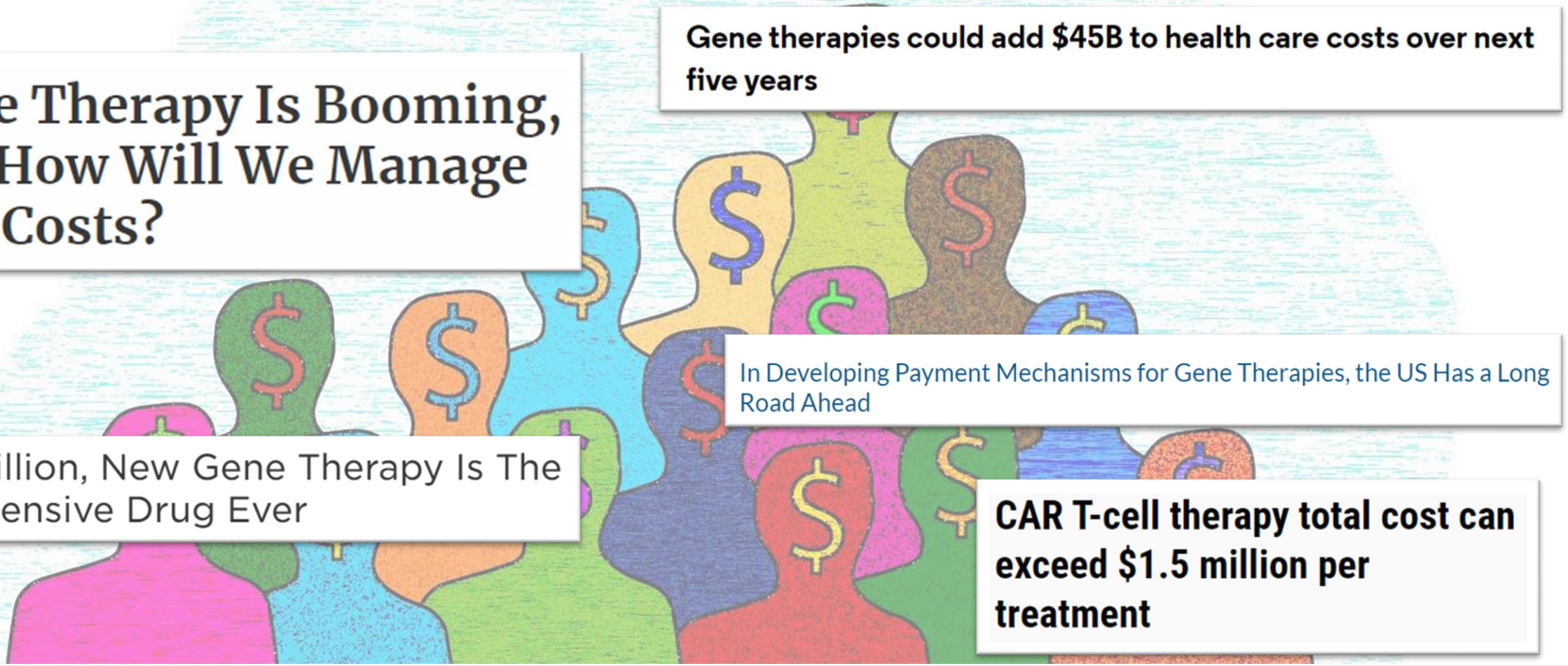
Gene and Cell Therapy Coverage, Reimbursement, and Pricing:
How Will We Pay for the Next Generation of Potentially Curative Medicines?

The Price of Hope: Weighing the Cost of CAR-T Cell Therapy in Treating Blood Cancers

Gene therapies could add $45B to health care costs over next five years

Gene Therapy Is Booming, But How Will We Manage The Costs?



