

# POLICY-RELATED BUILDING BLOCKS FOR ADVANCING PRECISION MEDICINE

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Managing Director, Science Policy  
and Government Affairs

**CANCER CROSSLINKS**  
Lund, Sweden  
October 14, 2015

## MY PRESENTATION TODAY

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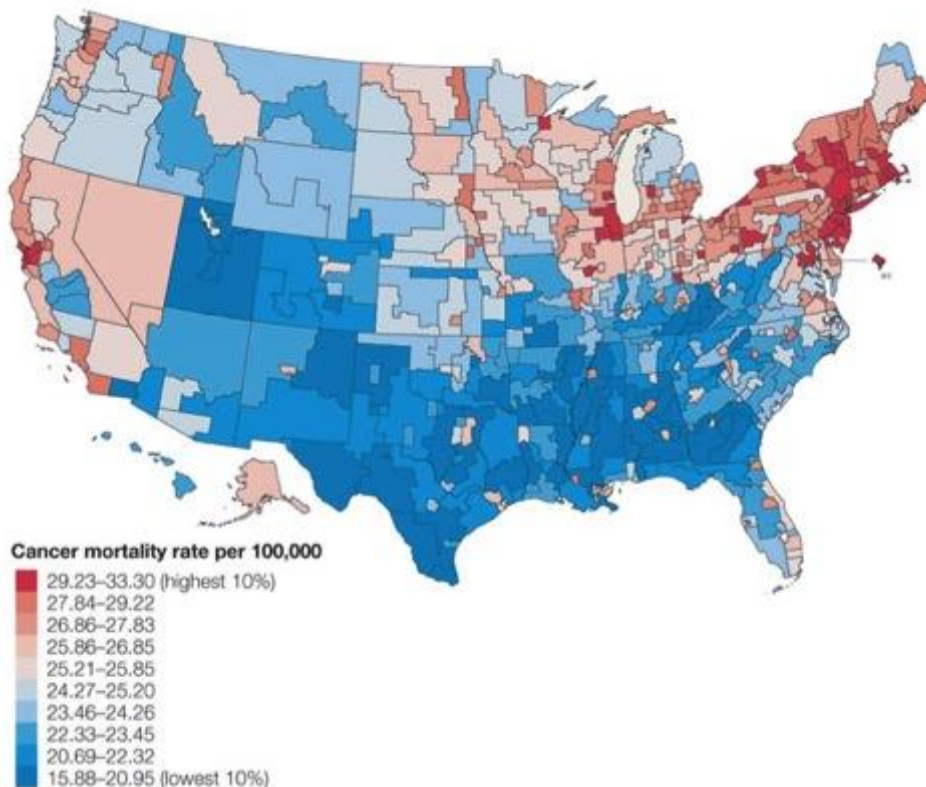
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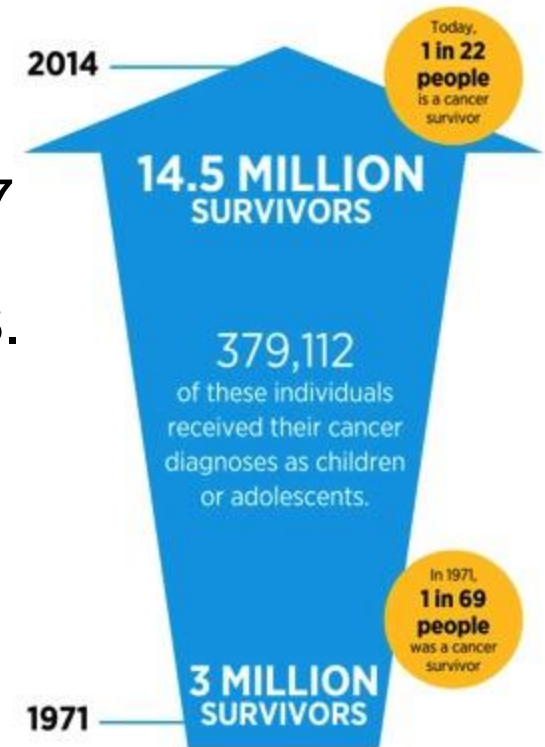
# THE STATUS OF CANCER IN THE U.S.



- Approximately 44% of men and 38% of women will develop cancer in their lifetimes
- 1,658,370 new cancer cases and 589,430 cancer deaths are expected in 2015 (1,620 per day)
- Cancer is the second most common cause of death from disease, the first being cardiovascular disease
- If left unabated, 2,387,304 new cases are expected to be diagnosed in the U.S. annually by 2035

# ENCOURAGING NEWS ABOUT CANCER IN THE U.S.

- The 5-year relative survival rate for all cancers diagnosed between 2005 and 2009 was **68%**, up from **49%** in 1975-1977
- The number of cancer survivors in the U.S. today is now **14.5 million**
- A cancer diagnosis is no longer an automatic death sentence
- Progress is moving at a faster pace than ever before in our history, but much more work needs to be done to achieve our goals



