



# Alliance Member Meeting

featuring

Paul T. Kim

February 17, 2022

# Today's Guest



**Paul T. Kim**  
Partner, Foley Hoag LLP

## SAFE

- Benefits outweigh its risks

## EFFECTIVE

- Substantial evidence that it has the effect it purports or is represented to have

- FDA decision informed by Advisory Committee vote
- Adequate and well controlled clinical investigations
- May be one trial with confirmatory evidence

***Federal Food, Drug & Cosmetic Act  
Public Health Service Act***

## REASONABLE & NECESSARY

- for diagnosis or treatment of illness or injury
- Policy: “adequate evidence to conclude that the item or service improves health outcomes”

- Historic coverage of FDA-approved indications
- Coverage options:
  - National Coverage Determination
  - Defer to regional MACs
  - Limited or no coverage

***Medicare Parts A & B under the  
Social Security Act***

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# Accelerated Approval

- In response to delays in treatments for HIV/AIDS, FDA adopted **regulatory reforms** leading to 1987 approval of AZT on the basis of a surrogate endpoint (CD4 counts) and to **1992 regulations**. (*57 Fed. Reg. 58942 (Dec. 11, 1992)*)
- A drug approved under accelerated approval is ***not*** a tentative approval or ‘conditional’ approval, it is a **full FDA approval** for marketing.
- Accelerated approval is subject to “**post-approval studies to verify... the predicted effect**” ... “where there is **uncertainty as to the relation** of the surrogate endpoint to clinical benefit” (*FFDCA sec 506/21 USC 356(c)(2); 21 CFR 314.510 and 601.41*)

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# Accelerated Approval

A drug is eligible for the pathway if it -

- treats a **serious or life-threatening condition**;
- provides a **meaningful advantage** over existing therapies; AND
- demonstrates an effect on a **surrogate endpoint** that is reasonably likely to predict clinical benefit or on an **intermediate clinical endpoint** (one measured earlier than irreversible morbidity or mortality (IMM) that is reasonably likely to predict an effect on IMM or other clinical benefit)

*(Section 506(c) of the FD&C Act; 21 CFR 314, subpart H, and 21 CFR part 601, subpart E)*



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# Prescription Drug Coverage

## Medicare

- **Part B** (Medical benefit)
  - FDA-approved; and
    - Physician Administered
    - DME
    - Specific statutory coverage
  - Generally open formulary, with MACs typically covering FDA-approved drugs to label
- **Part D** (Pharmacy benefit)
  - FDA-approved
  - Not otherwise covered in Part B
  - Generally restrictive formulary

## Medicaid

- Covered if:
  - Manufacturer pays rebate
  - Manufacturer participates in public health discount programs
  - Generally open formulary



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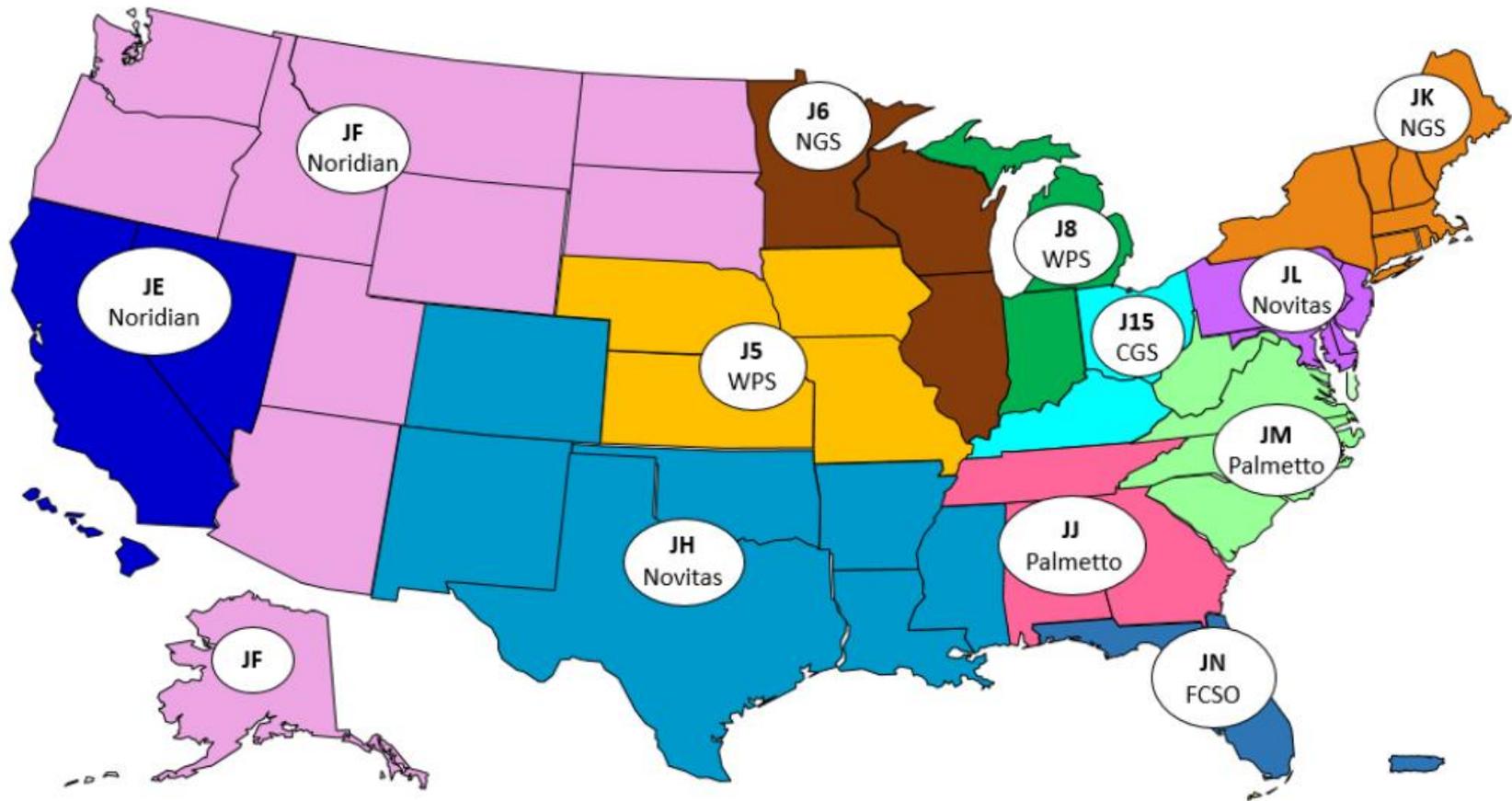
# Medicare: Reasonable and Necessary