In Developing Payment Mechanisms for Gene Therapies, the US Has a Long Road Ahead

At $2.1 Million, New Gene Therapy Is The Most Expensive Drug Ever

CAR T-cell therapy total cost can exceed $1.5 million per treatment

Medicare 101

Rochelle Fink, MD, JD, MBA
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Center for Medicare & Medicaid Services (CMS) Overview

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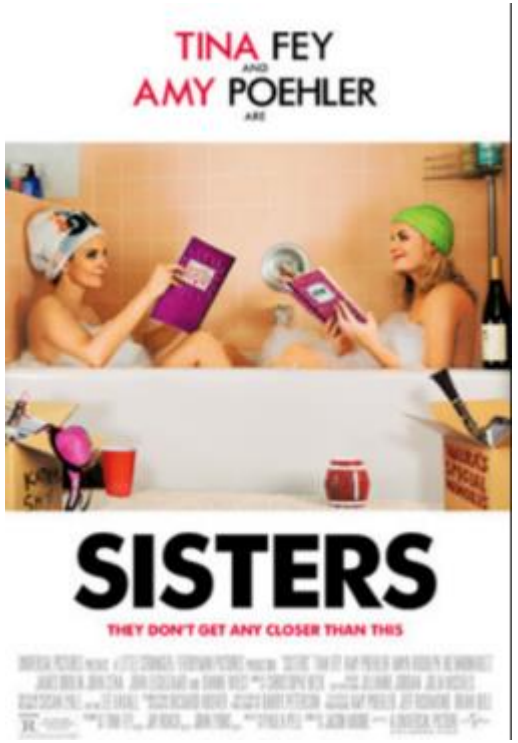

