Patient Involvement in the Alliance for Digital Pathology

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FRIENDS of CANCER RESEARCH

The Roles of the Patient Community In Medical Product Development Are Far Reaching

- **Patients are uniquely equipped to identify critical gaps and unmet needs in their disease areas, and to advocate for and develop collaborative solutions to meet those needs**
  - **Advocacy**
  - **Funding**
  - **Policy**
  - **Regulation**
  - **Science**

“Nothing about us, without us”

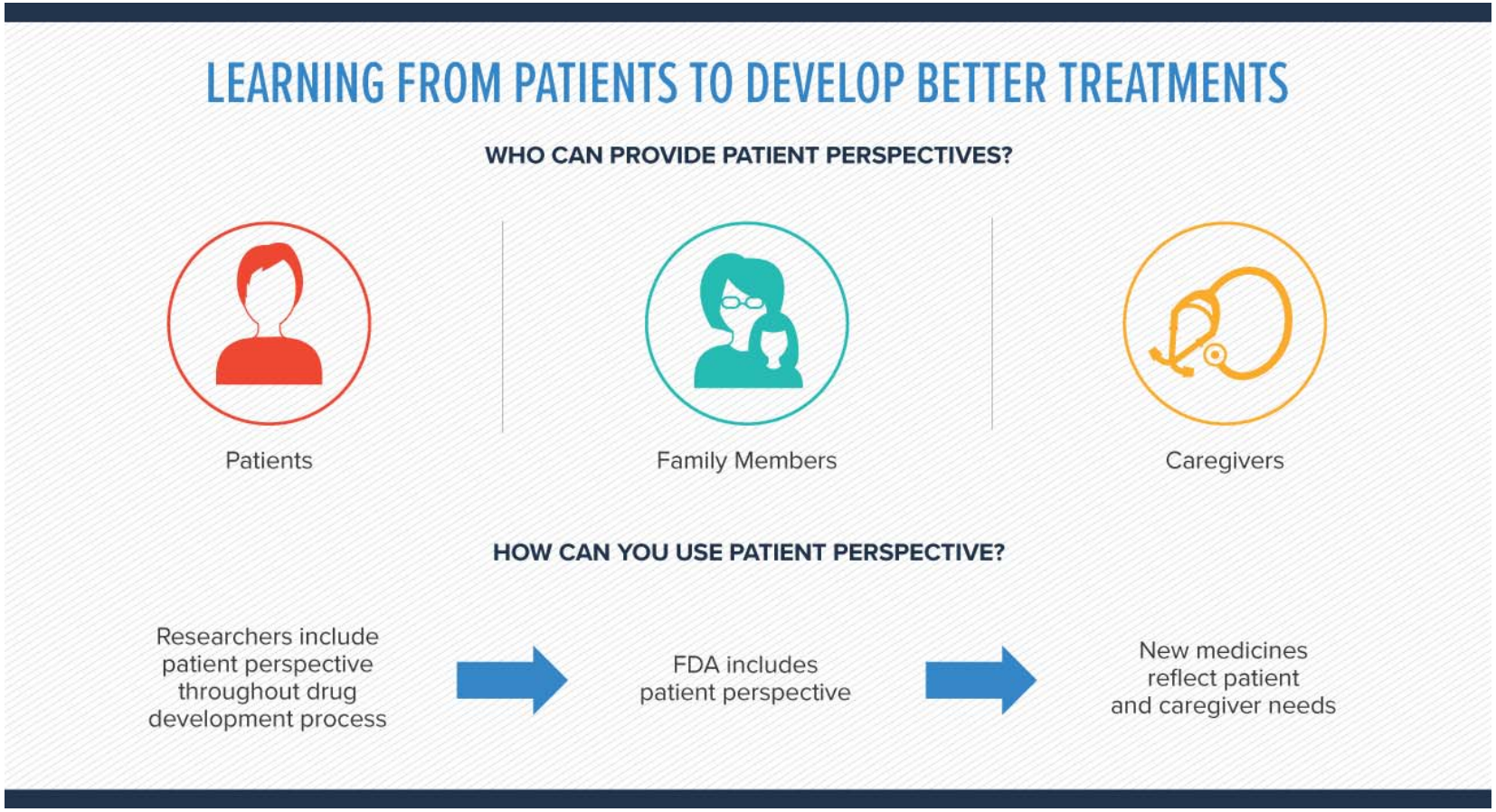
Patient groups not content to be merely cheerleaders for research, they want and need to be involved at all stages