# PROJECTIONS OF THE GLOBAL CANCER BURDEN

## 2015

- **15.2 million** new cases
- **8.8 million** deaths

## 2035

- **23.9 million** new cases
- **14.6 million** deaths



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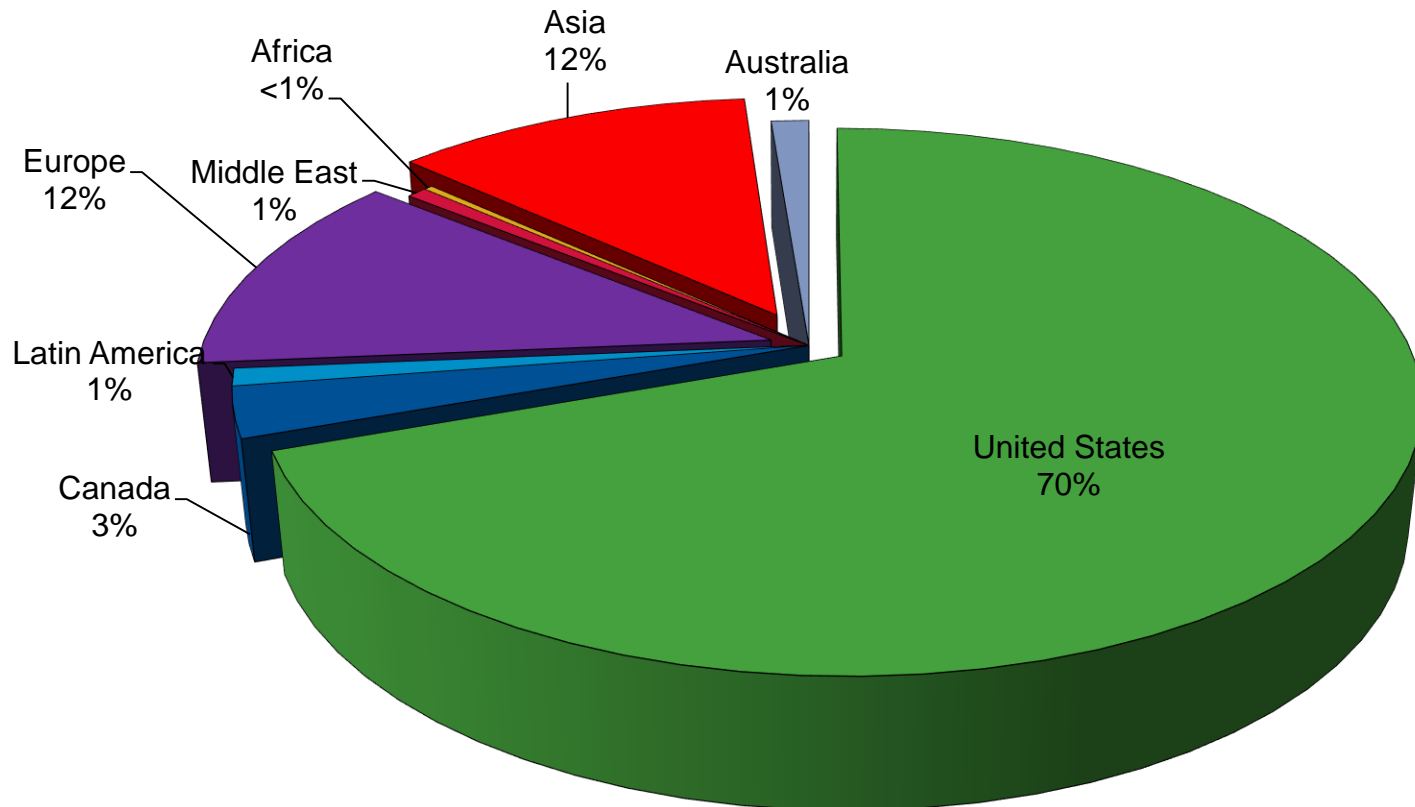
## To prevent and cure cancer through:

- Research
- Education
- Communication
- Collaboration

**THESE FOUR AREAS ARE DIRECTLY RELEVANT TO  
EVERYTHING WE ARE DOING FROM A SCIENCE POLICY  
PERSPECTIVE TO ADVANCE PRECISION MEDICINE**

# AACR MEMBERSHIP DISTRIBUTION ACROSS THE GLOBE

**The AACR is the home of over 35,000 members residing in 101 countries, representing all sub-disciplines and sectors in the cancer field**



# AACR'S FOOTPRINT IN THE CANCER RESEARCH COMMUNITY



- **Eight high-impact scientific journals**  
All AACR Journals rank in the top 30% of all Oncology journals in terms of Impact Factor, including three in the top twelve



# AACR'S FOOTPRINT IN THE CANCER RESEARCH COMMUNITY



## ■ Annual Meeting

Over 19,300 attendees at the 2015 meeting, with top-tier media coverage and social media excitement

