

# Medicare Coverage and Evidence of Clinical Utility



**NHGRI - WELLCOME TRUST WORKSHOP**

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**Presented by:**

**CENTERS FOR MEDICARE &  
MEDICAID SERVICES (CMS)**

# Medicare Begins: 1965



President Lyndon B. Johnson at the signing ceremony July 30, 1965, at the Truman Library in Independence, Missouri.

***Source: CMS / ORDI***

# Reasonable and Necessary



- *Medicare coverage process*: how CMS answers:
  - “is an item or service *reasonable and necessary* for the diagnosis or treatment of an illness or injury for Medicare beneficiaries?”
- This process is called ‘the National Coverage Determination Process’ or ‘the NCD process’.

# R&N $\equiv$ Evidence of Improved Outcomes



- *Working definition* of ‘reasonable and necessary’:
  - = ‘adequate evidence exists to conclude that the item or service improves clinically meaningful health outcomes for the Medicare population’.

# Key Evidence for the NCD Process



- Analytical validity:
- Clinical validity:
- **Clinical utility:**
  - Is available evidence adequate to conclude that testing for genetic variants leads to changes in physician decisions about therapy which improve patient outcomes?
- Ethical, legal, and social implications

*Source: <http://www.cdc.gov/genomics/gtesting/ACCE/index.htm>*

# MEDCAC: Guiding the Coverage Process



## Medical Evidence Development and Coverage Advisory Committee Meeting (MEDCAC)

1. Convened at CMS' option to provide guidance on evidence assessment and interpretation on specific topics; and
2. Includes input from subject matter experts, ethicists, clinical trialists, and patient representatives.
3. Three MEDCAC meetings have focused on genetic or genomic testing since 2009.

# Assessing evidence from clinical trials

## About testing for genetic variants



- ***More value*** associated with:
  - Prospective trials
  - Controlled trials
  - Objective comparators and endpoints
  - Using techniques to reduce bias, such as randomization, masking
  - Proper use of statistical tools and well-powered studies
- ***Less value*** associated with:
  - Retrospective studies
  - Uncontrolled studies
  - Studies based only on self-reported survey data
  - Small studies

# Coverage with Evidence Development (CED)



- **Role: Addressing the Evidence Dilemma**
  - “ ... CED ... has been used by CMS under certain scenarios and has been recommended by the IOM for the development of cancer biomarkers.”
    - ✦ *MJ Khoury, Berg A, et al., Health Affairs, 2008*
- **Example : Pharmacogenomic testing for warfarin responsiveness (2009) – A CED Determination**
  - ✦ Specific variants: *VKORC1* (A/B); *CYP2C9* \*2 and \*3
  - Purpose: If warfarin dosing is based on results of testing for these variants, do outcomes improve for patients starting anticoagulation?
  - Status: Two Medicare-approved clinical studies in progress



# Key question for coverage



Does testing for genetic variants lead to improved, clinically meaningful outcomes?

- “ ... **the next step must be** to mount clinical trials in which patients are stratified according to their biologic signature **to determine whether knowledge of this information leads to better clinical outcomes.** If personalized medicine is going to become a reality, we need to design and execute these critical trials.”
  - *Drazen JM. (editorial) New Engl J Med, Sep 26, 2011*

# For more information about coverage:



- CMS website, including the database of Medicare coverage decisions, available at

[www.cms.gov](http://www.cms.gov)

Brought to you by:



## CMS, especially:

- **Center for Medicare Management**
  - Hospital & Ambulatory Policy Group
  - Division of Ambulatory Services
- **Office of Clinical Standards and Quality**
  - Coverage and Analysis Group
  - Division of Items and Devices