with statements by all members in attendance including full Committee chair Henry Waxman (D-CA), who stated, “Cancer is a complex disease. We know that genetic, environmental, and other factors all contribute to an individual’s risk for developing cancer. Discovering cures and developing effective treatments are complex, difficult, and expensive endeavors as well.”

Dr. Anna Barker, Deputy Director of NCI, was the first to testify. Dr. Barker opened her testimony by expressing the challenges that cancer research faces, the advances in research, and how increased knowledge of cancer is allowing us to move from a “one size fits all” approach to fighting the disease.

Dr. Barker articulated what challenges are currently being addressed, saying, “NCI’s key challenge is to understand the changes in the genome and associated biology that ultimately cause cancer in order to enable the development of more effective diagnostics, therapies, and prevention strategies that can be delivered to cancer patients.”

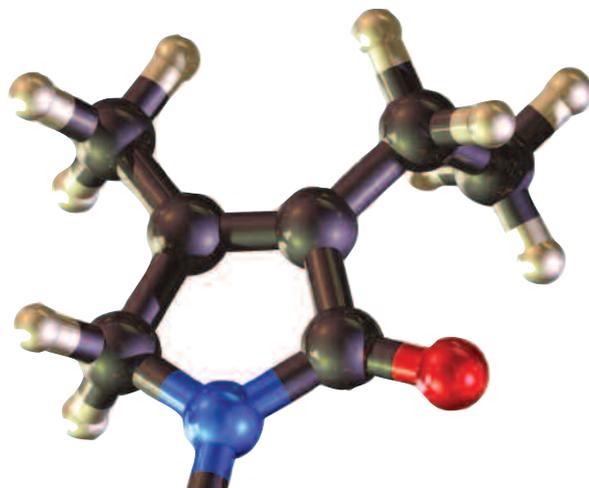
Dr. Barker emphasized the importance of how projects like The Cancer Genome Atlas (TCGA), a comprehensive and coordinated effort aimed to accelerate our understanding of the molecular basis of cancer through the application of genomic analysis technologies, will “re-define cancer targets and provide a rational basis for the development of new targeted diagnostics and therapeutics.” Dr. Barker also said that the American Recovery and Reinvestment Act funds that NCI received helped to significantly expedite this project, which was started in 2005.



Jeff Allen testifies before Congress



Rep. Pallone



Ellen Sigal Presents to the President's Cancer Panel

On December 14, Friends of Cancer Research (*Friends*) Chair and Founder, Dr. Ellen Sigal presented before the President's Cancer Panel in Bethesda, MD. Her presentation was given in conjunction with the release of a white paper titled, *Tearing Down the Silos: Addressing Systematic Barriers in the Research Process*.

During her presentation, Dr. Sigal called on research organizations, regulatory agencies, and industry to tear down the silos that often times exist within each institution. She went on to discuss how all components of the biomedical research community must identify joint priorities and commit to removing bureaucratic, communicative and scientific barriers that are prohibiting efficient use of limited resources to truly change the way in which we approach treating this disease.

The paper outlines three recommendations to push for effective policies to prevent current and future obstacles from hindering innovation and avoiding duplicative or wasteful processes.

1: Reevaluate the Activities of Health-related Agencies

The President should create a task force led by the Secretary of Health and Human Services, in collaboration with agency officials, academic researchers, and patient advocates, to comprehensively examine the various cancer-related efforts of federal agencies and the silos that exist amongst and between them.

2: Develop Multidisciplinary Mechanisms to Support Translational Research

As new programs designed to accelerate translation of new discoveries to available treatments begin to be implemented, individuals with specific expertise in drug development and commercialization should have more direct involvement in the grant writing and review processes.

3: Develop Processes in Healthcare Delivery that Enhance Research

During this time of widespread implementation the federal government should develop policies that enhance data collected within Electronic Health Records to optimally contribute to research activities.

Dr. Sigal concluded by calling on the cancer community to be guided by the principle that both outcomes and patient benefits will be greater when achieved through collaborations versus the disconnected efforts of the past.

"If we are to truly reduce the burden of cancer at a pace at which the millions of patients afflicted by this disease need and deserve, the entire cancer enterprise must take action to tear down the silos and adopt a philosophy of synergy and collaboration."

– Ellen Sigal, PhD

Dr. Jeff Allen gave oral testimony on the second panel, and also submitted a more detailed written testimony for the congressional record. Dr. Allen's statement gave perspective on the vital need for NCI to be in direct collaboration with other federal health agencies, the important role that public-private partnerships, such as the Foundation for the NIH and The Reagan-Udall Foundation, can play in spurring innovation, and the need to tear down the silos that currently exist within the biomedical research community.

He emphasized not only the need for sustained federal funding of research at NCI and NIH, but also the importance of supporting scientific capacity at the Food and Drug Administration, which he said "serves as the nexus between the progression of laboratory research and the clinical use of new therapies." In written testimony submitted to the subcommittee, *Friends* asked that Congress support the President's FY11 budget for FDA, which includes \$25 million for Regulatory Science programs at the FDA.