# AACR'S FOOTPRINT IN THE CANCER RESEARCH COMMUNITY



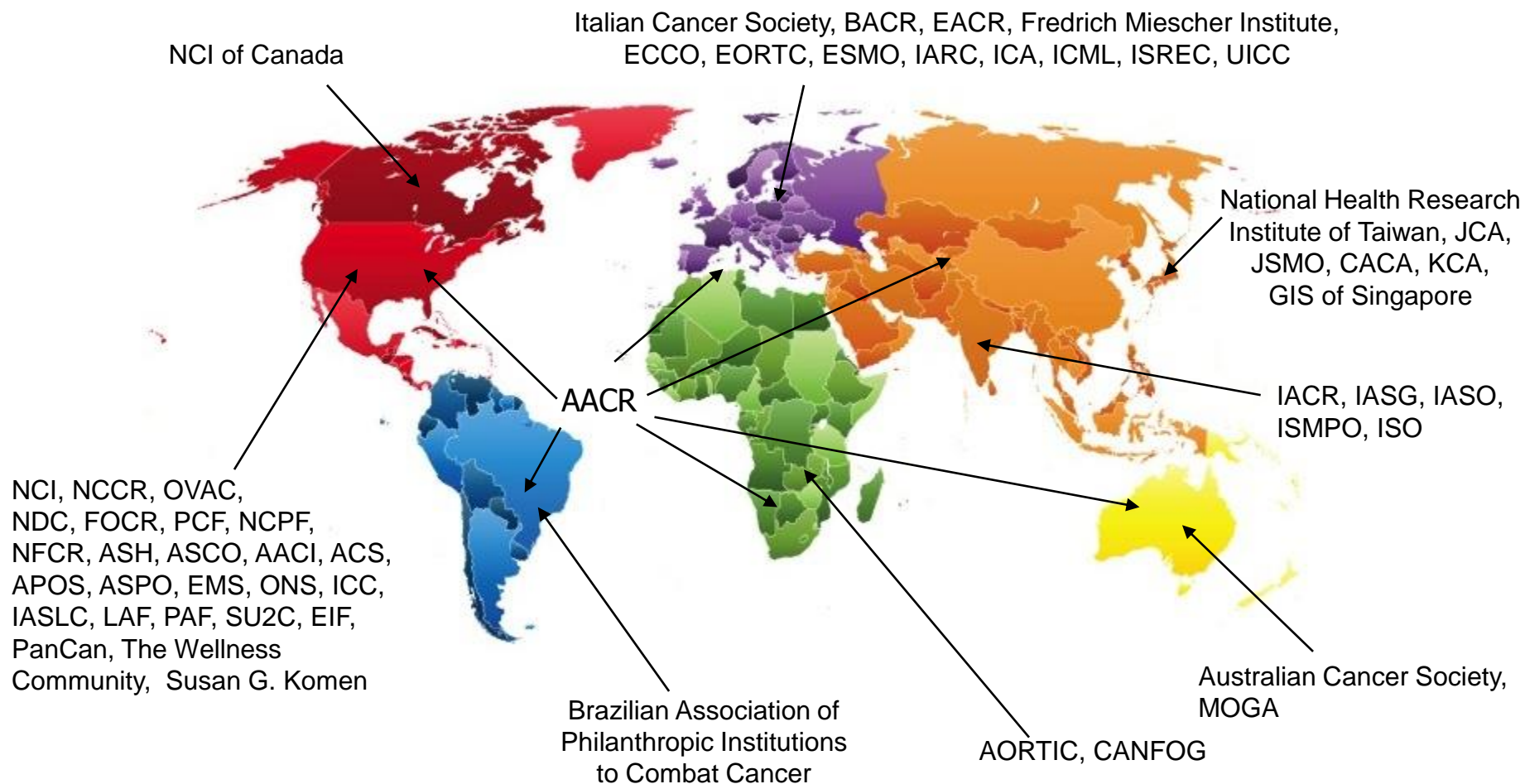
- **36 additional** national and international scientific meetings, conferences, and educational workshops, with a projected total attendance of over 30,000 in 2015



# RESEARCH GRANTS ADMINISTERED BY THE AACR

- As the scientific partner of Stand Up To Cancer, AACR has administered over **\$239 million** to fund translational research projects through its peer review mechanism
- The AACR has administered over **\$60 million** dollars in grants since 1993. Over **\$8 million** in 2015 to fund the research of 54 scientists
- There are more than **20** AACR Research Grant programs, and the applications for these funds are reviewed by AACR committees that are expert in various topics and organ sites
- The various grant mechanisms administered by the AACR are: Career Development Awards, Research Fellowships, Pathway to Leadership Grants, and other grants for established investigators
- Comprehensive reports are provided to the collaborators and funders, and post-award evaluation is being planned
- The AACR will be awarding the first group of “NextGen Grants for Transformative Cancer Research” in 2016. These grants will provide **\$450,000** over 3 years for young researchers performing groundbreaking research

# AACR NATIONAL AND INTERNATIONAL COLLABORATIONS



# AACR SCIENCE POLICY AND GOVERNMENT AFFAIRS GOALS AND ACTIVITIES

- Serve as an authoritative voice for important issues of science and public policy
- Advocate for increased funding for cancer research and related biomedical science
- Formulate policy recommendations to federal agencies:
  - NIH, NCI, FDA, OHRP, PCORI, CMS
- Produce and disseminate advocacy publications
- Inform policymakers and the public through meetings and special briefings





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# OPPORTUNITY FOR CHANGE: PRECISION MEDICINE

**“Change is the law of life. And those who look only to the past or present are certain to miss the future.”**

– John F. Kennedy



## ■ Science is leading the way

- Emerging molecular information is transforming cancer prevention and care
- Innovative clinical research (adaptive clinical trials) is delivering new therapies to patients more rapidly

## ■ Smart public policies are necessary to continue the momentum

- **Funding** – Robust and sustained investments are required
- **Regulatory** – Establishing effective frameworks for regulating, compiling and manipulating the influx of information that can keep pace with rapid scientific discoveries [FDA guidance on lab-developed tests (LDTs) & Next Generation Sequencing (NGS)]
- **Human Subject Research Protections** – “Common Rule” revisions must properly balance the need to protect research subjects, while reducing the burden on investigators
- **IT** – Data sharing, common standards (EHRs), protection of data is important
- **Reimbursement** - Paying for personalized medicine/reimbursement policies

# SMART PUBLIC POLICIES ARE NECESSARY TO CONTINUE THE MOMENTUM

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# THE NIH FUNDING CRISIS

- The NIH's ability to support lifesaving research has declined **25%** since FY 2004, when taking inflation into consideration
- The NIH and NCI budgets in FY 2015 (\$30.311 billion and \$4.95 billion, respectively) remain below FY 2010 levels (\$31.238 billion and \$5.1 billion, respectively)
- During FY 2014, the NCI awarded **1,207** competing Research Project Grants (RPGs), compared to **1,487** RPGs in FY 2004
- During FY 2014, the NCI overall success rate was **14%**, compared to a **27%** success rate in FY 2003 for NCI

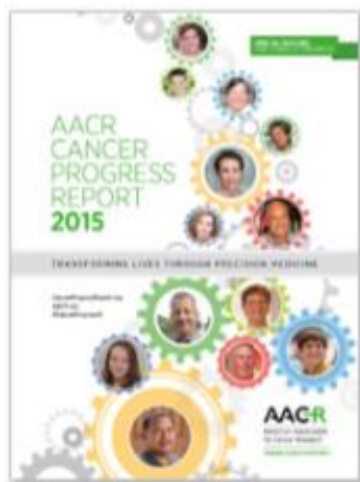


# AACR ANNUAL CANCER PROGRESS REPORT: AN IMPORTANT TOOL FOR ADVOCACY

**AACR** American Association  
for Cancer Research  
FINDING CURES TOGETHER™

## Our 5th annual *AACR Cancer Progress Report 2015*

- In the first four reports, 50 courageous individuals have shared their personal experiences with cancer



This annual report to Congress and the American public chronicles how research continues to transform lives such as those of 13 courageous individuals, including two Members of Congress, who have shared their experiences with cancer in this year's edition.