CMS has broad discretion to interpret this term, and makes Medicare Parts A and B coverage determinations on whether an item or service is -

- **safe and effective,**
- **not experimental or investigational,** and
- **appropriate for Medicare patients,** including the duration and frequency that is considered appropriate (*Medicare Program Integrity Manual, chapter 13.5.4*)
- CMS frequently interprets “reasonable and necessary” as “**adequate evidence** to conclude that the item or service **improves health outcomes**”

*(SSA section 1862(a)(1)(A) / 42 U.S.C. 1395y)*

# Local Coverage by Medicare Administrative Contractors (MACs)





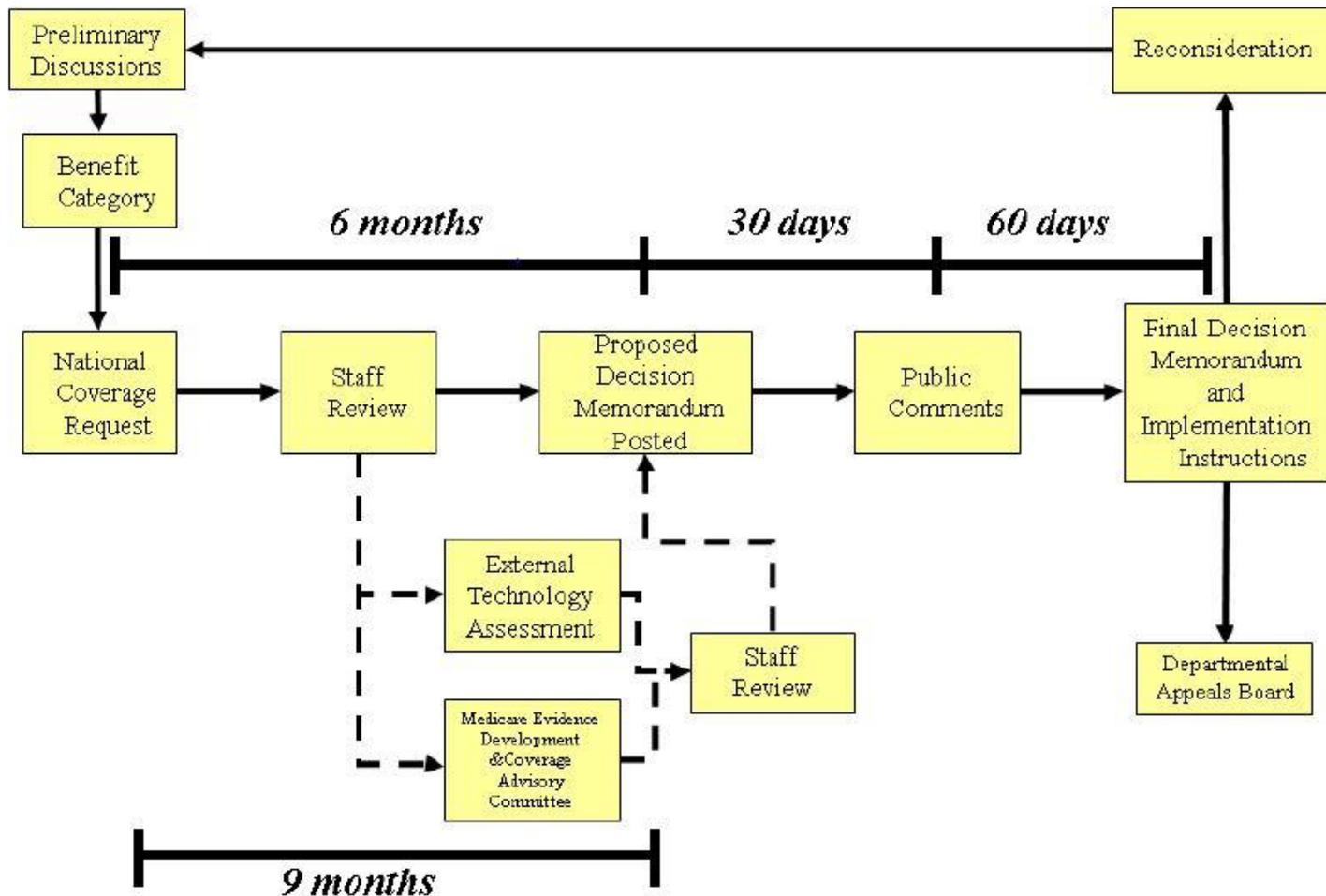
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# Coverage with Evidence Development