Dr. Allen concluded his testimony by asking Congress to continue its commitment to ending cancer and the need for a unified front in this fight.

"The advocacy community, and entire research community, must embrace our common goal and support science and collaboration that will enhance the battle against cancer on all fronts," said Dr. Allen. "It is our responsibility to represent patients' needs, and what is needed to end the burden of all diseases."

The second panel to testify also included: Dr. Robert DiPaola, Director, Cancer Institute of New Jersey, and Member of the Science Policy and Legislative Affairs Committee, American Association of Cancer Research (AACR); Megan Gordon, Director of Government Affairs, Pancreatic Cancer Action Network; and Kristin Fitzgerald, a former congressional staffer who lost her husband to cancer.

Symposium – The New Role of Academia in Drug Discovery and Development

On July 6th, Friends of Cancer Research and The University of Kansas Cancer Center, in conjunction with the Kauffman Foundation, Kansas Bioscience Authority and the Council for American Medical Innovation, convened a symposium on "The New Role of Academia in Drug Discovery and Development: New Thinking, New Competencies, New Results and New Paradigms in Cancer Research." The symposium was held at the Kauffman Foundation Conference Center in Kansas City, Missouri. The goal of the conference was to foster collaboration between academia, industry, government and patients to accelerate drug discovery and development in cancer research.

US Secretary of Health and Human Services Kathleen Sebelius delivered the keynote speech and stated, "we have no better partners than our academic centers" in research. She reiterated the Obama administration's commitment to science and emphasized that increasing research budgets alone is not enough; new partnerships are essential in order to achieve success with research dollars. Recently established collaborations within the federal government to cultivate partnerships in drug discovery include the new FDA/NIH Leadership Council announced earlier this year, and the Cures Acceleration Network established as a part of the healthcare reform legislation.

During a special panel discussion moderated by former House Majority Leader Richard Gephardt, FDA Commissioner Dr. Margaret Hamburg and NIH Director Dr. Francis Collins discussed programs they are implementing to capitalize on the strengths of each agency in advancing medical research. Dr. Collins stated that government collaboration is just one vehicle, but it will require collaboration with academia and industry to develop new innovations. Dr. Hamburg said that there is a need to step back and evaluate the FDA's role in advancing biomedical research. She discussed the need for an emphasis on regulatory science, which has the ability to bring a cutting-edge scientific approach to safety and efficacy evaluations of new products. Dr. Hamburg told the audience that President Obama included money in the 2011 budget specifically targeted to advance regulatory science.

The conference consisted of four panel discussions moderated by Reuters National Health Correspondent Lisa Richwine. The first panel focused on a new way of thinking about how industry and academia can better collaborate to successfully and efficiently translate scientific discoveries into new therapies. The panelists emphasized that moving an idea from basic research into clinical study is becoming increasingly difficult. New ideas often languish due in part to a scarcity of resources for early stage investment, and in part to a lack of expertise. The panelists agreed that a new model requires multi-disciplinary interaction at each step of the process to encourage the translation from basic science to clinical application.



Tom Thornton, Judy Baker, Rep. Gephardt, Ellen Sigal, Rep. Moore, FDA Commissioner Hamburg, Secretary Sebelius, NIH Director Collins, Barbara Atkinson, Bernadette Gray-Little



Secretary Sebelius delivers keynote

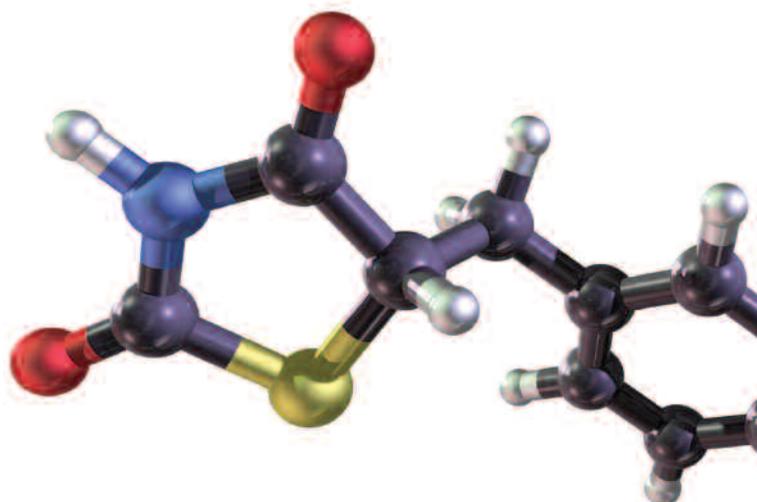


Secretary Sebelius delivers keynote

The second panel focused on how new collaborations can aid in the development of new tools to evaluate medical products and establish best practices for commercial agents. Increased collaboration between academic centers and FDA could help support regulatory science programs and develop core competencies for product development. Institutions with a specific area of expertise could serve as a "Center of Excellence" for routine interactions and research. Panelists also noted that the responsibilities of the FDA have increased while the resources have not. The current model of academic promotion and publication system does not reward collaboration and in order for collaboration between FDA and academic centers to occur, this culture must change. There is a role for the NIH/academia/FDA in developing standards and templates to administer guidelines that can promote cooperation as opposed to starting from the beginning each time a partnership is formed.