Promoting Research through Legislation

- **User Fee Agreements (PDUFA/MDUFA/GDUFA/BSUFA)** – allows FDA to collect fees from sponsors to fund the review of new medical products
  - Re-authorized every 5 years – opportunity for the adoption of new legislation to improve or modernize components of the development and review process
  - **Stakeholders include patient groups, academics, and pharmaceutical/device companies, who meet publicly with FDA to determine the goals and provisions for each UFA reauthorization**
- **21st Century Cures Act**
  - Incorporate patient experience/patient reported outcomes in the drug development process
  - Accelerate discoveries, streamline drug and device development process (Breakthrough Device Designation), and facilitate use of digital medicine for treatment delivery

Promoting Research through Regulatory Policy

- FDA's **Patient Focused Drug Development Program**
  - Systematically gather patients' perspectives on their condition and available therapies



*PhRMA

Promoting Research through Regulatory Policy

- FDA's **Patient Engagement Collaborative**
  - The Patient Engagement Collaborative (PEC) brings together 16 diverse representatives from the patient community to meet with the FDA several times a year and discuss how to achieve more meaningful patient engagement in medical product development and other regulatory discussions. The PEC, launched in December 2017, is a joint effort of CTTI and the FDA.

Promoting Research by Contributing to the Science

- Can define and help lead the scientific and clinical agenda for the field
- Priorities are driven by the needs of patient members
  - Biomarkers, Patient Reported Outcomes and "Real World Evidence"
- Provide/facilitate additional resources such as bio-banks and patient registries, e.g.
  - **Melanoma Research Alliance**
  - **Lungevity**
  - **Chordoma Foundation**
  - **Pancreatic Cancer Action Network**
  - **Castleman Disease Collaborative Network**

Melanoma Research Alliance

LUNGEVITY
Find it. Treat it. Live.

Promoting Research by Contributing to the Science

- Example: **Multiple Myeloma Research Foundation**
  - Established the Multiple Myeloma Research Consortium to accelerate early phase clinical trials – a clinical network of 16 institutions which has advanced >20 compounds through trials
- Example: **Leukemia and Lymphoma Society**
  - The *Therapy Acceleration Program® (TAP)* identifies and funds innovative projects related to therapies that have the potential to change the standard of care for patients with blood cancer, especially in areas of high unmet medical need.

