MEDICARE COVERAGE

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Liver Forum 7

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Disclosures

ADVI has consulting relationships with a large number of medical device, biopharmaceutical, and diagnostics companies. I have no conflicts of interest related to the material discussed in this presentation.

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THE LAW

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Social Security Act 1862(a)(1)

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—

(A) which, except for items and services described in a succeeding subparagraph or additional preventive services (as described in section 1395x(ddd)(1) of this title), are not reasonable and necessary for the diagnosis or treatment of illness or injury or to improve the functioning of a malformed body member,

***

(E) in the case of research conducted pursuant to section 1142, which is not reasonable and necessary to carry out the purposes of that section,

***

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What is the definition of R&N?

- Congress did not define it.
- HCFA attempted unsuccessfully to define via rulemaking in ‘89 and ‘00.
- CMS explored attempting rulemaking again, but there was no traction.
- For practical uses, CMS has operationalized the following definition:

Adequate evidence to conclude that the item or service improves clinically meaningful health outcomes for the Medicare population

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Kort v Burwell, 2016

"As the court has concluded above, Defendants' consideration of health outcomes and disease management was permissible under the Medicare Act itself."

https://www.gpo.gov/fdsys/pkg/USCOURTS-dcd-1_14-cv-01519/pdf/USCOURTS-dcd-1_14-cv-01519-0.pdf

R&N is not the same as “reasonable assurance of safety and effectiveness”

FDA

- Risk based standards - PMA v 510(k)
- “Reasonable assurance” language leaves more room for interpretation
- Approval/clearance decisions are centralized
- Evidence is in the label
- Sponsor is the main customer
- Propriety secrets kept from public

CMS

- R&N is not risk based
- Absence of language similar to “reasonable assurance”
- Coverage decisions are largely decentralized to regional contractors, who may disagree
- Need to consider evidence for unlabeled uses
- Beneficiaries and providers are the main customers.
- Public transparency

Medicare Fee for Service

- Most decisions are deferred to regional administrative contractors (MACs).
- The claims system defaults to payment if the code is not edited for nonpayment or suspension.
- Payment amounts generally determined by Congressional instructions.

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National Coverage Determinations

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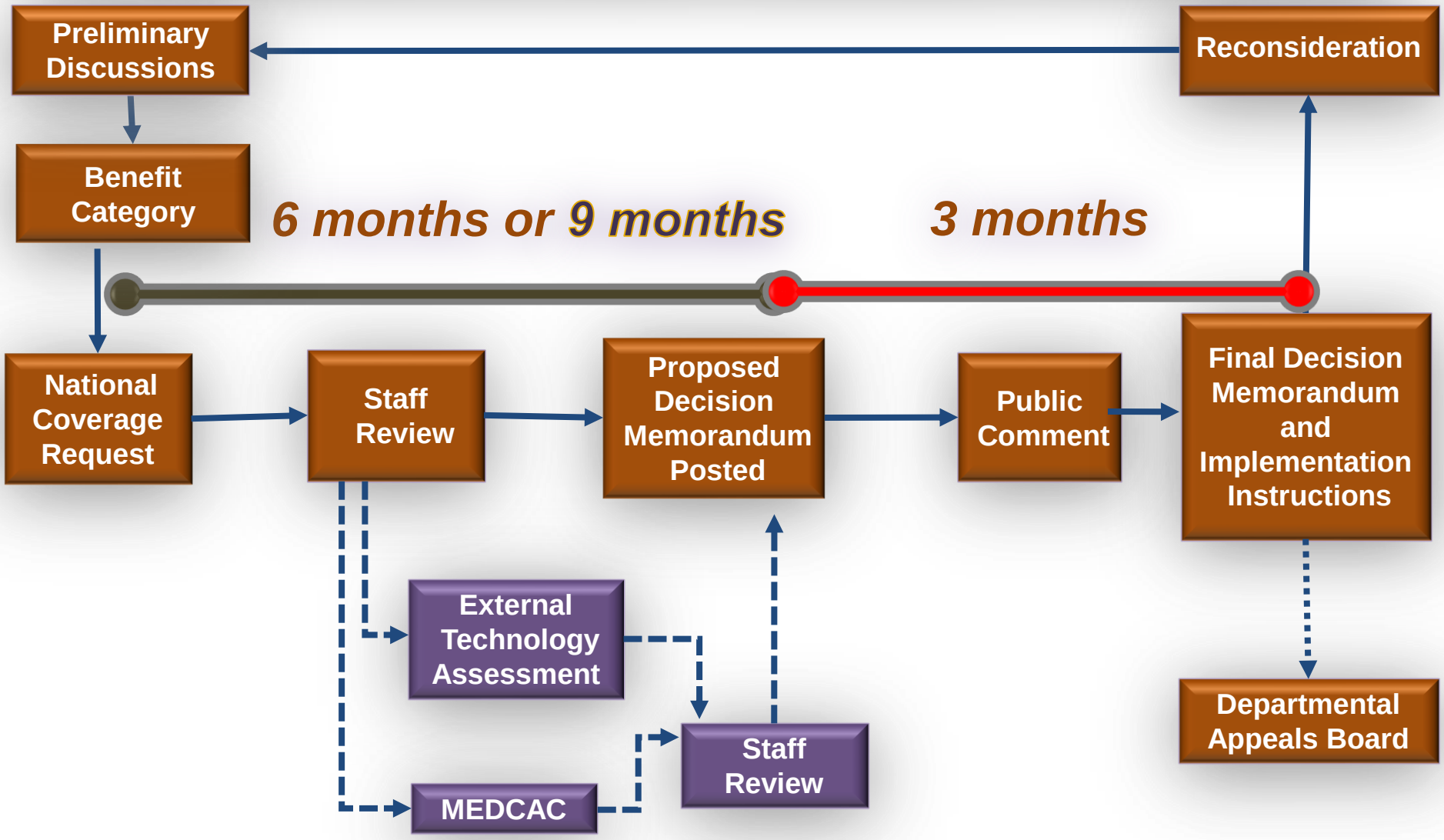
MEDICARE NATIONAL COVERAGE PROCESS