The third panel explored how directed research can efficiently lead to new therapies. New models of providing economic capital are serving as conduits to finding the right partners to commercialize proof-of-concept finding with signs of success. One example highlighted a partnership with the University of Kansas Institute for Advancing Medical Innovation and the Leukemia & Lymphoma Society as a model for directed funding to bring promising new research to the patient. The panel emphasized the need for all partners to align towards common goals and the need to have the right people with the right expertise in place at all levels to accelerate drug delivery.

The final panel discussed new policies that can help these collaborations thrive, such as increased federal support for these types of partnerships and new incentives for academic medicine. Panelists agreed that there is a need for academic administrators to be willing to support their faculty and create the infrastructure that is needed for this type of drug discovery research. It is critical for industry and academia to collaborate in order to deliver new biomedical innovations to patients.



Additional Presentations of Note:

Dr. Jeff Allen

Translational Medicine and Therapeutics: Engaging in a Dialogue with the Public. National Institutes of Health Scientific Management Review Board, September 15, 2010

Dr. Ellen Sigal

Evaluating and Funding Translational Research, AAAS Annual Meeting, February 19, 2010, San Diego, CA

NCI's Role Catalyzing the Research and Development Continuum, National Cancer Advisory Board Meeting, May 5, 2010, Bethesda, MD

2010 Conference on Clinical Cancer Research

To address critical issues in the development of new oncology drugs, Friends of Cancer Research and The Engelberg Center for Health Care Reform at Brookings Institute co-hosted the third annual Conference on Clinical Cancer Research with the support of The American Association for Cancer Research (AACR), The American Society of Clinical Oncology (ASCO), and Susan G. Komen for the Cure. The conference brought together leaders in cancer drug development from federal health and regulatory agencies, academic research, and the private sector for a focused discussion on key issues surrounding the development and regulation of cancer drugs and therapies.

In his opening keynote address, National Cancer Institute (NCI) director Dr. Harold Varmus discussed the promise of targeted cancer therapies as well as the challenges inherent in developing drug combinations to target specific physiological pathways.

In her afternoon keynote address, FDA commissioner Dr. Margaret Hamburg discussed the gap between the advancement of modern science and the availability of current therapies. She stressed the need for investment in regulatory science to bridge this gap.

The following four panels presented specific topics in clinical cancer research:



FDA Commissioner Hamburg

Panel One – Adaptive Clinical Trials Designs for Simultaneous Testing of Matched Diagnostics and Therapeutics

Howard I. Scher, MD, *Memorial Sloan-Kettering Cancer Center*

Richard Simon, DSc, *National Cancer Institute*

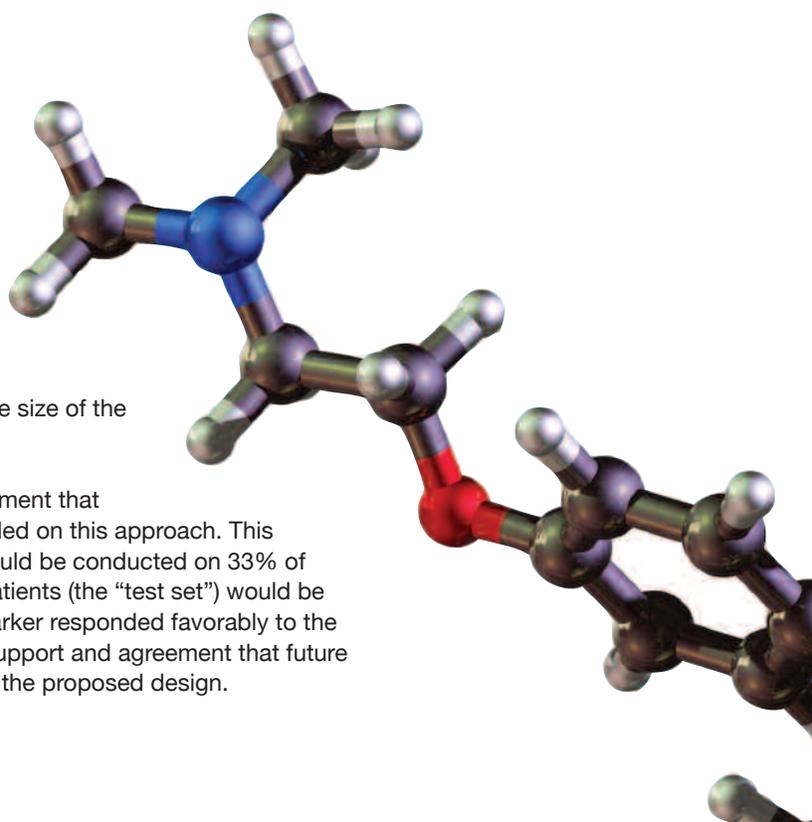
Rajeshwari Sridhara, PhD, *US Food and Drug Administration*

Eric Rubin, MD, *Merck*

Shelley Fuld Nasso, *Susan G. Komen for the Cure*

Panel one discussed a potential adaptive phase III trial design that could facilitate the co-development of matched diagnostics and therapeutics. It is often not possible to identify a predictive biomarker before the start of a clinical trial to test a therapeutic. However, adaptive phase III trial designs that can identify a suitable target population during the early course of the trial would enable the efficacy of an experimental therapeutic to be evaluated within the target population as a later part of the same trial. The use of adaptive design in phase III may offer new opportunities for matched diagnosis and treatment because the size of the trial can allow for subpopulation analysis.