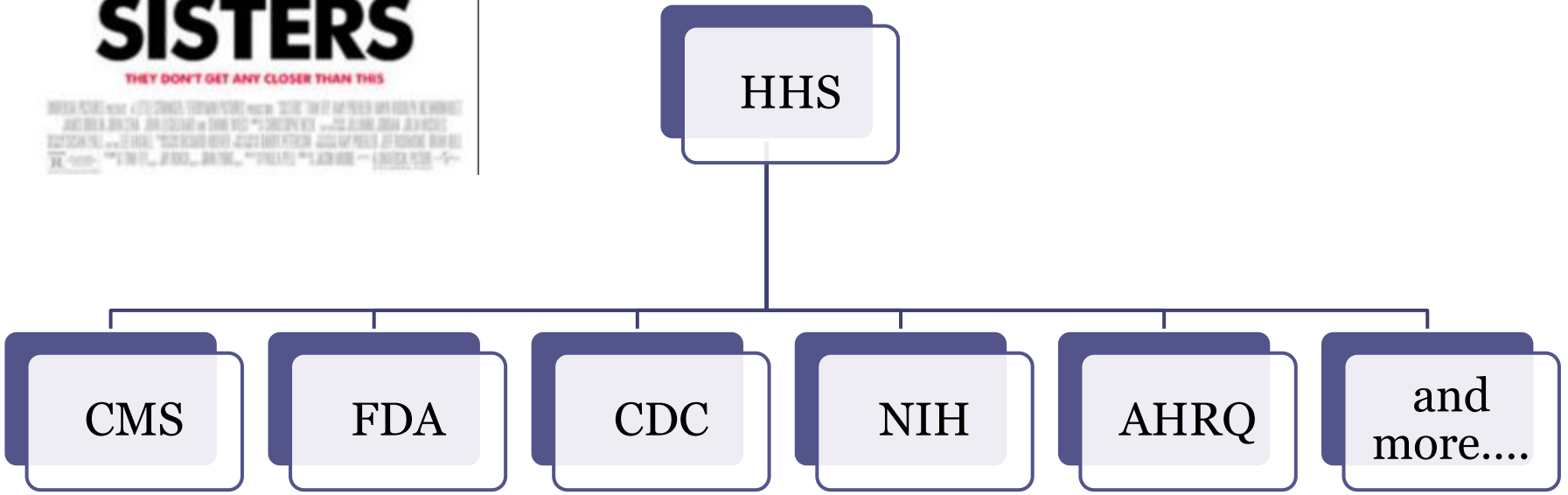

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TITLE XVIII—HEALTH INSURANCE FOR THE AGED AND DISABLED

- Title XVIII of the Social Security Act is administered by the Centers for Medicare and Medicaid Services
- Title XVIII appears in the United States Code as §§1395-1395lll, subchapter XVIII, chapter 7, Title 42
- Regulations of the Secretary of Health and Human Services relating to Title XVIII are contained in chapter IV, Title 42, and in subtitle A, Title 45, Code of Federal Regulations

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Part A—Hospital Insurance Benefits for the Aged and Disabled

- Inpatient care in a hospital
- Skilled nursing facility care
- Nursing home care (inpatient care in a skilled nursing facility that's not custodial or long-term care)
- Hospice care
- Home health care

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Part B—Supplementary Medical Insurance Benefits for the Aged and Disabled

- **Medically necessary services:** services or supplies that are needed to diagnose or treat your medical condition and that meet accepted standards of medical practice
- **Preventive services:** Health care to prevent illness (like the flue) or detect it at an early state, when treatment is most likely to work best

Examples: clinical research, ambulance services, durable medical equipment (DME), mental health, limited outpatient prescription drugs

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Part A & B

- Coverage
- Coding
- Payment

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Part C—Medicare+Choice Program

- Comparable to an HMO plan
- Sometimes called “Part C,” “Medicare Advantage (MA),” or “MA Plan”
- Offered by private companies approved by Medicare

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Part D—Voluntary Prescription Drug Benefit Program

- Prescription drug coverage
- Generally, self-administered
- Generally, administered outside of a health care setting

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Beneficiaries

Individuals 65 years +

&

Individuals under age 65 who:

- Receive disability benefits from Social Security of the Railroad Retirement Board
- Have End Stage Renal Disease (ESRD)

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Center for Medicare & Medicaid Services (CMS)

Timeframe: 2020

Total Number of Medicare Beneficiaries

Location	Original Medicare	Medicare Advantage	Total
United States [1]	37,745,095	23,467,152	61,212,247

** Kaiser Family Foundation, https://www.kff.org/medicare/state-indicator/total-medicare-beneficiaries/?currentTimeframe=0&sortModel=%7B%22colId%22:%22Location%22,%22sort%22:%22asc%22%7D

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Medicare vs. Medicaid

	Medicare	Medicaid
Age Qualification	X	
Income Qualification		X
State Program		X

DIFFERENCES BETWEEN FDA & CMS

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Public Law 94–295
94th Congress

An Act

To amend the Federal Food, Drug, and Cosmetic Act to provide for the safety and effectiveness of medical devices intended for human use, and for other purposes.

May 28, 1976
[S. 510]

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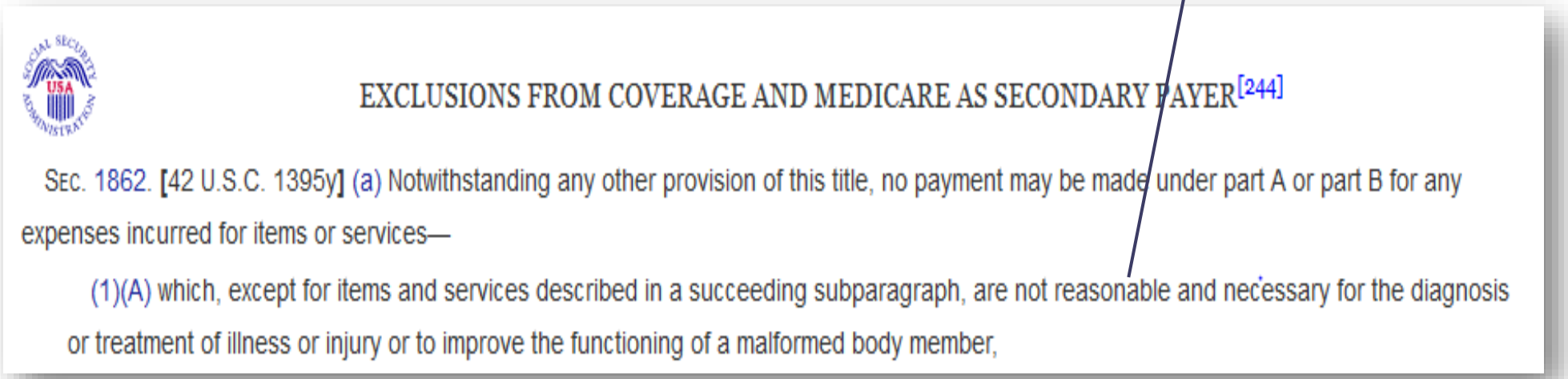
Coverage