Highlights the FDA-approved molecularly targeted agents in 2015





# AACR CANCER PROGRESS REPORT 2015 RELEASE

## US Capitol Hill September, 16, 2015

AACR Cancer Progress Report 2015  
Released to Standing Room  
Only Crowd



The AACR held a special briefing on September 16 on Capitol Hill to announce the release of the AACR Cancer Progress Report 2015. This event was the fifth release this annual report that highlights the progress and promise of cancer research and emphasizes the need for robust, sustained, and predictable increases in funding for the NIH, NCI and FDA.



# AACR: FOUNDING SPONSOR OF THE RALLY FOR MEDICAL RESEARCH (2013)

**AACR** American Association  
for Cancer Research  
FINDING CURES TOGETHER™





# RALLY FOR MEDICAL RESEARCH HILL DAY SEPTEMBER 17, 2015

**AACR** American Association  
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FINDING CURES TOGETHER™

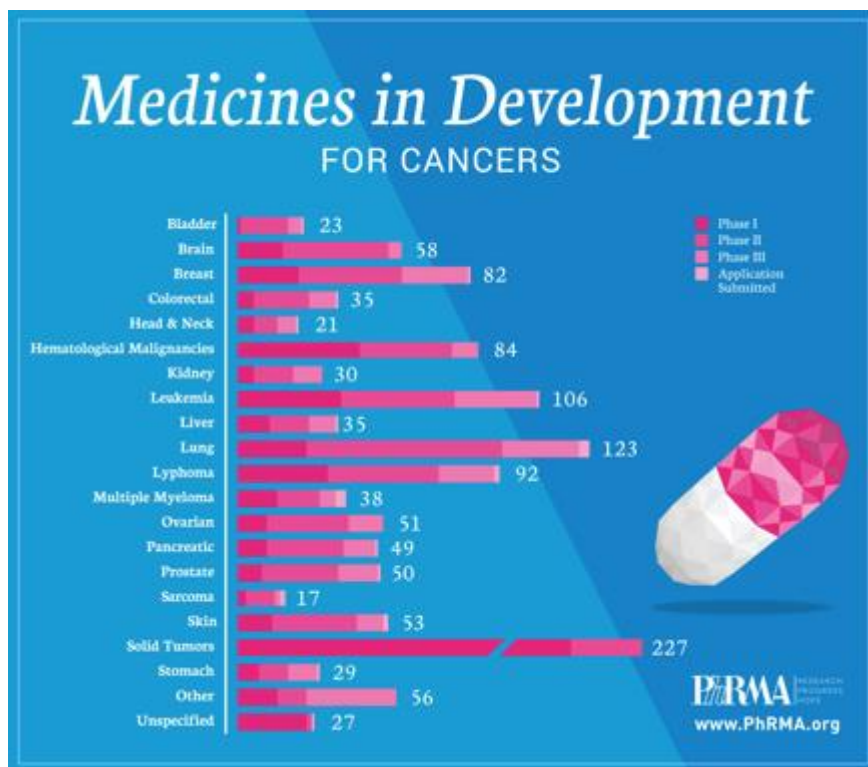
## Third Annual Rally for Medical Research Hill Day



On Sept. 17, 2015, the AACR and more than 300 advocacy organizations representing millions of people joined together for the third annual Rally for Medical Research Hill Day.



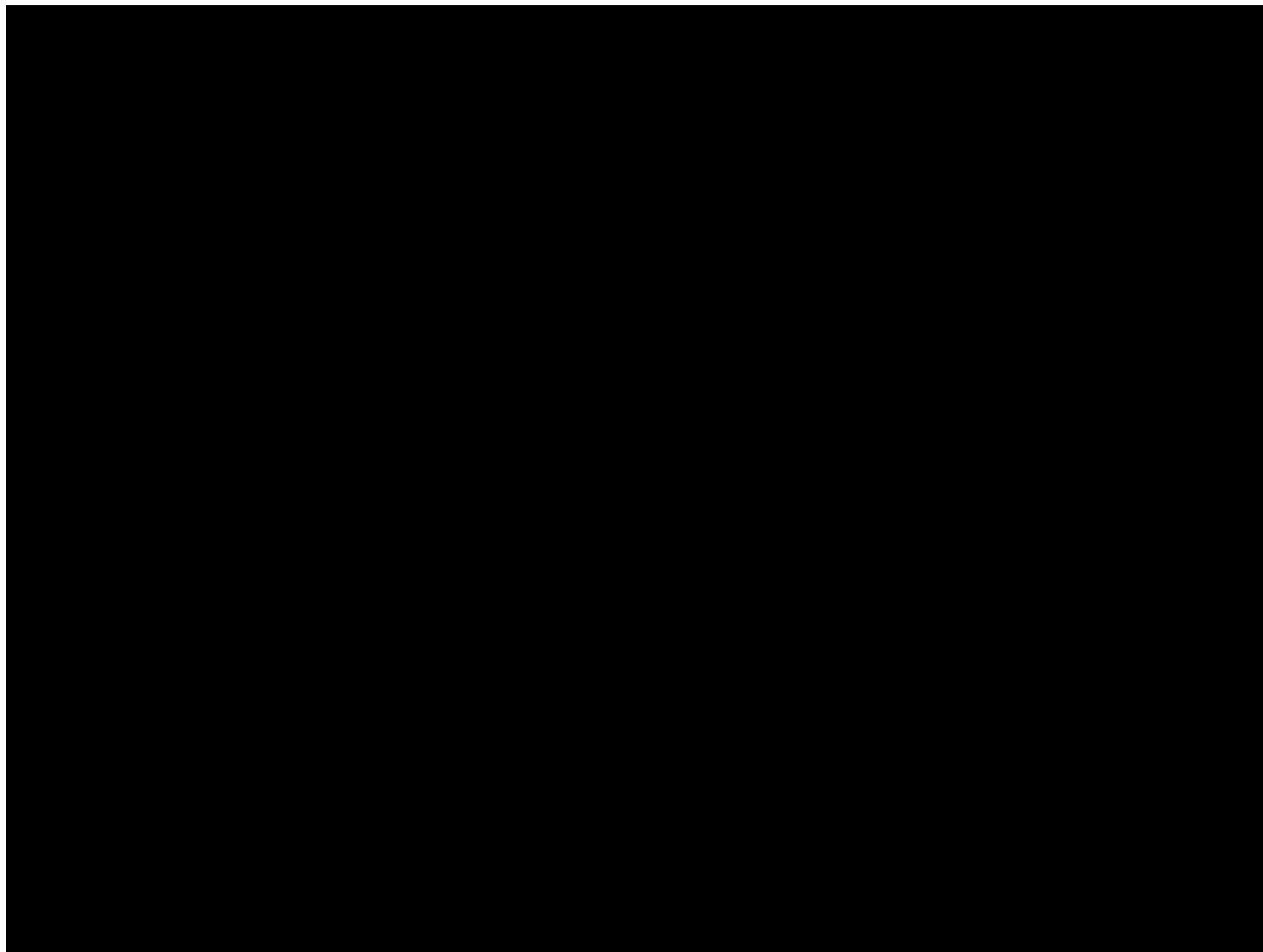
# 2015 PhRMA CANCER REPORT (RELEASED SEPTEMBER 9, 2015)