The proposed design garnered enthusiastic support and agreement that future adaptive trials for use in phase III testing could be modeled on this approach. This included a “training set” approach in which interim analysis would be conducted on 33% of patients to identify a classifying biomarker(s). The remaining patients (the “test set”) would be analyzed to determine if those positive for the predictive biomarker responded favorably to the test therapeutic. The proposed design garnered enthusiastic support and agreement that future adaptive trials for use in phase III testing could be modeled on the proposed design.





Dr. Varmus delivers morning keynote

Panel Two – Identification and Elucidation of the Biology of Adverse Events: The Challenges of Safety Assessment and Translational Medicine

Ken Turteltaub, PhD, *Battelle Memorial Institute*
John Leighton, PhD, *US Food and Drug Administration*
Myrtle Davis, DVM, PhD, *National Cancer Institute*
Leigh Ann Burns-Naas, PhD, *Pfizer Inc*
Adam Clark, *FasterCures*

Panel two focused on how to adopt a systems biology approach to evaluating toxicities in oncology treatments and how pre-clinical safety testing currently relies heavily on outdated animal models. The panel discussed how taking advantage of emerging technologies, such as genomics and proteomics, may lead to better safety decisions based on an understanding of the biology of an adverse event.

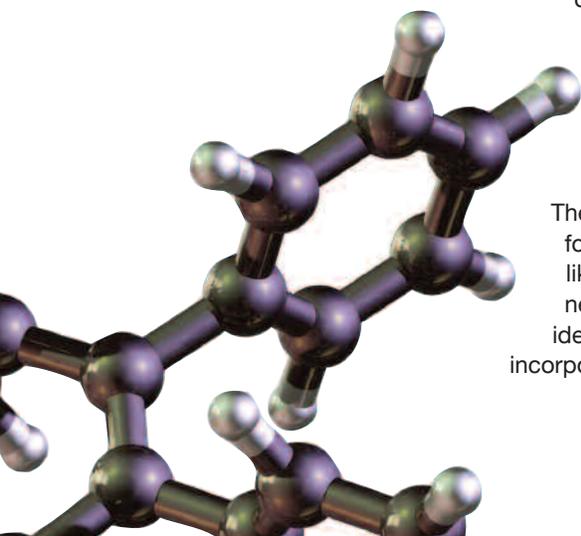
Panelists and attendees discussed the potential for systems biology to make pre-clinical safety testing more efficient and accurate. They also voiced the hope that systems biology could eventually be used to guide the modification of a therapeutic so that desired (“on-target”) effects were maintained while potential undesired (“off-target”) effects were eliminated or minimized. The need for validation of systems biology approaches was also discussed as a potential concern. Panel discussion ultimately led to plans for the formation of an oncology toxicity biomarkers consortium.

Panel Three – Integrating Pain Metrics into Oncologic Clinical and Regulatory Decision-Making

Charles Cleeland, PhD, *MD Anderson Cancer Center*
Laurie Burke, RPh, MPH, Office of New Drugs, CDER, *US Food and Drug Administration*
Ann O’Mara, PhD, RN, FAAN, *Palliative Care Research, Community Oncology Prevention, NCI*
Martin Zagari, MD, *Amgen*
Carole Baas, PhD, Advocate, *Physical Sciences in Oncology, NCI*

Panel three focused on how to incorporate pain metrics into clinical oncology studies and how pain measurements could contribute to regulatory decisions and/or labeling changes. Panelists looked at the prevalence and severity of cancer-related pain and that the majority of clinical trials do not include pain palliation or prevention as either a primary or secondary endpoint, due in part to the subjective nature of pain.

The panel discussed the feasibility of developing objective standards for pain measurement and that while assessments of pain would likely be in the form of patient-reported outcomes (PROs), there is a need to develop new tools for pain measurement, such as identifying circulating biomarkers indicative of pain. The incorporation of information regarding pain palliation or prevention into



labeling was also discussed. It was proposed that an accompanying trial with pain palliation as the primary endpoint could lead to pain or other symptom labeling for an NDA/BLA. It was noted that this would be in conjunction with a phase III trial with a more traditional primary endpoint, such as overall survival or progression-free survival.

