

Outcomes of the Cancer Moonshot: A Blue Print for Accelerating Progress

Elizabeth M. Jaffee

Co-Chair, Cancer Moonshot Blue Ribbon Panel

The Sidney Kimmel Cancer Center at Johns Hopkins

December 13, 2016

Vice President Biden's Moonshot and the Blue Ribbon Panel Recommendations

A roadmap to transform the unprecedented scientific progress of the past decade into meaningful interventions for patients with or at risk for cancer

Science is ripe but progress getting it to benefit patients is too slow and lacks efficient coordination

Importantly – Also Lacks the Level of Funding Needed to Get This Done!

GOALS OF THE CANCER MOONSHOT

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

(Presidential Memo 2016)

VICE PRESIDENT'S CANCER MOONSHOT WORKFLOW

Vice President's Office



Cancer Moonshot
Federal Task Force



NCI/NIH



National Cancer Advisory Board



"Blue Ribbon Panel"



Working Groups



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 to clinical trials and standard of care
- Identify and address any unnecessary regulatory barriers to drug development/approvals 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 – focus on drug development, treatment access and information systems

(Presidential Memo 2016)

FEDERAL TASK FORCE REPORT – STRATEGIC GOALS DIRECTLY IMPACT DRUG DEVELOPMENT

- **Catalyze new scientific breakthroughs**
 - ❖ Expand mobile device use and create tracking systems for patients
- **Unleash the power of data**
 - ❖ Best practices for consent, seamless data environment, open platforms, workforce development
- **Accelerate bringing new therapies to patients**
 - ❖ Modernize eligibility for clinical trials, develop disease site agnostic trials (eg MATCH), utilize real-world evidence
- **Strengthen prevention and diagnosis**
 - ❖ Improve HPV vaccination, smoking cessation strategies, expand CRC screening, screen environmental chemicals
- **Improve patient access and care**
 - ❖ Education, cancer survivorship, map cancer care across nation, develop virtual networks

CHARGE TO 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 ... Findings and recommendations of the Panel will be reported to the NCAB.”

(Presidential Memo 2016)

Tyler Jacks*
MIT

Co-Chairs
Elizabeth Jaffee*
Johns Hopkins

Dinah Singer
NCI

Peter C. Adamson, M.D.*
Children's Hospital of Philadelphia

James Allison
MD Anderson

David Arons
National Brain Tumor Society

Mary Beckerle
Univ. of Utah

Mitchel Berger*
UCSF

Jeffrey Bluestone
Parker Institute

Chi Dang*
U. Penn

Mikael Dolsten
Pfizer

James Downing
St. Jude Hospital

Levi Garraway
Harvard Medical School

Gad Getz
Broad Institute

Laurie Glimcher
Weill Cornell

Lifang Hou
Northwestern

Neal Kassell
Univ. Va.

Elena Martinez*
UCSD

Deborah Mayer
UNC

Edith Mitchell
Thomas Jefferson Univ.

Augusto Ochoa
Louisiana State Univ.

Jennifer Pietenpol
Vanderbilt Univ.

Angel Pizzaro
Amazon Web Services

Barbara Rimer
UNC

Charles Sawyers*
MSK

Ellen Sigal
Friends of Cancer Research

Patrick Soon-Shiong
NantWorks

Wai-Kwan Alfred Yung
MD Anderson

Blue Ribbon Panel Working Groups

- Seven Working Groups were formed
 - ❖ cancer immunology, clinical trials, implementation science, tumor evolution, precision prevention and early detection, pediatric cancer, data sharing
- Each Working Group had 12-15 members
- In total almost 150 individuals were engaged in the Working Groups, including academic researchers, clinicians, industry representatives and advocates
- Charge was to generate 2-3 recommendations of major scientific opportunities that are poised for acceleration
- The Working Groups met almost weekly to discuss and formulate their recommendations

WE ENGAGED ALL SECTORS OF THE SCIENTIFIC COMMUNITY

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
- One-on-one public input: email
- BRP Listening sessions
- Professional conferences

SUMMARY OF THE RECOMMENDATIONS

➡ A. Network for direct patient engagement

- ❖ Enlist patients in federated network that includes patient tumor profiling data and “pre-registers” patients for clinical trials

➡ B. Cancer immunotherapy translational science network

- ❖ Organize a network to discover and evaluate novel immune-based approaches for adult and pediatric cancers, and eventually develop vaccines for prevention

➡ 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

➡ 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 genetic lesions and cellular interactions in tumor/immune/other cells in tumor microenvironment

➔ **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 Translational Science Network Demonstration Project

- ❖ A national pediatric immunotherapy translational science 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

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

Improving the lives of patients is the major cross-cutting theme!