MM RF SM MULTIPLE MYELOMA Research Foundation

LEUKEMIA & LYMPHOMA SOCIETY®
fighting blood cancers

FRIENDS of CANCER RESEARCH

FRIENDS of CANCER RESEARCH

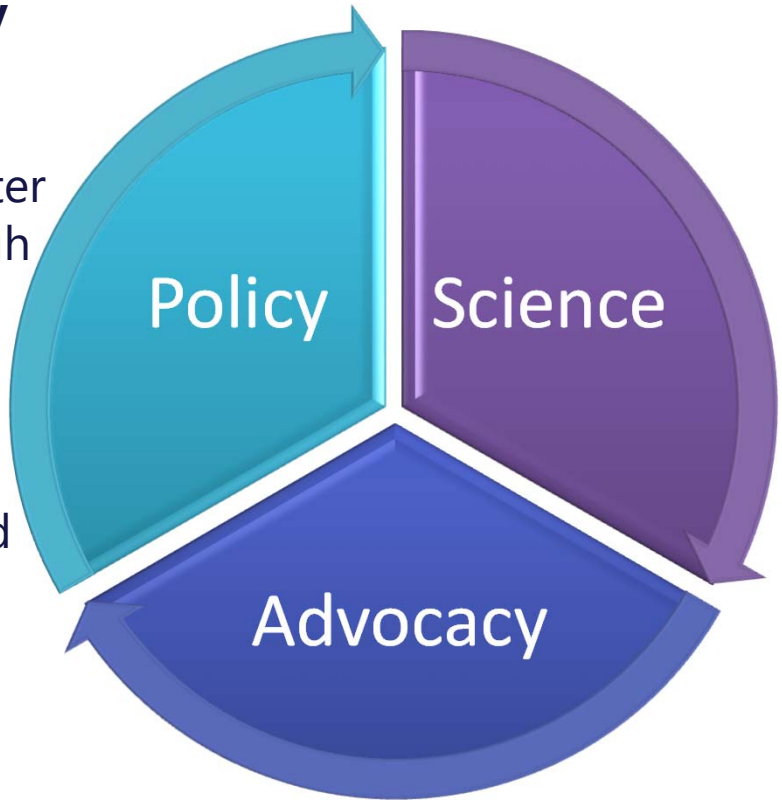
Accelerating the Pace of Innovation

Washington, DC-based Science Policy & Advocacy Organization

A unique model to create a path to better drug development and approval through **scientific**, **regulatory**, and **legislative solutions**.

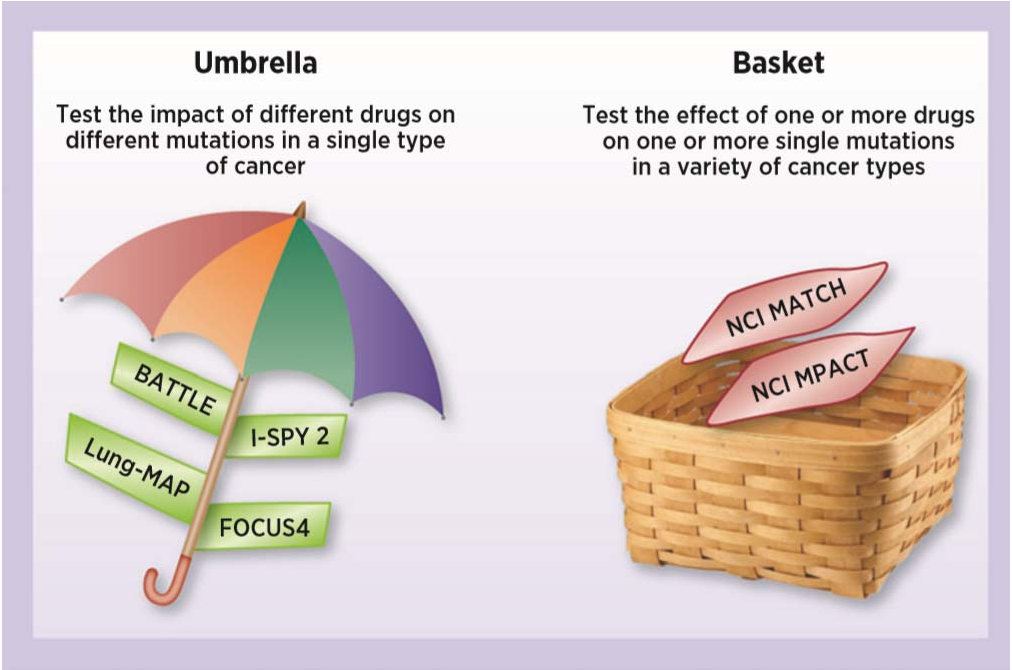
Develops groundbreaking partnerships:

- Federal Agencies (FDA, NIH, NCI, and CMS)
- Academic Research Centers
- Professional Societies
- Industry
- Advocacy Organizations