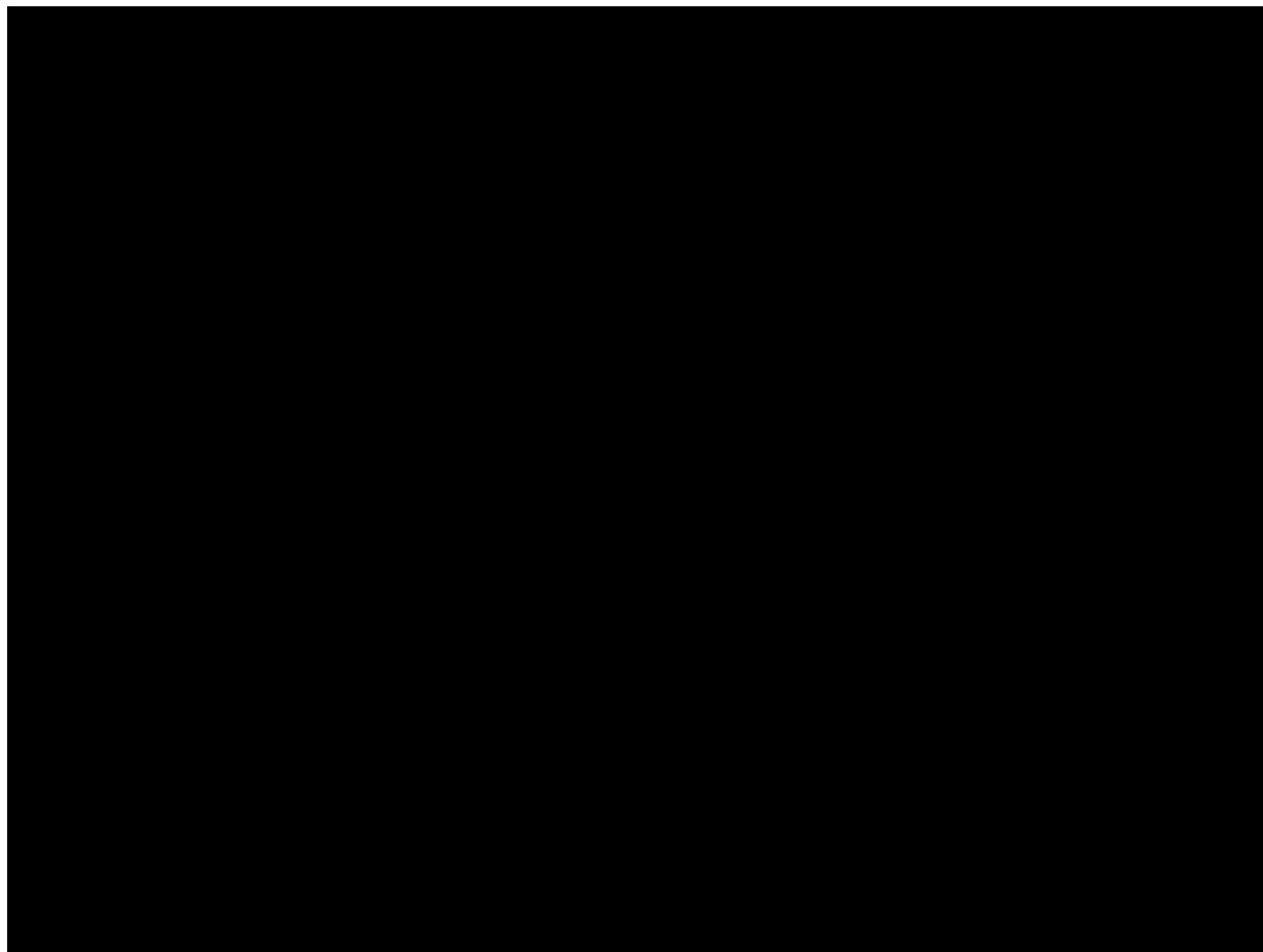
- **73%** of cancer medicines in the pipeline have the potential to be personalized medicines