government. As required by the Federal Property Management Regulations, Title 41, Code of Federal Regulation, Subpart 101–20.301, all persons entering in or on Federal controlled property and their packages, briefcases, and other containers in their immediate possession are subject to being x-rayed and inspected. Federal law prohibits the knowing possession or the causing to be present of firearms, explosives and other dangerous weapons and illegal substances. The meeting room accommodates approximately 100 people.

PURPOSE: This committee is charged with providing advice and making recommendations to the Secretary, Department of Health and Human Services; the Director, CDC; and the Director, NCHS, regarding the scientific and technical program goals and objectives, strategies, and priorities of NCHS.

MATTERS TO BE DISCUSSED: The agenda will include welcome remarks by the Acting Director, NCHS; Demo of the NHIS Online Analytic Real-time System (OARS); initiation of Office of Analysis and Epidemiology review.

Requests to make oral presentations should be submitted in writing to the contact person listed below. All requests must contain the name, address, telephone number, and organizational

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Centers for Medicare & Medicaid Services

[CMS–3284–N]

Medicare Program; Revised Process for Making National Coverage Determinations

AGENCY: Centers for Medicare & Medicaid Services (CMS), HHS.

ACTION: Notice.

SUMMARY: This notice updates the process we use for opening, deciding or reconsidering national coverage determinations (NCDs) under the Social Security Act (the Act). It addresses external requests and internal reviews for new NCDs or for reconsideration of existing NCDs. The notice further outlines an expedited administrative process to remove certain NCDs, thereby enabling local Medicare contractors to determine coverage under the Act. This notice does not alter or amend our regulations that establish rules related to the administrative review of NCDs.

DATES: This notice is effective on August 7, 2013.

FOR FURTHER INFORMATION CONTACT: Katherine Tillman, (410) 786–9252.

SUPPLEMENTARY INFORMATION:

I. Background

- What constitutes a complete, formal request for an NCD or formal request for reconsideration of an existing NCD.
- External requests for NCDs, including the following:
  - ++ Request by an external party for a new NCD.
  - ++ Request by an external party for reconsideration of an existing NCD.
  - ++ Request by an aggrieved party (as defined below) to issue an NCD when no NCD exists.
- CMS internally-generated review of NCDs, including the following:
  - ++ CMS internal review for a new NCD.
  - ++ CMS internal review for reconsideration of an existing NCD.
- An expedited process to remove NCDs under certain circumstances.