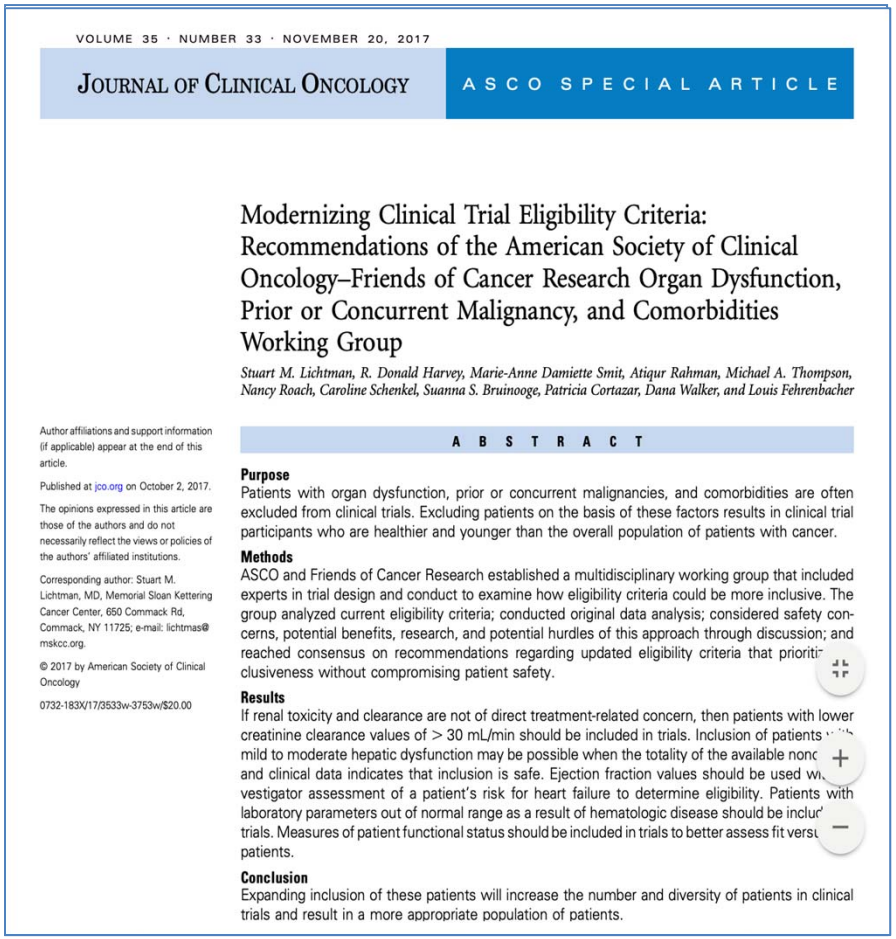


Advancing Clinical Trial Designs

- **Lung-MAP is a first-of-its-kind clinical trial model that uses a multi-drug, targeted screening approach to match patients with sub-studies testing investigational new treatments based on their unique tumor profiles.**



Advancing Clinical Trial Eligibility



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ASCO SPECIAL ARTICLE

Modernizing Clinical Trial Eligibility Criteria: Recommendations of the American Society of Clinical Oncology–Friends of Cancer Research Organ Dysfunction, Prior or Concurrent Malignancy, and Comorbidities Working Group

Stuart M. Lichtman, R. Donald Harvey, Marie-Anne Damiette Smit, Atiqur Rahman, Michael A. Thompson, Nancy Roach, Caroline Schenkel, Suanna S. Bruinooge, Patricia Cortazar, Dana Walker, and Louis Fehrenbacher

Author affiliations and support information (if applicable) appear at the end of this article.

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0732-183X/17/3533w-3753w/$20.00