Panel Four: Using Patient-Initiated Study Participation in the Development of Evidence for Personalized Cancer Therapy

Stephen Friend, MD, PhD, *Sage Bionetworks*
(Co-chair)

Richard L. Schilsky, MD, *University of Chicago*
(Co-chair)

Laurie Fenton Ambrose, *Lung Cancer Alliance*

Ken Buetow, PhD, *National Cancer Institute*

Jamie Freedman, MD, PhD, *GlaxoSmithKline*

Sue-Jane Wang, PhD, *US Food and Drug Administration*

Panel four discussed a possible patient-initiated process for collecting patient information to potentially lead to label changes on already approved oncology drugs. Patients would contribute biological specimens in addition to detailed diagnostic, clinical, and demographic data to a common database. Analysis of phenotypic or molecular traits could help identify patient subgroups that are unlikely to respond to specific therapeutics already on the market. Data obtained in this fashion may be used to alter the labeling to indicate a more defined subset of patients fit to receive a therapy.

The panel described a potential pilot study for non-small cell lung cancer to identify molecular signatures indicative of non-response to chemotherapy. Panel discussion focused on the feasibility of engaging patients to contribute, how to minimize bias in patient accrual, and the type of data and statistical analyses that might be needed to support a post-approval labeling revision. Sage Bionetworks plans to proceed with the study, which could form a model for future patient-initiated studies.