**More than 800 medicines and vaccines are currently in clinical testing for cancer**

# PRESIDENT OBAMA FORMALLY ANNOUNCES PRECISION MEDICINE INITIATIVE



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# PRESIDENT OBAMA'S PRECISION MEDICINE INITIATIVE

- Announced on January 30, 2015
- **\$200 million** overall investment in FY2016 for NIH
- **\$70 million** investment to NCI to scale up efforts to identify genomic drivers in cancer and apply this knowledge to more effective approaches to cancer treatment
- **\$130 million** to NIH to develop a voluntary national research cohort of over one million volunteers to advance our understanding of health and disease; engage research cohorts, patient groups, and the private sector in partnerships to expand genomics; and maximize open data sharing



AACR Junior Champion in Cancer Research Award Recipient Elana Simon and President Barack Obama Announce the Initiative, 1-30-15

# SMART PUBLIC POLICIES ARE NECESSARY TO CONTINUE THE MOMENTUM

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# FDA-APPROVED MOLECULARLY TARGETED AGENTS IN 2015

Five new anticancer therapeutics approved:

- **Palbociclib (Ibrance)**  
Certain type of breast cancer
- **Sonidegib (Odomzo)**  
Most common type of skin cancer
- **Lenvatinib (Lenvima)**  
Certain type of thyroid cancer
- **Panobinostat (Farydak)**  
Multiple myeloma
- **Dinutuximab (Unituxin)**  
Neuroblastoma

Two new indications for previously approved therapeutics:

- **Gefitinib (Iressa)**  
Certain type of lung cancer
- **Ibrutinib (Imbruvica)**  
Certain type of non-Hodgkin lymphoma

Exciting research is underway  
to identify novel ways to  
expedite cancer drug  
discovery and development

**This is precision  
medicine in action!**



# AACR-ADVAMEDDX SYMPOSIUM (OCT. 29, 2013)

**AACR** American Association  
for Cancer Research  
FINDING CURES TOGETHER™





- *Transforming Cancer Care Through Diagnostics and Personalized Medicine*
- Importance of diagnostics in personalized cancer care.
- Regulatory and reimbursement policy challenges and opportunities.
- Opening keynote - Harold E. Varmus, MD
- Lunch conversation between Francis S. Collins, MD, PhD and Margaret A. Hamburg, MD moderated by Charles L. Sawyers, MD

# AACR ISSUES A POLICY STATEMENT ON LAB-DEVELOPED TESTS - SEPT. 9, 2014

**AACR** American Association  
for Cancer Research  
FINDING CURES TOGETHER™

Published OnlineFirst September 9, 2014



# CONGRESSIONAL HEARING ON LABORATORY DEVELOPED TESTS

- On Sept. 9, 2014, AACR President Charles Sawyers testified before the House Energy and Commerce committee and reiterated the AACR's position on the regulation of Laboratory Developed Tests (LDTs).
- The AACR policy statement, co-written by Dr. Sawyers and Dr. Laura van 't Veer, was published in *Cancer Clinical Research* on September 9, 2014.
- The AACR believes that a unified regulatory framework for all diagnostic tools, including LDTs, is critical to ensuring patient safety and to spurring innovation on a national and global level.



Image: Dr. Sawyers testifying before the U.S. House Energy and Commerce committee on September 9<sup>th</sup>, 2014



# DIAGNOSTICS AND PRECISION MEDICINE BRIEFING ON JUNE 8, 2015

AACR and AdvaMedDx cohosted this Congressional briefing on June 8, 2015, to discuss the leading role diagnostics have in precision medicine and how President Obama's Precision Medicine Initiative will revolutionize our understanding and treatment of diseases.



# PRESIDENT OBAMA'S PRECISION MEDICINE INITIATIVE

- Announced on January 30, 2015
- **\$10 million** investment to FDA for the development of databases to support the regulatory structure needed to advance innovation in precision medicine and public health



# REGULATORY SCIENCE AND POLICY PRIORITIES AND ACTIVITIES

- Sponsored FDA Workshops
  - FDA-AACR-ASCO workshop on Harmonizing Companion Diagnostic Development (across class of targeted therapies, such as PDL1) – March 24, 2015
  - FDA-AACR Workshop on Dose Finding (optimizing early-phase dosing for developing the next generation of precision oncology medicines) – May 18-19, 2015
  - 2016 FDA-AACR Workshops – Liquid Biopsies, Immunotherapy, and a second workshop on Dose Finding are planned
- AACR Science Series at the FDA
- Regulatory Science and Policy Track at AACR Annual Meeting
- AACR Partnerships with European organizations

# COMPLEXITIES IN PERSONALIZED MEDICINE: HARMONIZING COMPANION DIAGNOSTICS ACROSS A CLASS OF TARGETED THERAPIES



Co-chairs (L-R): Drs. van 't veer,  
Mansfield, and Schilsky

On March 24, 2015, FDA, AACR, and ASCO sponsored a workshop that examined challenges in the development and use of multiple targeted therapy-companion diagnostic pairs directed at the same or similar biomarker(s).

A blueprint proposal from six industry stakeholders to examine performance characteristics of tests was released at the workshop.



# DOSE-FINDING OF SMALL MOLECULE ONCOLOGY DRUGS

On May 18-19, 2015, FDA and AACR cosponsored a public workshop titled “Dose-finding of Small Molecule Oncology Drugs.”

The two-day workshop provided an interdisciplinary forum to discuss the best practices of dose finding and dose selection for small molecule kinase inhibitors developed for oncology indications.