reasonable & necessary



EXCLUSIONS FROM COVERAGE AND MEDICARE AS SECONDARY PAYER[244]

SEC. 1862. [42 U.S.C. 1395y] (a) Notwithstanding any other provision of this title, no payment may be made under part A or part B for any expenses incurred for items or services—

(1)(A) which, except for items and services described in a succeeding subparagraph, are not reasonable and necessary for the diagnosis or treatment of illness or injury or to improve the functioning of a malformed body member,

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Coverage

- National Coverage Determinations
- Local Coverage Determinations
  - Medicare Administrative Contractors (MAC)

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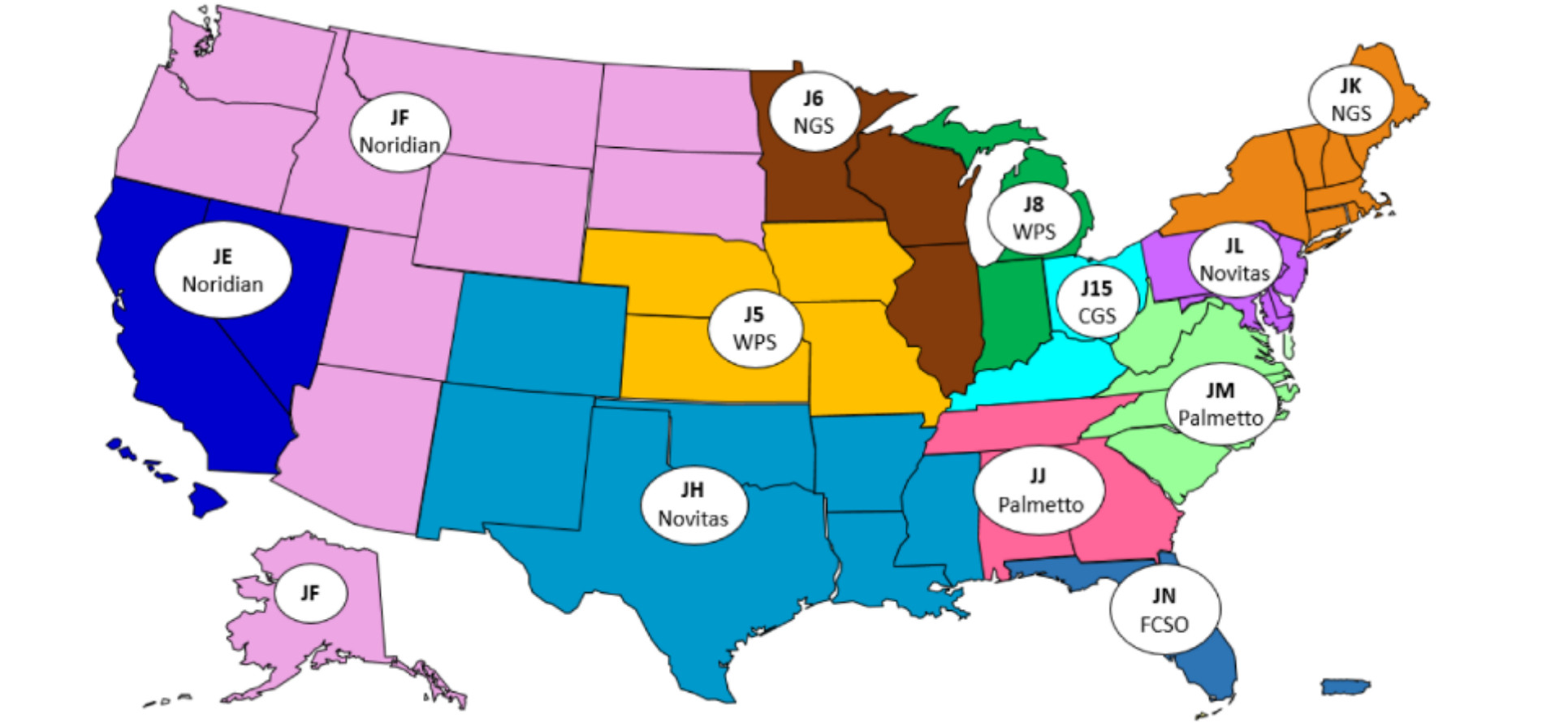
A/B MAC Jurisdictions
as of June 2019