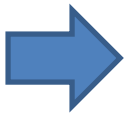
A B S T R A C T

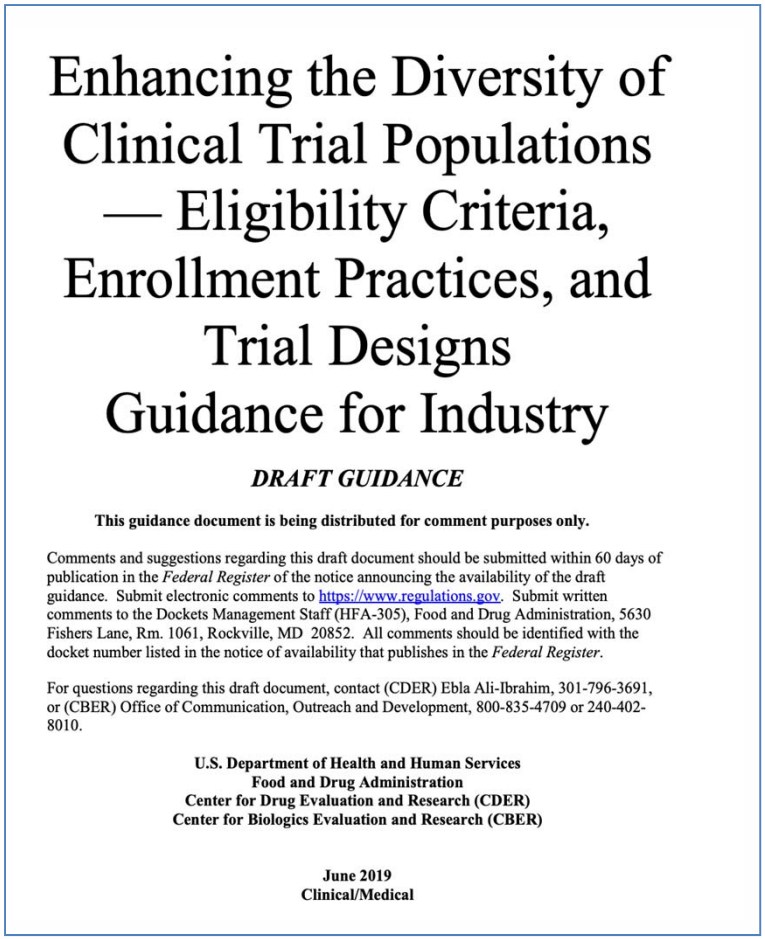
Purpose
Patients with organ dysfunction, prior or concurrent malignancies, and comorbidities are often excluded from clinical trials. Excluding patients on the basis of these factors results in clinical trial participants who are healthier and younger than the overall population of patients with cancer.

Methods
ASCO and Friends of Cancer Research established a multidisciplinary working group that included experts in trial design and conduct to examine how eligibility criteria could be more inclusive. The group analyzed current eligibility criteria; conducted original data analysis; considered safety concerns, potential benefits, research, and potential hurdles of this approach through discussion; and reached consensus on recommendations regarding updated eligibility criteria that prioriti clusiveness without compromising patient safety.

Results
If renal toxicity and clearance are not of direct treatment-related concern, then patients with lower creatinine clearance values of > 30 mL/min should be included in trials. Inclusion of patients mild to moderate hepatic dysfunction may be possible when the totality of the available nonc and clinical data indicates that inclusion is safe. Ejection fraction values should be used w vestigator assessment of a patient's risk for heart failure to determine eligibility. Patients with laboratory parameters out of normal range as a result of hematologic disease should be inclu trials. Measures of patient functional status should be included in trials to better assess fit versu patients.

Conclusion
Expanding inclusion of these patients will increase the number and diversity of patients in clinical trials and result in a more appropriate population of patients.





Enhancing the Diversity of Clinical Trial Populations — Eligibility Criteria, Enrollment Practices, and Trial Designs Guidance for Industry

DRAFT GUIDANCE

This guidance document is being distributed for comment purposes only.

Comments and suggestions regarding this draft document should be submitted within 60 days of publication in the *Federal Register* of the notice announcing the availability of the draft guidance. Submit electronic comments to https://www.regulations.gov. Submit written comments to the Dockets Management Staff (HFA-305), Food and Drug Administration, 5630 Fishers Lane, Rm. 1061, Rockville, MD 20852. All comments should be identified with the docket number listed in the notice of availability that publishes in the *Federal Register*.

For questions regarding this draft document, contact (CDER) Ebla Ali-Ibrahim, 301-796-3691, or (CBER) Office of Communication, Outreach and Development, 800-835-4709 or 240-402-8010.