# 2015 REGULATORY SCIENCE AND POLICY TRACK

The Role of Neo-Adjuvant Treatment in Drug Development for Solid Tumors	Clinical Trial Enrichment Strategies: Designing a Prospectively Defined Retrospective Analysis Study
Evolution of Personalized Drug Development in Oncology-Kinase Inhibitors	Integrating Patient Reported Outcomes (PROs) into Regulatory Evaluation
New Drugs: Breakthrough Therapies and Beyond	FDA's Proposed Framework to Regulate Laboratory Developed Tests (LDTs)
Regulatory Strategies in Oncology Drug Development	The Past, Present, and Future of FDA Regulation of Next Generation Sequencing (NGS)
Incorporating Next-Generation Sequencing (NGS) into Oncology Diagnostics, Therapeutics, and Treatment	



# 2015 AACR-NCI-EORTC INTERNATIONAL CONFERENCE ON MOLECULAR TARGETS AND CANCER THERAPEUTICS

**AACR** American Association  
for Cancer Research  
FINDING CURES TOGETHER™

- The premier annual international drug development meeting, featuring novel targets, cancer therapeutics, and immunotherapeutics
- November 5-9, 2015, Boston, MA
- Levi A. Garraway, MD, PhD (AACR), Lee J. Helman, M.D. (NCI), Jean-Charles Soria, MD (EORTC), Co-chairs
- Projected attendance: >2,600
- >50% of attendees are from industry

SAVE THE DATE!

AACR-NCI-EORTC International Conference on

**MOLECULAR TARGETS AND  
CANCER THERAPEUTICS**

The Premier International Meeting Featuring Novel Cancer Therapeutics

November 5-9, 2015 • Hynes Convention Center • Boston, MA

*We look forward to seeing you in Boston!*

**ORGANIZING COMMITTEE**

 Carlos L. Arteaga, MD  
Vanderbilt-Ingram Cancer Center,  
Nashville, TN (AACR)

 James H. Doroshow, MD  
National Cancer Institute-DCTD,  
Bethesda, MD, USA (NCI)

 Roger Stupp, MD  
University Hospital Zurich (USZ),  
Zurich, Switzerland (EORTC)

**SCIENTIFIC COMMITTEE CO-CHAIRPERSONS**

 Levi A. Garraway, MD, PhD  
Dana-Farber Cancer Institute,  
Boston, MA, USA (AACR)

 Lee J. Helman, MD  
National Cancer Institute,  
Bethesda, MD, USA (NCI)

 Jean-Charles Soria, MD, PhD  
Institut Gustave-Roussy,  
Villejuif, France (EORTC)

**ABOUT THIS CONFERENCE**

Join us for the premier international meeting featuring novel cancer therapeutics. This meeting brings together nearly 3,000 academics, scientists, and pharmaceutical industry representatives from across the globe to discuss innovations in drug development, target selection, and the impact of new discoveries in molecular biology.

**WHAT PAST ATTENDEES SAID...**

"Very exciting conference with excellent speakers. Great opportunity to broaden my knowledge of cancer biology and therapeutics."

"I felt stimulated to work in cancer and science again! Thank you!"

For more information and to bookmark this important event, go to [www.AACR.org/Targets15](http://www.AACR.org/Targets15)

AACR American Association for Cancer Research  
NCI NATIONAL CANCER INSTITUTE  
EORTC European Organization for Research and Treatment of Cancer



# A NEW COLLABORATION WITH THE EORTC AND EMA

## EORTC-NCI-EMA-AACR International Conference on Innovation and Biomarkers in Cancer Drug Development

- December 3-4, 2015, Brussels, Belgium
- To be held every other year in the U.S. with the FDA, to be named “AACR-NCI-FDA-EORTC”
- Meeting in the U.S. to take place in December 2016

The screenshot shows the official website for the IBCD 2015 conference. The header includes navigation links: IBCD, Faculty, Program, Accreditation, Registration, Abstracts, Sponsorship & Exhibit, and Hotel. The main banner features the title "Innovation and Biomarkers in Cancer Drug Development" and the dates "3-4 December 2015, Brussels, Belgium". Logos for EORTC, National Cancer Institute, European Medicines Agency, and AACR are displayed. The page content includes a "Dear Colleagues" message, a "Registration deadlines" section with dates for opening, early, and late registration, an "Abstract deadline" section with submission dates, and a "More events" section listing a joint meeting by ASCO, EORTC & NCI.

IBCD Faculty Program Accreditation Registration Abstracts Sponsorship & Exhibit Hotel

### Innovation and Biomarkers in Cancer Drug Development

3-4 December 2015, Brussels, Belgium

EORTC NATIONAL CANCER INSTITUTE EUROPEAN MEDICINES AGENCY AACR

Dear Colleagues,

We are honored to be able to invite you to attend IBCD 2015, **Innovation and Biomarkers in Cancer Drug Development**, a first of a kind joint meeting organized by the European Organisation for Research and Treatment of Cancer (EORTC), an academic research organization, the US National Cancer Institute (NCI), a governmental institution, the European Medicines Agency (EMA), a regulatory agency, and the American Association for Cancer Research (AACR), a professional scientific association.

Never before has cancer drug and biomarker development been so complex and required such a diverse range of expertise, and IBCD 2015 will help forge the collaborations needed for developing improved treatments for patients with cancer. The intention is to shine a spotlight on multi-stakeholder approaches to cancer drug development with new cancer biomarkers in a scientific program which includes input from regulators, industry, academia, patients, and payers.

The combined efforts of the EORTC, NCI, EMA, and AACR have resulted in a unique program. The opening session will address quality assurance and quality control (QA & QC) from pre-analytical steps to clinical utility and focus on quality management along the pathway to health care implementation as well as in the collection and management of biospecimens for clinical trials biobanking. It will also address standardization and comparison of next generation sequencing (NGS) pipelines and also the question of when, with respect to QA & QC, is an assay ready.

This session transitions naturally into subsequent presentations concerning assay development and their integration into drug development with the intention of stimulating the dialog between those conducting clinical trials and those working in the laboratory. A panel discussion will look into the topic of patient access and regulatory challenges for clinical trials in the era of molecularly defined personalized therapies and include short presentations on existing molecular screening platforms and the US and European regulatory bodies. The panel will include key opinion leaders representing regulators, payers, industry, patients, and academia.