BRP DERIVED ONGOING INITIATIVES WITH IMPACT ON DRUG DEVELOPMENT

On-going NCI-Related Cancer Moonshot Activities

- **NCI Drug Formulary** – leveraging lessons learned from the MATCH Trial to forge relationships with 20-30 public-private partners to expedite researcher access to investigational agents and approved drugs for combination trials – reduce negotiation time – available soon
- **New approaches for disseminating clinical trials information** - Application Programming Interface (API) – first step in building an ecosystem - 3rd party innovators are taking part including Smart Patients, Synapse, Cure Forward, and Antidote to build applications, integrations, search tools, and digital platforms to disseminate clinical information to community
- **Strategic Computing Partnership between the DOE and the NCI to Accelerate Precision Oncology** – 3 new pilots to apply the most advanced supercomputing capabilities to analyze data from preclinical models, molecular interaction data for RAS and cancer surveillance data
 - ❖ 4 DOE labs – Argonne, Los Alamos, Lawrence Livermore, Oak Ridge + Frederick National Labs
 - ❖ Supported by the CANcer Distributed Learning Environment (CANDLE) DOE exascale computing project which will deliver new computing technologies
 - ❖ Partnership with Broad Institute, Institute for Systemic Biology, Seven Bridges Genomics

On-going NCI-Related Cancer Moonshot Activities

- **NCI partnership with NASA to study Particle Beam Radiotherapy**
- **Development of an Open Access Resource for Sharing Cancer Data via the Genomic Data Commons (GDC)**
 - ❖ Foundation Medicine is doubling the total number of patients
 - ❖ Providing a mechanism for broad sharing and Government/Academia/Industry partnerships
 - ❖ Complemented by NCI's Genomics Cloud Pilots which will expand to include other data sets – imaging, proteomics, immunotherapeutics
- **Public Private Partnership for Accelerating Cancer Therapies**
 - ❖ Collaborating with 12 biopharmaceutical companies, research foundations, philanthropies and the Foundation for the NIH
 - ❖ Partnership for Accelerating Cancer Therapies (PACT)
- **Applied Proteogenomics Organizational Learning Outcomes (APOLLO)**
 - ❖ Partnership between the DOE, NCI and VA
 - ❖ New technologies to rapidly identify new targets and pathways for detection and intervention

Planned NCI-Related Cancer Moonshot Activities

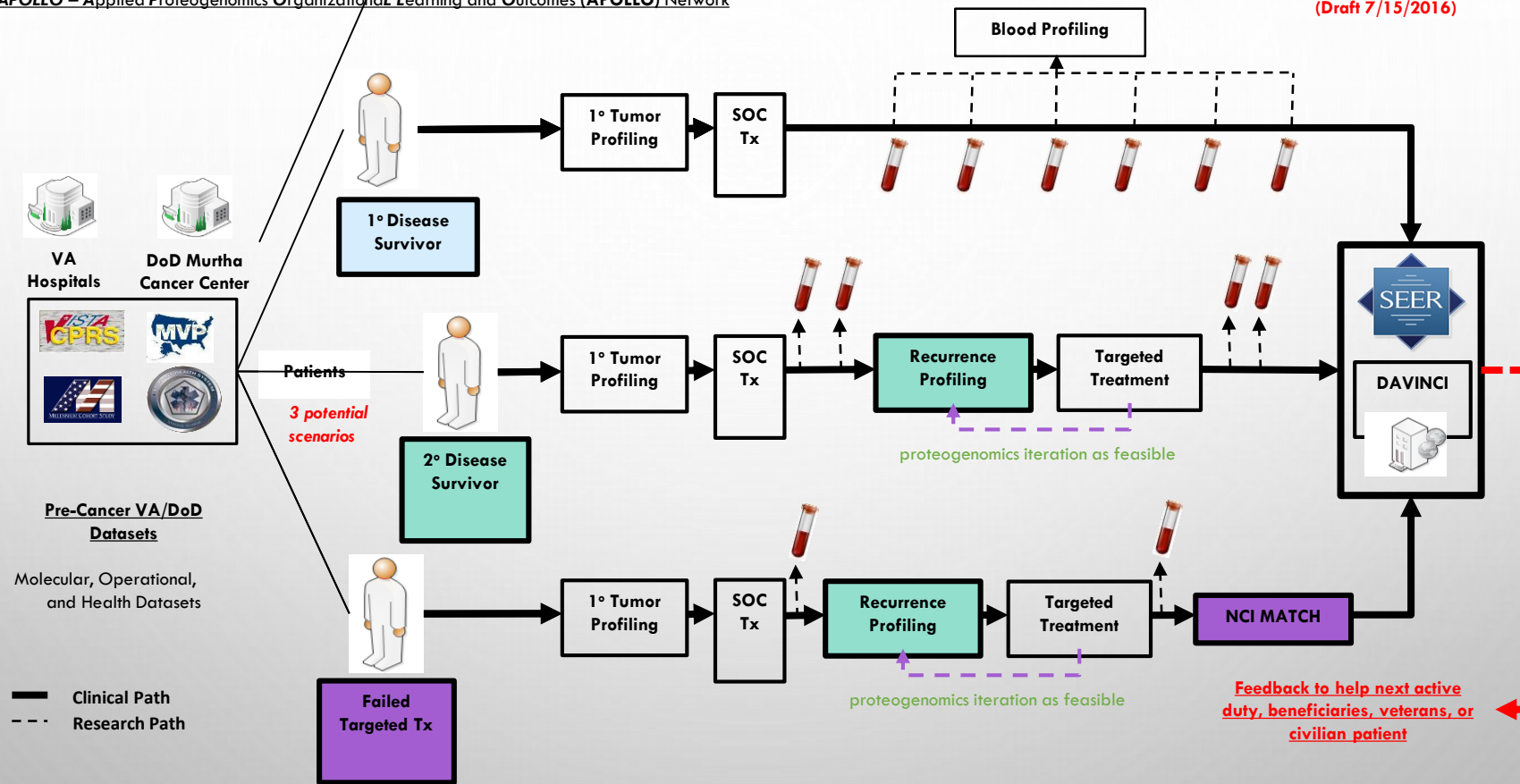
- **Develop predictive computer algorithms to rapidly develop, test and validate predictive preclinical models**
 - ❖ NCI and DOE partnership
 - ❖ Support treatment choices of physicians and patients
 - ❖ Develop set of models that can predict drug responses
 - ❖ Integrate mechanistic models and mechanism –based constraints into the machine learning framework
- **Build computational collaborative relationships with private sector and academia**
 - ❖ Partnership between U of CA, Intel, IBM, and GE working with DOE and NCI
 - ❖ Intersection of technology, cancer and data to support a virtual data ecosystem