FDA-CMS Working Together

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Parallel Review FR Notices

Purpose: Reduce the time between FDA marketing approval and a CMS national coverage determination (NCD)

First announced: 75 Fed. Reg. 57045 (Sept. 17, 2010)

Described: 76 Fed. Reg. 62808 (Oct. 11, 2011)

Extended: 78 Fed. Reg. 76628 (Dec. 18, 2013)

Updated: 81 Fed. Reg. 73113 (Oct. 24, 2016)

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Program Candidates

- Sponsor candidate has had sufficient pre-IDE interaction with FDA or approved IDE application
- Technology for which an original or supplemental application for PMA or petition for de novo review would be required
- Technologies that fall within a **defined benefit category**—Sec. 1861 of the Social Security Act

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CMS Feedback Outside of Parallel Review

- Vast majority of feedback provided by CMS
- No application process
- Set up a time to speak with the Coverage Group
- CMS provides significant feedback on IDE trials
- Go to CMS to request CMS input

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Lessons Learned from CMS-FDA-Company Interactions

- Companies benefit from speaking to CMS & FDA at the pivotal clinical trial design stage
- Companies need to know their benefit category before requesting to speak with CMS
- CMS can provide helpful input on IDE trials
- Concurrent review by the Agencies of clinical evidence can reduce time from FDA decision to NCD decision

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Private Payor Opportunities

- Payor Communication Task Force | FDA

Company Name	Company Mailing Address
Aetna, a CVS Health Company	151 Farmington Avenue Hartford, CT 06156
BlueCross BlueShield Association	225 North Michigan Avenue Chicago, IL 60601
CareFirst BlueCross BlueShield (HealthWorx)	1501 S. Clinton Street, 17th Floor Baltimore, MD 21224
Center for Medicare & Medicaid Services (CMS)	7500 Security Boulevard, Baltimore, MD 21244
Cigna	900 Cottage Grove Road Bloomfield, CT 06002
Clover Health	30 Montgomery Street 15th Floor Jersey City, NJ 07302
Duke Evidence Synthesis Group, Duke Clinical Research Institute, Duke University	2400 Pratt Street Duke Clinical Research Institute Durham, NC 27705
ECRI Institute Headquarters	5200 Butler Pike Plymouth Meeting, PA 19462-1298
Humana	500 West Main Street Louisville, KY 40202
Kaiser Permanente	393 East Walnut Street Pasadena, CA 91188
National Institute for Health and Care Excellence (NICE)	Level 1A City Tower, Piccadilly Plaza, Manchester, M1 4BT, United Kingdom
Premier Inc.	13034 Ballantyne Corporate Pl. Charlotte, NC 28277
United Health Group	9900 Bren Road East Minnetonka, MN 55343