Bioinformatic approaches to big data analysis and the clinical decision process will be scrutinized based on concrete examples for treatment decision as well as identification of recurrent patterns in cancer using big data.

A second panel discussion will look into the impact on health care systems, a multi stakeholder societal challenge for research, pharma and patients. This session will have a debate format, a point-counter point style, and arguments will be made concerning value, macro-economic sustainability, regulatory systems, and pricing and reimbursement in the context of implementation of cancer precision medicine.

#### Registration deadlines

Opens on 21 May 2015  
Early from 21 May 2015 to 20 August 2015  
Late from 21 August 2015 to 12 November 2015

#### Abstract deadline

Submission from 13 April 2015 to 20 August 2015

#### More events

Also join the EORTC Regulatory Workshop (2 December 2015)

Markers in Cancer Diagnostic Development Tutorial, a joint meeting by ASCO, EORTC & NCI (1-2 December 2015)

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# HUMAN SUBJECT RESEARCH PROTECTIONS: UPDATING THE “COMMON RULE”

- Overall Goal: Enhancing protections for research subjects and reducing burden, delay, and ambiguity for investigators, institutions and IRBs
- The Notice of Proposed Rulemaking (NPRM) requests input on:
  - Research use of identifiable private information & biospecimens
  - Calibrating the review process
  - Exclusions and exemptions
  - Improve informed consent process & privacy safeguards
  - Single IRBs
  - Expanding the scope of the rule

# ANNUAL TURNING THE TIDE CONFERENCE



Co-convoked by: **AACR** American Association  
for Cancer Research  
FINDING CURES TOGETHER™

**PMC**  
PERSONALIZED  
MEDICINE COALITION  
Celebrating 10 Years

Feinstein Kean Healthcare  
An Ophry Company



- Advancing patient-centric research and care
- Innovative drug development; incorporation of patient needs
- The urgency to improve public health, and the need to reduce health care costs, requires support for innovative, transformative research

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# PRESIDENT OBAMA'S PRECISION MEDICINE INITIATIVE

- Announced on January 30, 2015
- **\$5 million** to the Office of the National Coordinator for Health Information Technology to support the development of interoperability standards and requirements that address privacy issues and enable secure exchange of data across systems

# AACR PROJECT GENIE

AN EXCITING NEW DATA-SHARING INITIATIVE TO SUPPORT  
THE IMPLEMENTATION OF PRECISION MEDICINE

## Genomics; Evidence; Neoplasia; Information; Exchange (GENIE)

### VISION

A scalable platform to facilitate sharing of genomic and clinical outcomes data to catalyze the full implementation of precision cancer medicine leading to:

- Drug repositioning
- Development of new precision anticancer therapeutics
- Proof of the clinical utility of large-scale sequencing efforts, which should lead to insurance coverage and broader access.

### PILOT PROJECT

An innovative network of seven national and international academic cancer centers that will share genomic and clinical data over a five-year period to answer novel clinical questions. First major meeting took place on July 15-16, 2015, at the AACR Office, Charles L. Sawyers, MD, Chairperson, with 26 participants. Formal announcement will be made in early November at the AACR-NCI-EORTC Molecular Targets and Therapeutics meeting in Boston

### WHY NOW?

Many cancer centers and academic medical centers are actively engaged in independent large-scale clinical sequencing efforts, but still lack sufficient data to effectively make clinical decisions based on these efforts. Pooling data is viewed as essential and synergistic to this goal.

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# PAYING FOR PERSONALIZED MEDICINE/REIMBURSEMENT POLICIES

- Personalized medicine – shifts emphasis from reactive to preventive
- Personalized medicine – controls costs through making it more effective and efficient, while reducing trial-and-error prescribing
- Fair reimbursement for diagnostic tests is vitally needed
- Jan., 2015, Department of HHS Secretary Sylvia Burwell announces goal of tying 50 percent of traditional, or fee-for-service, Medicare payments to quality or value Alternative Payment Models by the end of 2018
- Payers will require evidence-based examples of successes, as high cost of cancer treatment currently exists
- Lack of access to quality personalized cancer care exists for many Americans (awareness and education must increase)

# COLLABORATION IS THE CATALYST FOR ADVANCING PRECISION MEDICINE AND IMPROVING PATIENT CARE





# WORLD ECONOMIC FORUM

- The Global Agenda Council on Personalized & Precision Medicine
  - Presented the economic, medical and societal rationale for investing in personalized health.
  - Working on accelerating personalized health through data sharing, including collaborating with a larger information and communications technology project on “data commons” led by the World Economic Forum, highlighting the need for data interoperability.
  - Working to remove roadblocks to the implementation of personalized and precision health through a series of discussions as well as a policy paper on regulatory and payer harmonization across different geographies.



# AACR'S NICHE IN PRECISION CANCER MEDICINE

- AACR leadership in advocating for budget increases for the NIH, NCI and FDA
- AACR scientific journals that focus on this research area:  
*Cancer Discovery, Clinical Cancer Research, Molecular Cancer Therapeutics,*  
*and Cancer Immunology Research*
- AACR Clinical and Translational Cancer Research Committee focused on research and science policy areas that are key to advancing personalized cancer medicine
- AACR Regulatory Science and Policy Subcommittee of the Science Policy and Government Affairs Committee
- AACR sponsorship of the Annual Personalized Medicine Conference and partnership with the Personalized Medicine Coalition
- Strong translational focus at AACR Annual Meetings, Special Conferences, and mid-size meetings (e.g., the AACR-NCI-EORTC Molecular Targets and Therapeutics Meeting series)
- Regular interactions with AACR's industry partners

THANK YOU!

Thank you again for your kind invitation to participate in  
this important meeting.