NCI Director Varmus



Ken Buetow



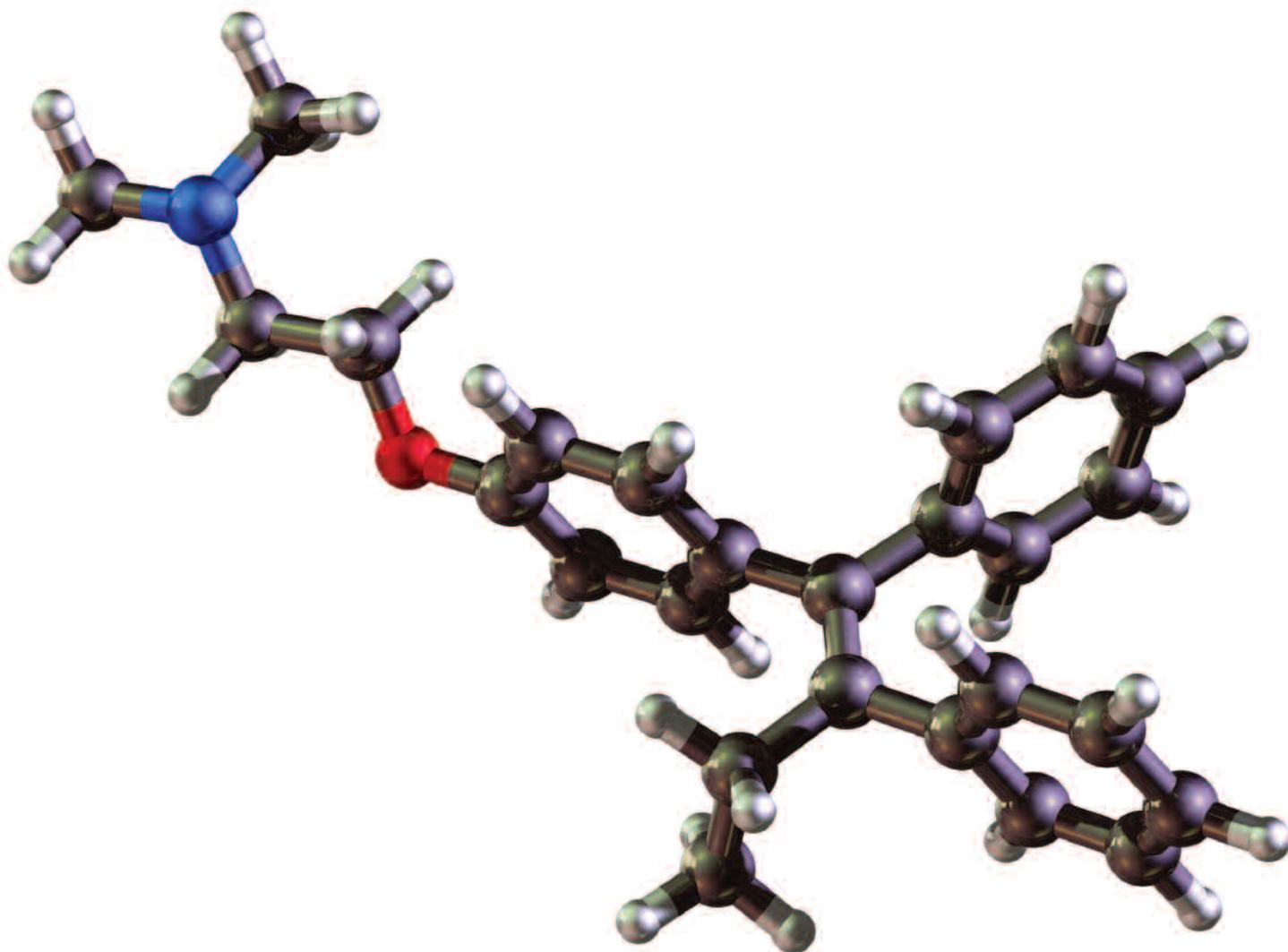
Janet Woodcock

FDA Releases Guidance Based on 2009 CCCR Panel

In follow-up to the 2009 Conference on Clinical Cancer Research, Friends submitted a draft guidance to the FDA based on a panel discussion titled *Development of Rational Drug Combinations with Investigational Targeted Agents*. The panel examined specific situations for which a large, four-arm phase III clinical trial could be modified and proposed criteria and potential development plans that would generate the desired data on the combination as well as the individual agents. The proposed document, submitted in March 2010, addressed the unique challenges surrounding development of therapies involving two (or more) new molecular entities (2NMEs).

On December 14, 2010, the FDA released a draft guidance document that mirrors the 2009 panel, subsequent white paper, and submitted draft guidance. This was the product of a great deal of collaborative work between many individuals and organizations, with special acknowledgment to conference supporters the American Association for Cancer Research, the American Society of Clinical Oncology and Susan G. Komen for the Cure.

Media coverage of draft guidance included articles published in *The Wall Street Journal*, *The Pink Sheet*, *Nature*, and the *Massachusetts Medical Device Journal*.



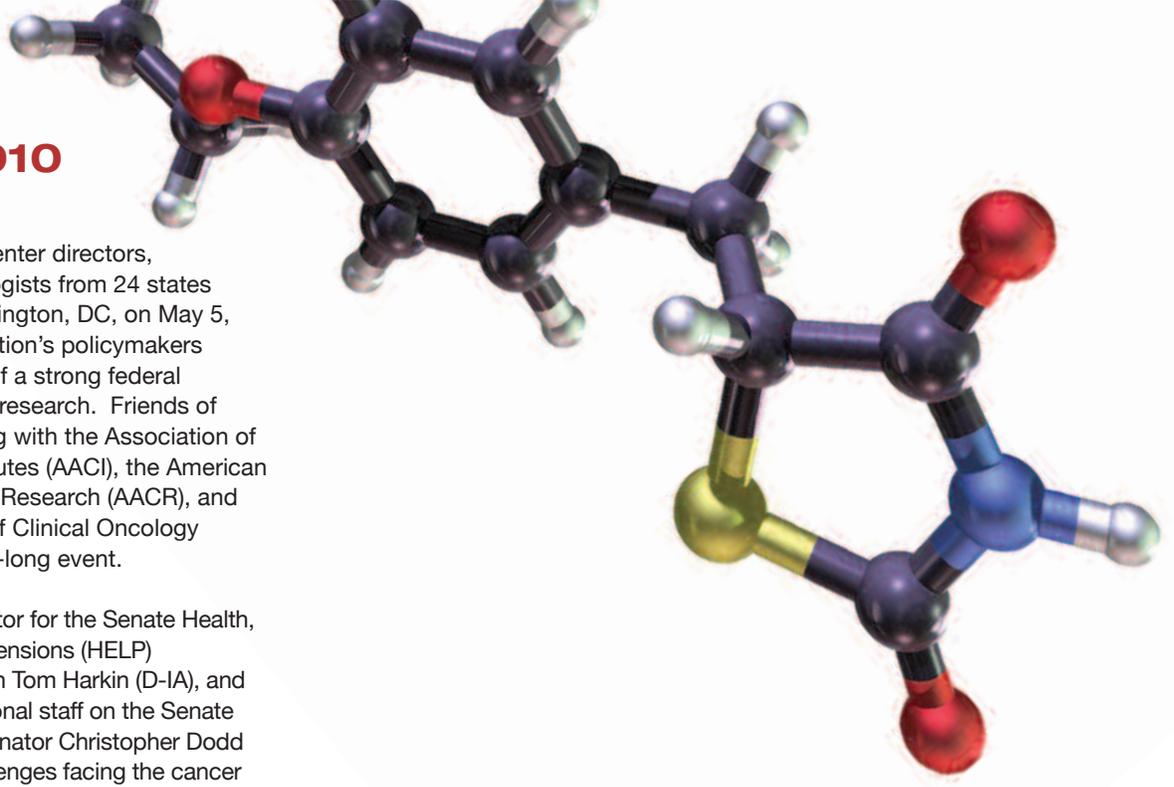
Hill Day 2010

More than 80 cancer center directors, researchers and oncologists from 24 states descended upon Washington, DC, on May 5, 2010 to educate the nation's policymakers about the importance of a strong federal commitment to cancer research. Friends of Cancer Research, along with the Association of American Cancer Institutes (AACI), the American Association for Cancer Research (AACR), and the American Society of Clinical Oncology (ASCO) hosted the day-long event.

Daniel Smith, staff director for the Senate Health, Education, Labor and Pensions (HELP) Committee for Chairman Tom Harkin (D-IA), and Jeremy Sharp, professional staff on the Senate HELP Committee for Senator Christopher Dodd (D-CT), addressed challenges facing the cancer research enterprise in the current political climate and urged advocates to educate Congress on the economic and lifesaving value of cancer research. The cancer center representatives then spread out across the Capitol and advocated for increased federal funding for cancer research during the 144 meetings with Members of Congress and their aides.

