PARTNERS IN PROGRESS: CANCER PATIENT ADVOCATES AND THE FDA

A Workshop Sponsored by the FDA’s Oncology Center of Excellence, with Support from AACR, ASCO, and ASH
November 13, 2017

Jon G. Retzlaff, MBA, MPA
Chief Policy Officer
Vice President, Science Policy and Government Affairs
MY PRESENTATION TODAY

- Present an overview of the AACR
- Discuss how the AACR is working with advocates to accelerate progress and address the challenges in the field
- Summarize how patient advocates are playing a crucial role in our ongoing collaborative efforts to prevent and cure cancer.
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AACC VISION AND MISSION

To be the most effective catalyst for the cures and prevention of all cancers through:

- Research
- Education
- Communication
- Collaborations
- Funding for cancer research
- Advocacy

Founded 110 years ago, the AACR is the first and now the largest cancer research organization in the world dedicated to the conquest of cancer.
AACR membership includes more than 37,000 laboratory, translational, and clinical researchers; population scientists; other health care professionals; and patient advocates residing in 108 countries.

AACR annually convenes more than 30 conferences and educational workshops, the largest of which is the AACR Annual Meeting with more than 21,900 attendees.

AACR publishes eight prestigious, peer-reviewed scientific journals and a magazine for cancer survivors, patients, and their caregivers.

AACR funds meritorious research directly as well as in cooperation with numerous cancer organizations, including serving as the Scientific Partner of Stand Up To Cancer.

AACR actively communicates with legislators and other policymakers about the value of cancer research and related biomedical science in saving lives from cancer.
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The AACR Cancer Progress Report captures progress against cancer in a given year.

- Emphasizes how progress is benefiting cancer patients.
- Its principal goals are: scientific communication, education, and advocacy.
- The seventh edition was released in Washington, DC on September 13, 2017.
AACR CANCER PROGRESS REPORT 2017
RECENT PROGRESS IN THE CLINIC AGAINST CANCER

FROM AUGUST 2016 TO MAY 2017, THE FDA APPROVED:

8 new anticancer therapeutics

10 new uses for previously approved therapeutics

1st New approval for AML in 27 years.
Leading to the FDA approval of new therapeutics, including:

- A new PARP inhibitor, *niraparib*, for treating patients with advanced ovarian cancer, like **Teri Woodhull**

- A PDGFRA-targeted therapeutic, *olaratumab*, which is benefitting patients with soft tissue sarcoma, like **Evan Freiberg**
Leading to new and expanded uses of **checkpoint inhibitors**:

- **Pembrolizumab**, for treating patients like Adrienne Skinner, based on their tumor’s genetic characteristics, rather than site of origin.

- **Pembrolizumab**, for treating patients with head and neck cancer, like Bill McCone.

- **Avelumab**, as the first ever treatment option for patients with Merkel cell carcinoma, like Carrie Best.

Leading to the first **adoptive T-cell therapy (CAR-T)**, for kids and young adults with B-cell acute lymphoblastic leukemia.
On September 13, 2017, the AACR unveiled the seventh annual AACR Cancer Progress Report on Capitol Hill. Congressman Jamie Raskin (D-MD) and Senator Sherrod Brown (D-OH) delivered opening remarks during the briefing, which included individuals whose story was told in the Report, as well as Margaret Foti, PhD, MD (hc), AACR CEO; Michael A. Caligiuri, MD, AACR president and the director of the Ohio State University Comprehensive Cancer Center; and John D. Carpten, PhD, chair of the Department of Translational Genomics at the University of Southern California Keck School of Medicine.
Following the Capitol Hill briefing in 2016, the AACR leaders and survivors whose story was included in the Report met with Vice President Biden’s senior staff at the White House staff to deliver the report.

(And met President Obama’s dogs!)
On September 14, over 350 advocates from 40 states participated in 242 meetings on Capitol Hill, where they urged Congress to continue robust, sustained and predictable funding increases for the NIH.
The AACR Scientist ↔ Survivor Program was launched in 1999 at the AACR Annual Meeting. It is a unique program designed to build bridges and unity between the leaders of the scientific and cancer survivor and patient advocacy communities. The program aims to develop collaborations among scientists and advocates to address issues in survivorship, quality of life, and science and public policy. It also seeks to increase participation in clinical trials, improve the design of clinical trials, and increase the number of effective cancer drugs. Additionally, the program facilitates access to cancer information for the general public, high-risk individuals, and minority and medically underserved populations.
AACR encourages SSP members to participate in various advocacy-related initiatives and activities, including Congressional briefings, the Rally for Medical Research Hill Day, and FDA-AACR co-sponsored workshops, as well as AACR Regulatory Science and Policy Sessions that occur at many of the annual AACR conferences and meetings.
SSP participants annually meet with FDA leaders in a special session at the AACR Annual Meeting.
On April 4, 2017, at the AACR Annual Meeting, AACR leaders and SSP participants attended and participated in a Congressional briefing.
Regulatory Science and Policy Track
at the 2017 AACR Annual Meeting

Understanding Mechanism-based, Cardiovascular Adverse Events Associated with Immune Checkpoint Blockade: Implications for Prevention and Management
Laleh Amiri Kordestani (Co-chair) – FDA
Javid Mosleh (Co-chair) – Vanderbilt Univ.
George Demetri – Dana-Farber Cancer Institute
David Feltquate – Bristol-Myers Squibb
Shiv Pillai – Harvard Medical School
Suzanne Topalian – Johns Hopkins Kimmel Cancer Ctr.