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)
Center for Biologics Evaluation and Research (CBER)

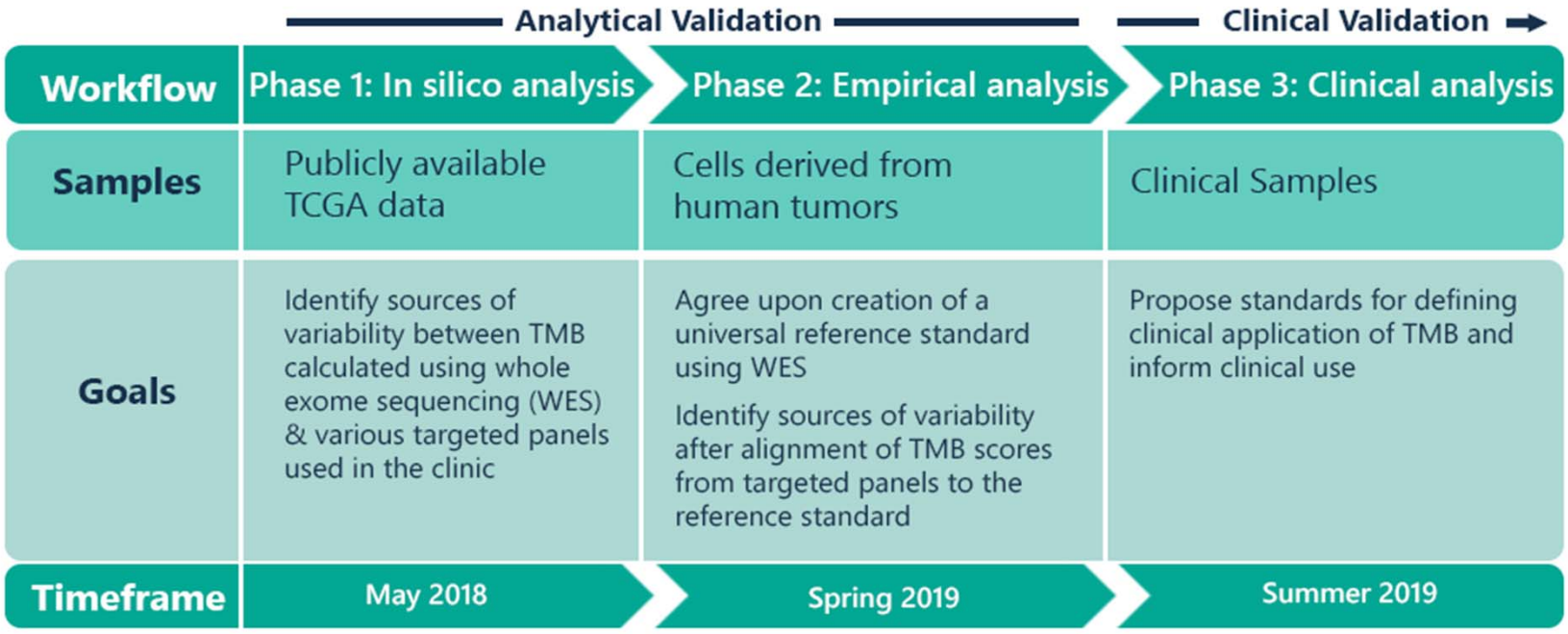
June 2019
Clinical/Medical

RWE Pilot Project: Establishing the Utility of Real-World Endpoints

Workflow	Step 1: Standardize Analysis of Real-World Endpoints	Step 2: Data Analyses	Step 3: Public Meeting
Goals	Define real-world endpoints Describe analysis methods for measuring real-world endpoints	Work with partners to develop datasets to assess real-world endpoints definition and methodology Assess quality metrics and propose refinements to real-world endpoint definitions and methodology	Present findings Establish recommendations for real-world endpoint analysis Evaluate whether real-world endpoints are indicators of clinical benefit
Timeframe	February 2019	Spring 2019	September 18, 2019

10 Healthcare research organizations and additional stakeholders participating in this pilot project include: Aetion, ASCO CancerLinQ/Concerto HealthAI, Cancer Research Network, COTA, FDA, Flatiron Health, IQVIA™, Mayo Clinic, McKesson, NCI SEER-Medicare Linked Database, OptumLabs®, Syapse, Tempus