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INNOVATORS' GUIDE TO NAVIGATING MEDICARE

Version 3
2015

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Thank You

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Reimbursement Challenges for Cell and Gene Therapies

Michael J. Werner
Partner, Holland & Knight, LLP
Co-founder, Alliance for Regenerative Medicine

FDLI, June 9, 2021

Holland & Knight

Broader Drug Pricing Politics

- HR 3 still promoted by the House Democratic Leadership
  - Would allow negotiating prescription drug prices for Medicare. Would also cap the prices of a select number of drugs in both Medicare Part B and D, based on an international price index. The bill would require a drug's price to be set at or below 120% of the average price across six high-income countries: Australia, Canada, U.K., France, Germany, and Japan.
  - The drugs chosen for index-pricing would be selected on the basis of their high cost and lack of competition. HHS must negotiate the prices of at least 25 such drugs for 2024 and of at least 50 such drugs thereafter.
  - The bill also (1) requires drug manufacturers to issue rebates to the Centers for Medicare & Medicaid Services for covered drugs that cost $100 or more and for which the average manufacturer price increases faster than inflation; and (2) reduces the annual out-of-pocket spending threshold, and eliminating beneficiary cost-sharing above this threshold, under the Medicare prescription drug benefit.
- Upcoming Senate Finance Committee hearing
- International reference pricing proposals

Medicare Hospital Inpatient Payment

- New Technology Add on Payment (NTAP) program available for new technologies
  - Ex, CAR-Ts
- Criteria:
  - Newness
  - Significant clinical improvement
  - Costly and inadequately reimbursed under existing system
- NTAP available for CGT administered in-hospital on temporary basis. NTAP was increased 50% to 65% at the end of 2019 and there was no change in 2020.
  - CMS had ruled that CAR-T products (Kymriah, Yescarta) were eligible
- FY 2021 IPPS final rule established a DRG for CAR-Ts that target blood borne diseases.
- FY 2022 IPPS proposed rule is recommending to expand the therapies that will be covered by the “CAR-T DRG” to include other cellular therapies (example: those that target solid tumors).

Innovative Payment Models - VBP

- Current reimbursement models not designed for CGT. Statutory and regulatory barriers prevent Medicare and Medicaid from using innovative payment models for reimbursement of new, single or limited administration, durable/potentially curative therapies.
- “Best Price” (BP) and “Average Manufacturing Price” (AMP) reporting requirements inadvertently pose significant barriers due to the structure of these new innovative payment models as they limit the discounts that can be offered based on the outcome of the therapy.
- CMS promulgated a rule at the very end of last year that, among other things, addresses the BP and AMP barriers
  - CMS has delayed implementation
- Representative Brett Guthrie (R-KY) and Markwayne Mullin (R-OK) are drafting an “outcomes-based agreement” (OBA) bill that exempts best price and AMP reporting requirements for gene therapies (defined as a treatment for a serious or life-threatening rare disease or condition that will result in either a cure or a reduction in symptoms) in “outcomes-based agreements.” Additionally, they would exclude from these arrangements Anti-Kickback Statute and Stark Law restrictions.

Strategic Questions

- Understand your product -- indication, site of service, etc.
- Understand the reimbursement process
  - Obtaining a code -- CPT (medical procedure; process determined by AMA) and HCPCS (product, process determined by CMS)
  - Coverage decision – By CMS or private insurer. Medicare has formal coverage process, usually local carriers based on advisory panel. Private insurers also use advisory panels to evaluate new technologies. Based on scientific/clinical data
  - Payment – Based on site of service
  - Timetable – Each component operates on a different timetable and all are different from FDA
- Gather health economics and comparative clinical effectiveness data.
- Get to know key stakeholders – physicians, patients, etc. Physicians determine coding policy at AMA and influence coverage decisions and can impact product uptake. Patients provide “political” support for coverage and payment of breakthrough treatments.
- Get Congressional allies