- CED provides beneficiary access through **CMS-approved studies or other programs** to generate real-world data about safety and effectiveness
- Since 2005, **CMS has used this pathway in a few cases** for interventions ranging from amyloid PET for Alzheimer’s clinical evaluation, implantable cardioverter defibrillators, and lung volume reduction surgery
- Requirements have ranged from **randomized controlled trials** to **patient registries** and other less structured data instruments
- CED relies on **statutory authority** enabling the Agency for Healthcare Research and Quality’s (AHRQ) to conduct research on outcomes, effectiveness and appropriateness of services for Medicare beneficiaries  
*(SSA sec 1862(a)(1)(E)/42 USC 1395y)*

# National Coverage Determination (NCD) Process



# H. R. 6000

To continue the acceleration of the discovery, development, and delivery of 21st century cures, and for other purposes.

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## IN THE HOUSE OF REPRESENTATIVES

NOVEMBER 17, 2021

Ms. DEGETTE (for herself and Mr. UPTON) introduced the following bill; which was referred to the Committee on Energy and Commerce, and in addition to the Committees on Ways and Means, the Budget, Science, Space, and Technology, Agriculture, Education and Labor, Armed Services, Natural Resources, Veterans' Affairs, Homeland Security, and the Judiciary, for a period to be subsequently determined by the Speaker, in each case for consideration of such provisions as fall within the jurisdiction of the committee concerned

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## A BILL

To continue the acceleration of the discovery, development, and delivery of 21st century cures, and for other purposes.

1 *Be it enacted by the Senate and House of Representa-*  
2 *tives of the United States of America in Congress assembled,*

### 3 SECTION 1. SHORT TITLE.

4 This Act may be cited as the “Cures 2.0 Act”.

### 10 SEC. 305. IMPROVING FDA-CMS COMMUNICATION REGARD- 11 ING TRANSFORMATIVE NEW THERAPIES.

12 (a) IN GENERAL.—Upon the designation of a product  
13 as a breakthrough therapy, a fast track product, or a  
14 product eligible for accelerated approval under subsection  
15 (a), (b), or (c), respectively, of section 506 of the Federal  
16 Food, Drug, and Cosmetic Act (21 U.S.C. 356), the Com-  
17 missioner of Food and Drugs and the Administrator of  
18 the Centers for Medicare & Medicaid Services shall—

19 (1) maintain communication with each other re-  
20 garding approval and coverage decisions with respect  
21 to such product; and

22 (2) share such information with each other as  
23 may be appropriate to inform and coordinate such  
24 decisions.

1 (b) SEPARATE AND DISTINCT.—In approving or des-  
2 ignating a product described in subsection (a), the Com-  
3 missioner of Food and Drugs and the Administrator of  
4 the Centers for Medicare & Medicaid Services shall ensure  
5 that the process for approval or designation remains sepa-  
6 rate and distinct.

# Join us next time!

Wednesday, March 2, 2022, 1:15 p.m. ET



**France A. Córdova, PhD**  
President, Science  
Philanthropy Alliance