TMB Harmonization Project



Partners: Government: National Cancer Institute (NCI), U.S. Food and Drug Administration (FDA) **Academia:** Brigham & Women's Hospital, Columbia University, EORTC, Johns Hopkins University, Massachusetts General Hospital, MD Anderson Cancer Center, Memorial Sloan Kettering Cancer Center **Diagnostics:** ACT Genomics, Caris Life Sciences, Foundation Medicine, Inc., Guardant Health, Inc., Illumina, Inc., NeoGenomics Laboratories, Inc., OmniSeq, Personal Genome Diagnostics (PGDx), Q^2 Solutions, QIAGEN, Inc., Thermo Fisher Scientific **Industry:** AstraZeneca, Bristol-Myers Squibb Company, EMD Serono, Inc., Genentech, Merck & Co., Inc., Pfizer, Inc., Regeneron Pharmaceuticals **Operational:** precisionFDA, SeraCare

Companion Diagnostics: Soup to Nuts

- *Friends*-Alexandria Blueprint Forum

  2019 - A Blueprint for Breakthrough: Validating Real-World Endpoints for an Evolving Regulatory Landscape
  2018 - A Blueprint for Breakthrough: Research and Reimbursement in the Age of Precision Medicine
  2017 - A Blueprint for Breakthrough: Charting the Course for Precision Medicine
  2016 - A Blueprint for Breakthrough: Exploring the Utility of Real World Evidence
  2015 - A Blueprint for Drug/Diagnostic Development: Standardization of Genetic Databases
  2014 - A Blueprint for Drug/Diagnostic Development: Next-Generation Sequencing (NGS)
  2013 - A Blueprint for Drug/Diagnostic Co-Development: Breakthrough Therapies
  2012 - Forum and Blueprint for Future Drug/Diagnostic Co-Development

- Biomarker Harmonization (TMB)
- VALID Act (Verifying Accurate Leading-edge In vitro Diagnostics)
  - Pre-specified modifications
  - Performance thresholds for test groups
- CMS National Coverage Decision-NGS Tests
  - Flexible coverage framework to support innovation

FRIENDS OF CANCER RESEARCH

Annual Meeting

Tuesday, November 12, 2019
8:00AM - 4:00PM
The Willard
1401 Pennsylvania Ave NW

Register Today!

- Characterizing the Use of External Comparators for Augmenting Randomized Control Arms and Confirming Benefit
- Data Generation (and Review Considerations) for Use of a Companion Diagnostic for a Class of Oncology Therapeutic Products
- Immuno-Oncology Combination Drug Development for Patients with Relapsed/Refractory Disease After Initial PD-(L)1 Therapy

Engaging Patients: A How-To

Check for updates

Clinical Trials: Original Research

DIA

Therapeutic Innovation
& Regulatory Science
2018, Vol. 52(2) 206-213

DOI: 10.1177/2168479017720247
tirs.sagepub.com

The Rules of Engagement: CTTI Recommendations for Successful Collaborations Between Sponsors and Patient Groups Around Clinical Trials

Diane Bloom, PhD, MPH[1], Joel Beetsch, PhD[2], Matthew Harker, MPH, MBA[3], Sharon Hesterlee, PhD[4], Paulo Moreira[5], Bray Patrick-Lake, MFS[6], Wendy Selig, MSJ[7], Jeffrey Sherman, MD, FACP[8], Sophia K. Smith, PhD[9], James E. Valentine, JD, MHS[10], and Jamie N. Roberts, MPH, MA[6]