During lunch, Senator Sherrod Brown (D-OH) accepted an award from all four organizations in recognition of his leadership on the Access to Cancer Clinical Trials Act, which was enacted into law as part of the Patient Protection and Affordable Care Act.

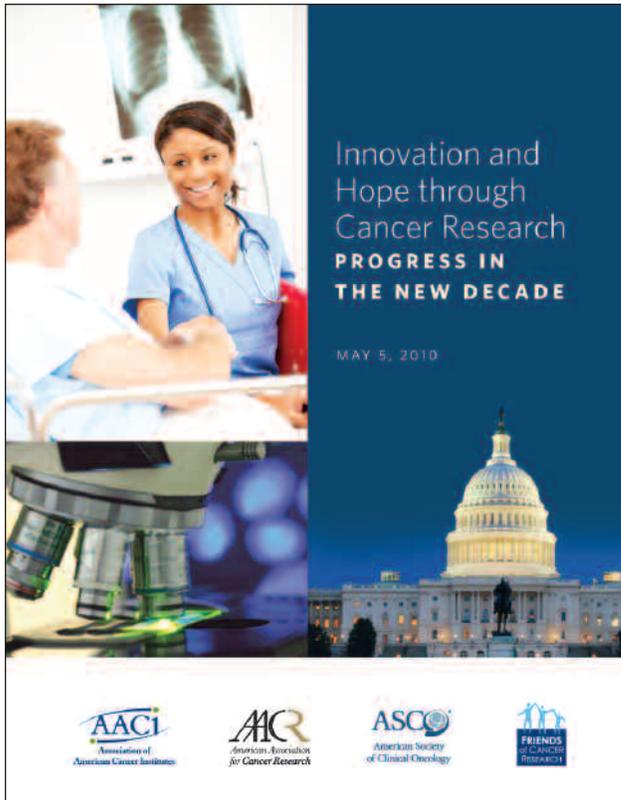
During a reception, hosted the evening before the Hill day, AACI, AACR, ASCO and *Friends*, along with cancer center directors from around the country, honored Representative Lois Capps (D-CA) for her support of cancer research and for drafting the House companion to the Senate's 21st Century Cancer ALERT Act. Senator Frank Lautenberg (D-NJ), a recent cancer survivor, also attended the event and praised the four organizations for their efforts.



Advocates meet with Sen. Specter



Rep. Capps receives award at reception



Advocates meet in House office



Sen. Brown



Additional Presentations of Note:

Ryan Hohman, JD, MPA

Director, Communication & Policy

National Dialogue for Healthcare Innovation Summit on Physician-Industry Collaboration, October 4, 2010

Presented testimony from the advocacy perspective along with: Diane Bieri, Executive VP and General Counsel of PhRMA; Emilia DiSanto, Chief Counsel to the Minority, Senate Finance Committee; Dr. Thomas Fogarty, Fogarty Institute for Innovation; and Christopher White, General Counsel to AdvaMed.

Friends Annual Cancer Leadership Awards Reception

On March 3, 2010, Friends of Cancer Research (*Friends*) hosted its Annual Cancer Leadership Awards Reception at the Hotel Monaco in Washington, DC. The reception was attended by supporters of *Friends* and by key leadership from advocacy, government, and industry.

Awards were presented to Senator Daniel Inouye (D-HI); Dr. Margaret Hamburg, Commissioner of the U.S. Food and Drug Administration; Dr. Francis Collins, Director, the National Institutes of Health; and Ambassador Nancy G. Brinker, Founder and CEO of Susan G. Komen for the Cure. The honorees were awarded in recognition of their extraordinary efforts to advance the fields of cancer research, awareness, treatment, and detection. Former Senator Ted Stevens (R-AK) accepted the award on behalf of Mr. Inouye who was at work taking votes during the event. *Friends* Chair and Founder Dr. Ellen Sigal presented Dr. Hamburg with her award for being a champion for the advancement of regulatory science with the FDA. Senator and cancer survivor Arlen Specter (D-PA) presented Dr. Collins with his award in recognition of his new leadership at the NIH and commitment to advancing cancer and biomedical research. Finally, *Friends* President Marlene Malek closed out the program and presented Ms. Brinker, a tireless advocate for cancer prevention and research, with her award.



Ambassador Nancy Brinker



NIH Director Collins and Sen. Specter

Senator Ted Stevens (1923-2010)

Friends of Cancer Research would like to express our most heartfelt condolences to the family and friends of Senator Ted Stevens. Senator Stevens was a prostate cancer survivor, and, during his decades in Congress, a steadfast champion of cancer and biomedical research.