Real World Evidence in Oncology and its Implications
Amy Abernethy (Chair) – FlatIron Health
Kassa Ayalew – FDA
Sean Khizin – FDA
Jeff Allen – Friends of Cancer Research
Jacqueline Law – Genentech, Inc.
Raymond DuBois – Medical Univ. South Carolina

Tables Turned: A Conversation with the Press about the Future of Cancer Research and Treatment
Richard Pazdur (Chair) – FDA
Adam Feuerstein – The Street
Matthew Herper – FORBES
Laurie McGinley – The Washington Post
Meg Tirrell – CNBC

Regulatory Considerations for Utilizing Liquid Biopsies in Drug and Diagnostic Development
Pasi Jänne (Co-chair) – Dana-Farber Cancer Inst.
Gideon Blumenthal (Co-chair) – FDA
Reena Philip (Co-chair) – FDA
Abraham Tzou – FDA
Suzanne Jenkins – AstraZeneca
Gary Kellogg – National Cancer Institute
Walter Koch – Roche
Howard Scher – Memorial Sloan Kettering Cancer Ctr.
Phil Stephens – Foundation Medicine
AmirAli Talasaz – Guardant Health

Immuno-oncology Combination Therapies
Geoffrey Kim (Chair) – FDA
Chao Liu – FDA
Amy Rosenberg – FDA
Daniel Chen – Genentech, Inc.
Bernard Fox – Earle A. Chiles Research Inst.
Elizabeth Jaffee – Hopkins Kimmel Cancer Ctr.
Sreeneeranj Kasichayanula – Amgen, Inc.

New Drugs – A Review of Recently Approved Breakthrough Therapies
Amy Mckee (Chair) – FDA
Sanjeeve Balasubramaniam – FDA
Leslie Doros – FDA
Daniel Suzman – FDA
Deborah Armstrong – Hopkins Kimmel Cancer Ctr.
Dan Theodorescu – Univ. of Colorado Cancer Ctr.
Katie Thornton – Dana-Farber Cancer Institute

Advancing Clinical Trial Design in Regulatory Science and Policy*
Lisa LaVange (Co-chair) – FDA
Lillian Siu (Co-chair) – Princess Margaret Cancer Centre

Reference Materials for Next Generation Sequencing (NGS)-based Tests
Elaine Mardis (Co-chair) – Nationwide Children’s Hosp.
David Litwack (Co-chair) – FDA
Zivana Tezak – FDA
Mary Ellen de Mars – ATCC
Girish Puthia – Freenome
Marc Salt – NIST
Kenna Mills Shaw – MD Anderson Cancer Ctr.
Jeffrey Trent – TGen

Recent Trends in Regulatory Science*
Julia Beaver (Co-chair) – FDA
Martha Donoghue (Co-chair) – FDA

*minisymposia
The Potential Impact on Cancer Patients of a Repeal or Revision of the Affordable Care Act

**Gilbert S. Omenn.** Univ. of Michigan, Ann Arbor, MI (Chair)

Diana Chingos. Patient Advocate, Los Angeles, CA

Chiara D`Agostino. Patient Advocate, Montclair, NJ

Ernest T. Hawk, MD, PhD, MD Anderson Cancer Center

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E-cigarettes: Are they a Public Health Threat or a Useful Cessation Tool?

**Benjamin A. Toll.** Yale Cancer Center, New Haven, CT (Chair)

Roy S. Herbst. Yale Cancer Ctr., New Haven, CT

Rachel Grana. National Cancer Institute, Bethesda, MD

Brian A. King. Centers for Disease Control and Prevention, Atlanta, GA

Lion Shahab. University College London, London, United Kingdom
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PROGRESS REQUIRES COLLABORATIONS ACROSS AN ARRAY OF STAKEHOLDERS AND SECTORS

- Academic and government researchers from a diverse array of specialties;
- Health care providers;
- Regulators;
- Biotechnology, pharmaceutical, diagnostics, and medical device companies;
- Philanthropic organizations and cancer-focused foundations;
- Individual citizen advocates and members of advocacy groups;
- Federal funding organizations; and
- Payers.

- Patients, survivors, and their caregivers, family members, and friends;
- Policymakers;
Scientists and patient advocates, working together, can fundamentally change the face of cancer!!

Empower patients through active engagement in their own personal health care and shared decision making

Improve access of all patient populations to the entire continuum of quality cancer care

Improve clinical trial designs that allow for
  • Fewer patients in trials informed by genomics data
  • Better access to clinical trials, including for minorities and medically underserved or underrepresented groups
  • Patient-reported outcomes to assess the overall risk-benefit profile

Advocate for regulatory policies that speed new drug approvals
IN SUMMARY: THE BASIS FOR CONTINUED EXTRAORDINARY PROGRESS

- New technologies have enabled our current deeper understanding of the biology of cancer and offered opportunities in imaging, high-throughput screenings for drug discovery, precision medicine, and cancer interception.
- The private sector is now developing novel compounds that alter the relevant biological proteins and processes and that are more effective than standard of care.
- Colleagues in the government sector are expediting approvals while ensuring the safety and efficacy of products.
- Expert health care providers are giving patients the best personalized care possible.
- Patients are giving selflessly with tissues and data to advance progress.

Advocates are playing a crucial role in educating patients about their options, serving on committees and thereby contributing to clinical research and regulatory policy, increasing awareness among legislators and the general public, and raising precious philanthropic funds for cancer research.
ARE CANCER DRUGS GETTING BETTER?

- Drug development decisions are being made based on the molecular basis of the disease.
- “Drugs in the recent years have had exceptional response rates that we have not observed before. . . The question is not whether we (the FDA) should approve the drug, but how quickly we can approve the drug.”
- This represents a new approach on the part of FDA and is the result of the new cancer science and of FDA leadership.

Richard Pazdur, MD, Director, Oncology Center of Excellence, FDA. The Cancer Letter, 2-14-14
THANK YOU FOR YOUR KIND ATTENTION!