Cancer Moonshot
Blue Ribbon Panel Report 2016

National Cancer Advisory Board Meeting
September 7, 2016

Liz Jaffee | Tyler Jacks | Dinah Singer
Goals of the Cancer Moonshot

- Accelerate progress in cancer, including prevention & screening
  - From cutting edge basic research to wider uptake of standard of care
- Encourage greater cooperation and collaboration
  - Within and between academia, government, and private sector
- Enhance data sharing

(Presidential Memo 2016)
Federal Task Force Goals

- Accelerate our understanding of cancer, its prevention, early detection, treatment and cure;
- Support greater access to new research, data, and computational capabilities;
- Improve patient access and care;
- Identify and address any unnecessary regulatory barriers and consider ways to expedite administrative reforms;
- Identify opportunities to develop public-private partnerships and increase coordination of the Federal Government’s efforts with the private sector, as appropriate.

(Presidential Memo 2016)
Blue Ribbon Panel

- “The Blue Ribbon Panel … will provide expert advice on the vision, proposed scientific goals, and implementation of the National Cancer Moonshot. ..The panel may also recommend other cancer research activities to enhance this effort.

- “The Panel will provide an intensive examination of the opportunities and impediments in cancer research… the Panel may call upon special consultants, assemble ad hoc work groups, convene workshops, and conduct other activities. Findings and recommendations of the Panel will be reported to the NCAB.

- “The NCAB will use the Panel’s findings and recommendations to provide final recommendations to the NCI Director.”

(Presidential Memo 2016)
## Blue Ribbon Panel

<table>
<thead>
<tr>
<th>Member</th>
<th>Institution/Position</th>
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<tbody>
<tr>
<td>Tyler Jacks*</td>
<td>MIT</td>
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<tr>
<td>Peter C. Adamson, M.D.*</td>
<td>Children's Hospital of Philadelphia</td>
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<tr>
<td>James Allison</td>
<td>MD Anderson</td>
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<td>David Arons</td>
<td>National Brain Tumor Society</td>
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<td>Mary Beckerle</td>
<td>Univ. of Utah</td>
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<td>Mitchel Berger*</td>
<td>UCSF</td>
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<td>Jeffrey Bluestone</td>
<td>Parker Institute</td>
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<td>Chi Dang*</td>
<td>U. Penn</td>
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<td>Mikael Dolsten</td>
<td>Pfizer</td>
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<td>Co-Chairs</td>
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<td>Elizabeth Jaffee*</td>
<td>Johns Hopkins</td>
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<td>James Downing</td>
<td>St. Jude Hospital</td>
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<td>Levi Garraway</td>
<td>Harvard Medical School</td>
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<td>Gad Getz</td>
<td>Broad Institute</td>
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<td>Laurie Glimcher</td>
<td>Weill Cornell</td>
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<td>Lifang Hou</td>
<td>Northwestern</td>
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<td>Neal Kassell</td>
<td>Univ. Va.</td>
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<tr>
<td>Elena Martinez*</td>
<td>UCSD</td>
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<td>Deborah Mayer</td>
<td>UNC</td>
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<td>Edith Mitchell</td>
<td>Thomas Jefferson Univ.</td>
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<td>Dinah Singer</td>
<td>NCI</td>
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<td>Augusto Ochoa</td>
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<td>Jennifer Pietenpol</td>
<td>Vanderbilt Univ.</td>
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<td>Angel Pizzaro</td>
<td>Amazon Web Services</td>
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<td>Barbara Rimer</td>
<td>UNC</td>
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<td>Charles Sawyers*</td>
<td>MSK</td>
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<td>Ellen Sigal</td>
<td>Friends of Cancer Research</td>
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<td>Patrick Soon-Shiong</td>
<td>NantWorks</td>
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<td>Wai-Kwan Alfred Yung</td>
<td>MD Anderson</td>
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*NCAB/BSA member
Charge to Blue Ribbon Panel

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(Presidential Memo 2016)
## BRP Working Groups

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<thead>
<tr>
<th>Working Group</th>
<th>Co-Chair</th>
<th>NCI Staff</th>
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<tbody>
<tr>
<td><strong>Cancer Immunology</strong></td>
<td>Liz Jaffee, Jim Allison</td>
<td>Toby Hecht, Kevin Howcroft</td>
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<tr>
<td><strong>Precision Prevention and Early Detection</strong></td>
<td>Mary Beckerle, Jennifer Pietenpol</td>
<td>Elisa Woodhouse, Tracy Lively</td>
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<td><strong>Tumor Evolution</strong></td>
<td>Chi Dang, Levi Garraway</td>
<td>Joanna Watson, Suresh Mohla, Tony Dickherber</td>
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<td><strong>Clinical Trials</strong></td>
<td>Charles Sawyers, Mitch Berger</td>
<td>Jeff Hildesheim, Meg Mooney</td>
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<tr>
<td><strong>Implementation Sciences</strong></td>
<td>Elena Martinez, Augusto Ochoa</td>
<td>Bob Croyle, Worta McCaskill-Stevens</td>
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<td><strong>Pediatric Cancer</strong></td>
<td>Peter Adamson, Jim Downing</td>
<td>Judy Mietz, Malcolm Smith</td>
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<tr>
<td><strong>Enhanced Data Sharing</strong></td>
<td>Angel Pizarro, Gaddy Getz</td>
<td>Juli Klemm, Betsy Hsu, Jennifer Couch</td>
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Scientific and Community Outreach Activities

Goal:
- Provide opportunities for the public and experts ways to submit ideas
- Increase the public’s participation in the Cancer Moonshot