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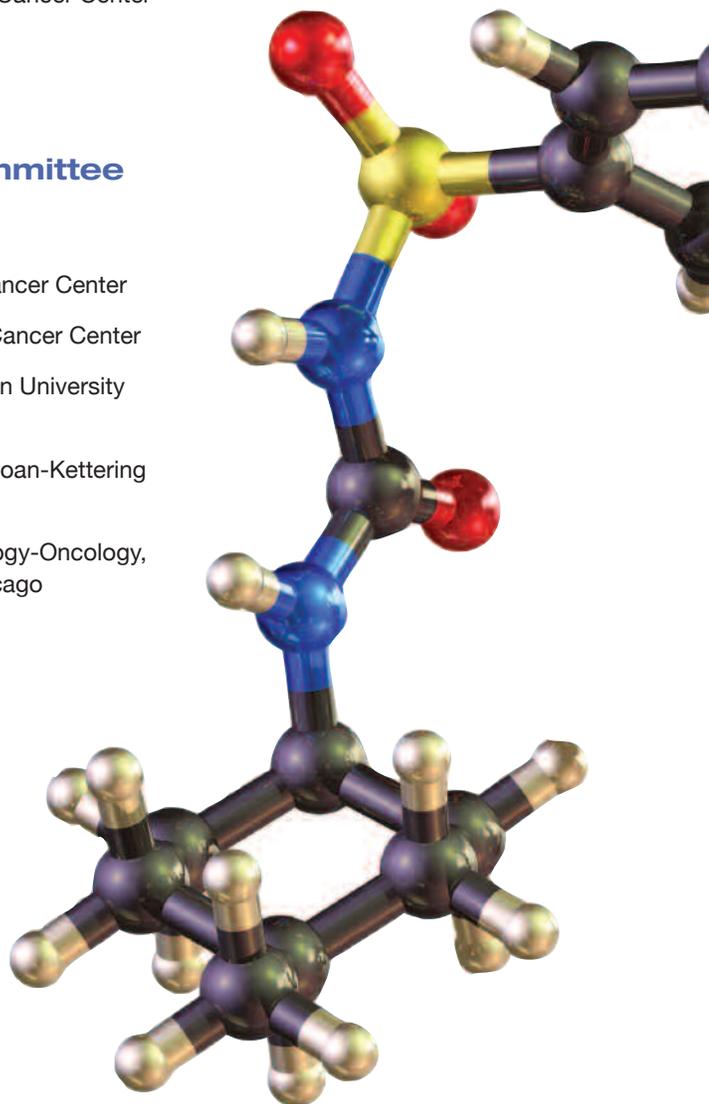
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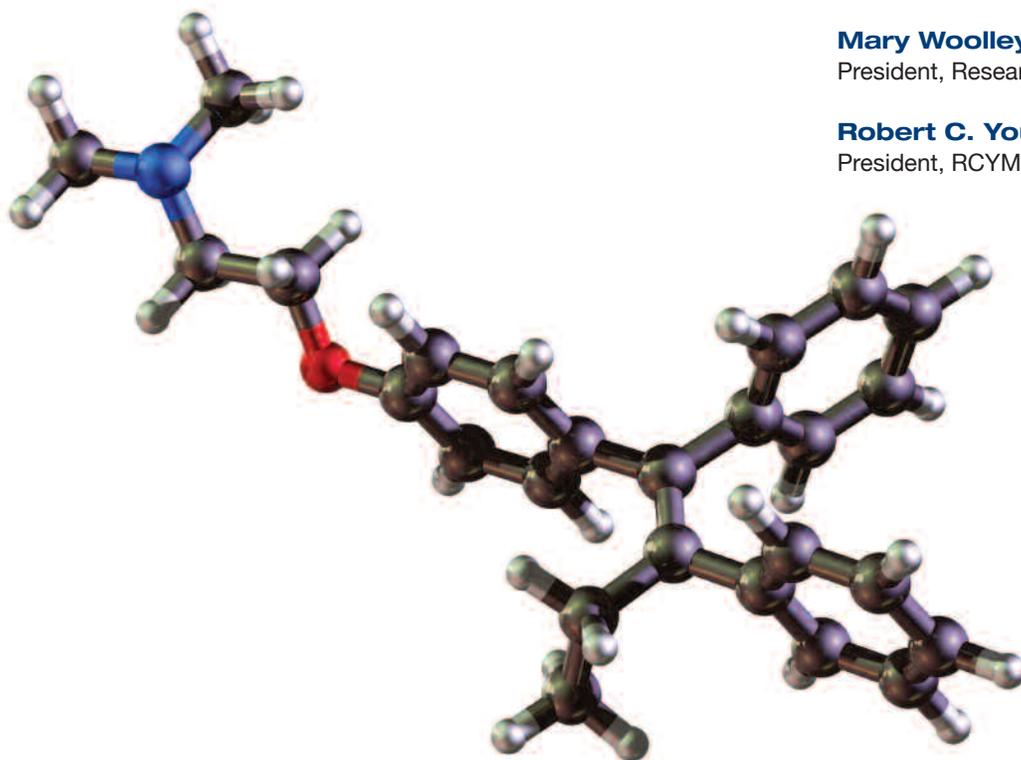
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