Partnership for Accelerating Cancer Therapies (PACT)

- PACT will fund pre-competitive cancer research and share broadly all data generated for further research
- NIH is collaborating with 12 biopharmaceutical companies, multiple research foundations, philanthropies, and the Foundation for the NIH
- Potential initial focus areas include understanding responses to cancer therapies, clinical trial platforms for combination therapies, predictive modeling approaches, and therapies for rare cancers
- NIH identified two focus areas ideally suited to such a multi-sector and coordinated effort:
 - ❖ Identification and validation of biomarkers for response and resistance to cancer therapies, with a special emphasis on immunotherapies
 - ❖ Establishment of a platform for selecting and testing combination therapies using the collective “toolkit” of the pharmaceutical industry
- NIH will serve as a hub of coordinated efforts and information sharing across public and private sectors



10/18/16: Blood Profiling Atlas Face 2 Face



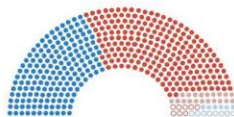
POLICY ISSUES IDENTIFIED AS BARRIERS BY THE BRP TO DRUG DEVELOPMENT

- Medical coverage and reimbursement
 - ❖ Costs of screening and preventive care not currently covered, including follow-up colonoscopy; reimbursement for referral, home-based care, clinical trials and non-physician health care providers
- Enhanced patient engagement
 - ❖ Design uniform informed consent, increase patient access to data
- Delivery of cancer care in the community
 - ❖ Reduce economic burden of clinical trial enrollment on community practitioner, sharing of electronic medical record data, best practices for state vaccine registries
- Improve clinical trials system
 - ❖ Central IRB, data sharing among federal agencies, improve clinical trial site evaluation
- Improve the outcome for children with cancer
 - ❖ Incentives for pediatric cancer drug development, especially molecularly targeted agents
- Develop data sharing mechanisms and incentives
 - ❖ Common ontologies, data standardization, umbrella licensing, private sector sharing



House roll call (592) vote on H.R.-34 21st Century Cures

If the legislation also passes in the Senate, **the president is expected to sign the bill**



House Votes by Party	Yes	No	Not Voting
Republicans	218	200	8
Democrats	174	6	8
Total	392	206	26

Sources: National Journal Research 2016

21st Century Cures:



- Increases NIH funding to aid: Precision Medicine Initiative, BRAIN Initiative, cancer research, regenerative medicine



- Implements mental health reform



- Funds state grants for opioid initiatives



- Funds FDA, implements FDA reforms intended to speed regulatory process



“ We’re closing in on what’s truly
*a once-in-a-generation
 transformational opportunity*
 to change the way
 we treat disease.”

- Chairman Fred Upton | 21st Century Cures

#CuresNow



Questions?



www.cancer.gov/brp