Approaches:
- Online public idea repository – over 1600 submissions
- One-on-one public input: email
- BRP Listening sessions
- Professional conferences
Overview of Blue Ribbon Panel Report

- The 7 Working Groups submitted a total of 14 recommendations
- All 14 were discussed at the July 20 meeting of the Blue Ribbon Panel
  - Thirteen were approved as “Moonshot recommendations”
  - One recommendation was converted to a demonstration project
Overview of Blue Ribbon Panel Report

- For purposes of the printed report, some recommendations were combined:
  - Pediatrics Cancer and Tumor Evolution Working Groups both recommended efforts to identify new therapeutic targets to overcome cancer resistance.
  - Tumor Evolution, Cancer Immunology and Precision Prevention/Early Detection Working Groups proposed generation of human atlases of cancer.
  - Both the Precision Prevention/Early Detection and Implementation Science Working Groups focused on the importance of screening.
  - Development of technologies cited throughout the recommendations was combined into a single recommendation.
Overview of Blue Ribbon Panel Report (Cont.)

- The printed Report summarizes the recommendations of exceptional research opportunities that could lead to powerful advances in our understanding of cancer

- Three sidebars highlight proposed demonstration projects

- The online Report will include all recommendations in their entirety
Cross-Cutting Themes

- National network of patient biological and clinical data
- Prevention
- Health disparities research
- Biomarkers
- Development of technology and preclinical models
- Data sharing, analytics and predictive computational modeling
- Collaboration; public-private partnerships
Summary of the Recommendations

A. Network for direct patient engagement:
   • Enlist patients in federated network where patients can “pre-register” for clinical trials and contribute their tumor profile data to expand knowledge about what therapies work and in whom.

B. Cancer immunotherapy clinical trials network.
   • Organize a network to discover and evaluate novel immune-based approaches for adult and pediatric cancers, and eventually develop vaccines.

C. Therapeutic target identification to overcome drug resistance.
   • Launch interdisciplinary studies to delineate mechanisms that lead cancer cells to become resistant to previously effective treatments.

D. Creation of a national cancer data ecosystem.
   • Create an ecosystem to collect, share, and interconnect datasets.
Summary of the Recommendations (continued)

E. Fusion oncoproteins in pediatric cancer.
   • Improve understanding of the abnormal fusion proteins that result from chromosomal translocations and drive many pediatric cancers.

F. Symptom management research.
   • Support research to accelerate development of guidelines for management of patient-reported symptoms to improve quality of life and adherence to treatment regimens.

G. Precision prevention and early detection:
   • Implementation of evidence-based approaches. Conduct implementation science research to encourage broader adoption of HPV vaccination, colorectal cancer screening, and tobacco cessation.
Summary of the Recommendations (continued)

H. Retrospective analysis of biospecimens from patients treated with standard of care.
   • Analyze biopsies to learn which features predict outcome to better plan treatment for future patients.

I. Creation of human tumor atlas.
   • Catalog the evolution of genetic lesions and cellular interactions in tumor/immune/other cells in tumor microenvironment from the earliest detected lesions to metastasis

J. Development of new enabling technologies.
   • Support development of technologies to accelerate testing of therapies and tumor characterization.
Summary of the Demonstration Projects

Prevention: Lynch Syndrome Demonstration Project
• A national effort to systematically screen all CRC and endometrial cancer patients for Lynch syndrome (LS)
• First degree relatives of patients with LS would be given the option to be screened and provided with genetic counseling

Therapy: Pediatric Cancer Immunotherapy Network Demonstration Project
• A national pediatric immunotherapy clinical trials network to facilitate the testing of new immunotherapy approaches in childhood cancer
• Establish a robust research pipeline to advance pediatric immunotherapy

Emergent Technologies: Tumor Pharmacotyping Demonstration Project
• Develop intra- and extra-tumoral technologies for determining the most effective therapeutic agents for individual patients
Next Steps

- Acceptance of the Report by the NCAB

- Transmission of the Report to the Director, NCI.
  - The Report will advise NCI on future directions and programs that relate to the Cancer Moonshot

- Director, NCI, will forward the Report to the Vice President’s Cancer Moonshot Task Force, which will issue its report this fall

- Policy issues identified by the BRP as barriers (e.g. coverage and reimbursement; uniform informed consent) have been forwarded to the Task Force for consideration.
  - Implementation will depend on the extent to which these barriers are addressed.
Next Steps (continued)

- NCI will consider approaches for implementation of the recommendations
  - Identify those recommendations that are most feasible to implement in FY17
  - Determine which ongoing or planned NCI initiatives can be leveraged to advance the goals of the recommendations
  - New mechanisms (e.g. Other Transaction Authority) will be considered to enable efficient and effective implementation
  - Approaches to streamlining existing mechanisms (review, contracts) will be developed
  - Establish public-private partnerships and partnerships with other agencies
Next Steps (continued)

- Extent and rate of implementation will depend on Congressional appropriations
- NCI will look to its advisory boards and the Blue Ribbon Panel for advice in the implementation
- Continued investment in investigator-initiated research and research areas beyond the scope of the Blue Ribbon Panel remains a high priority
Acknowledgments

- Working Group members
- Doug Lowy, Jim Doroshow, Warren Kibbe, Anne Lubenow, Francis Collins, Larry Tabak
- NCI CRS – Diane Palmieri, Michelle Bennett
- NCI DCB - Chris Siemon, Karen Fleming, Jason Bunting
- NCI Program Staff: Kevin Howcroft, Elisa Woodhouse, Joanna Watson, Jeff Hildesheim, Meg Mooney, Judy Mietz, Malcolm Smith, Juli Klemm, Betsy Hsu, Jennifer Couch
- NCI OCPL - Becky Chasan, Nancy Murphy
Questions?