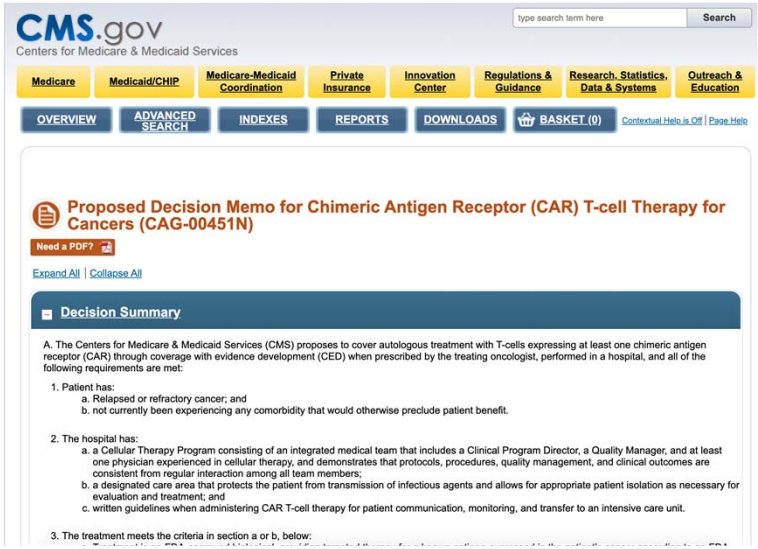
Contact me @ llasiter@focr.org

Patient Experience Data

Enhancing Use of Patient Experience Data

Incorporating the patient experience into drug development is a crucial next step to ensuring that those suffering with this disease are getting the safest and most effective treatments possible.

- *Friends* Roundtable White Paper - Enhancing Use of Patient-Centered Data in Regulatory Decision-Making
- *Friends* Annual Meeting Panel - Capturing Symptomatic Adverse Events From the Patients' Perspective



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Proposed Decision Memo for Chimeric Antigen Receptor (CAR) T-cell Therapy for Cancers (CAG-00451N)

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Decision Summary

A. The Centers for Medicare & Medicaid Services (CMS) proposes to cover autologous treatment with T-cells expressing at least one chimeric antigen receptor (CAR) through coverage with evidence development (CED) when prescribed by the treating oncologist, performed in a hospital, and all of the following requirements are met:

1. Patient has:
   a. Relapsed or refractory cancer; and
   b. not currently been experiencing any comorbidity that would otherwise preclude patient benefit.

2. The hospital has:
   a. a Cellular Therapy Program consisting of an integrated medical team that includes a Clinical Program Director, a Quality Manager, and at least one physician experienced in cellular therapy, and demonstrates that protocols, procedures, quality management, and clinical outcomes are consistent from regular interaction among all team members;
   b. a designated care area that protects the patient from transmission of infectious agents and allows for appropriate patient isolation as necessary for evaluation and treatment; and
   c. written guidelines when administering CAR T-cell therapy for patient communication, monitoring, and transfer to an intensive care unit.

3. The treatment meets the criteria in section a or b, below:

Use of Patient-Reported Outcomes to Understand & Measure the Patient Experience of Novel Cell and Gene Therapies

Abstract

Patient reported outcomes (PROs) are the gold standard for assessing patients' experience of treatment in oncology. PROs provide a comprehensive assessment of the benefits and risks of new medical products, as well as essential data to inform real-world use. Although RCTs the ultimate source for information for evaluating products in development, they are not always feasible for rare diseases with few or no effective treatment options available. Thus, it is important to consider other measures that can help to improve the strength of evidence for cell and gene therapies targeting rare indications. While collection of PROs and other patient experience endpoints does not resolve the difficulty of conducting trials in small populations, doing so contributes empirical evidence that informs both product development and patient access. Additionally, including routine collection of PROs in registries may provide supplemental data to further characterize the benefit:risk profile of cell and gene therapies at follow-up times that would be infeasible to operationalize in a clinical trial setting.

Background: Cell & Gene Therapies

Therapies derived from human cells and genes are providing novel treatment options for patients with life-threatening conditions. Gene therapies seek to modify a patient's genes to treat or cure disease. The transferred genetic material changes how a single protein or group of proteins is produced by the cell. Gene therapy can be used to reduce levels of a disease-causing version of a protein, increase production of disease-fighting proteins, or to produce new/modified proteins. Cell therapy is the transfer of intact, live cells into a patient to help lessen or cure a disease. Cell therapies alter the biological properties of living cells, either a patient's own cells as in autologous cell therapies, or from a donor as in allogeneic cell therapies, for therapeutic use. The cells used in cell therapy can be classified by their potential to transform into different cell types. Though cell and gene therapies have different mechanisms of action, the US FDA regulates both treatment modalities as gene therapies.