Based on our experience since 2003 with the current NCD process, we are establishing a new procedure to be used in circumstances in which we have previously issued an NCD, but have now determined that the NCD is no longer needed. Since we would not be establishing a new NCD, we would use an expedited process to remove these NCDs. After the effective date of the removal of the NCD, local Medicare contractors would determine coverage under section 1862(a)(1) of the Act for those specific items or services previously addressed through the NCD. We describe this process and the opportunity for public participation in

http://www.cms.gov/Medicare/Coverage/DeterminationProcess/Downloads/FR08072013.pdf

General Methodological Principles of Study Design (Section VI of the Decision Memorandum)

When making national coverage determinations, CMS evaluates relevant clinical evidence to determine whether or not the evidence is of sufficient quality to support a finding that an item or service is reasonable and necessary. The overall objective for the critical appraisal of the evidence is to determine to what degree we are confident that: 1) the specific assessment questions can be answered conclusively; and 2) the intervention will improve health outcomes for patients.

We divide the assessment of clinical evidence into three stages: 1) the quality of the individual studies; 2) the generalizability of findings from individual studies to the Medicare population; and 3) overarching conclusions that can be drawn from the body of the evidence on the direction and magnitude of the intervention's potential risks and benefits.

The methodological principles described below represent a broad discussion of the issues we consider when reviewing clinical evidence. However, it should be noted that each coverage determination has its unique methodological aspects.

Health Outcomes of Greater Interest

More Impressive

- Longer life and improved function/participation
- Longer life with arrested decline
- Significant symptom improvement allowing better function/participation
- Reduced need for burdensome tests and treatments

Less Impressive

- Improved disease-specific survival without improved overall survival
- Surrogate test result better
- Doctor feels better

Quality of Life

Medicare has stated publicly that as a matter of policy that it does not generally consider cost in making national coverage determinations.

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GENERAL EVIDENTIARY CHALLENGES

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The Preferred Road to Therapeutic Coverage

- ✓ Provide adequate evidence that
- ✓ A treatment strategy using the new therapeutic technology compared to alternatives
- ✓ Leads to improved clinically meaningful health outcomes
- ✓ In Medicare beneficiaries

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The Preferred Road to Diagnostic Coverage

- ✓ Provide adequate evidence that
- ✓ The incremental information obtained by new diagnostic technology compared to alternatives
- ✓ Changes physician recommendations
- ✓ Resulting in changes in therapy
- ✓ That improve clinically meaningful health outcomes
- ✓ In Medicare beneficiaries

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42 CFR 410.32

(a) *Ordering diagnostic tests.* All diagnostic x-ray tests, diagnostic laboratory tests, and other diagnostic tests must be ordered by the physician who is treating the beneficiary, that is, the physician who furnishes a consultation or treats a beneficiary for a specific medical problem and who uses the results in the management of the beneficiary's specific medical problem. Tests not ordered by the physician who is treating the beneficiary are not reasonable and necessary (see § 411.15(k)(1) of this chapter).

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POLICY, REGULATION & ETHICS

PERSPECTIVE

Medicare coverage: engaging on evidence

Tamara Syrek Jensen & Louis B Jacques*

Experience tells us that many developers of innovative technologies fail to anticipate the evidentiary needs of insurers, particularly of Medicare. Some assume that Medicare payment begins *pro forma* upon approval or clearance by the US FDA with little regard to the distinct role of the Centers for Medicare & Medicaid Services (CMS). We offer our own suggestions, hoping they will lead to mutually satisfying discussions as we consider coverage of regenerative medicine technology. Medicare is governed by Title XVIII of the Social Security Act, which among other provisions describes the scope of the insurance benefit, methods of payment for items and services that may be covered and the process timelines for national coverage determinations. CMS implements these provisions with regulations, instructions in manuals and other guidance that are available to the public. We will focus our comments on the 'reasonable and necessary' requirement for coverage under Part A and Part B of items and services in Section 1862(a)(1)(A) of the Social Security Act.

Jensen, TS, Jacques LB. Medicare coverage: engaging on evidence. Regenerative Medicine, Vol. 6, No. 6s, November 2011: 99-101.

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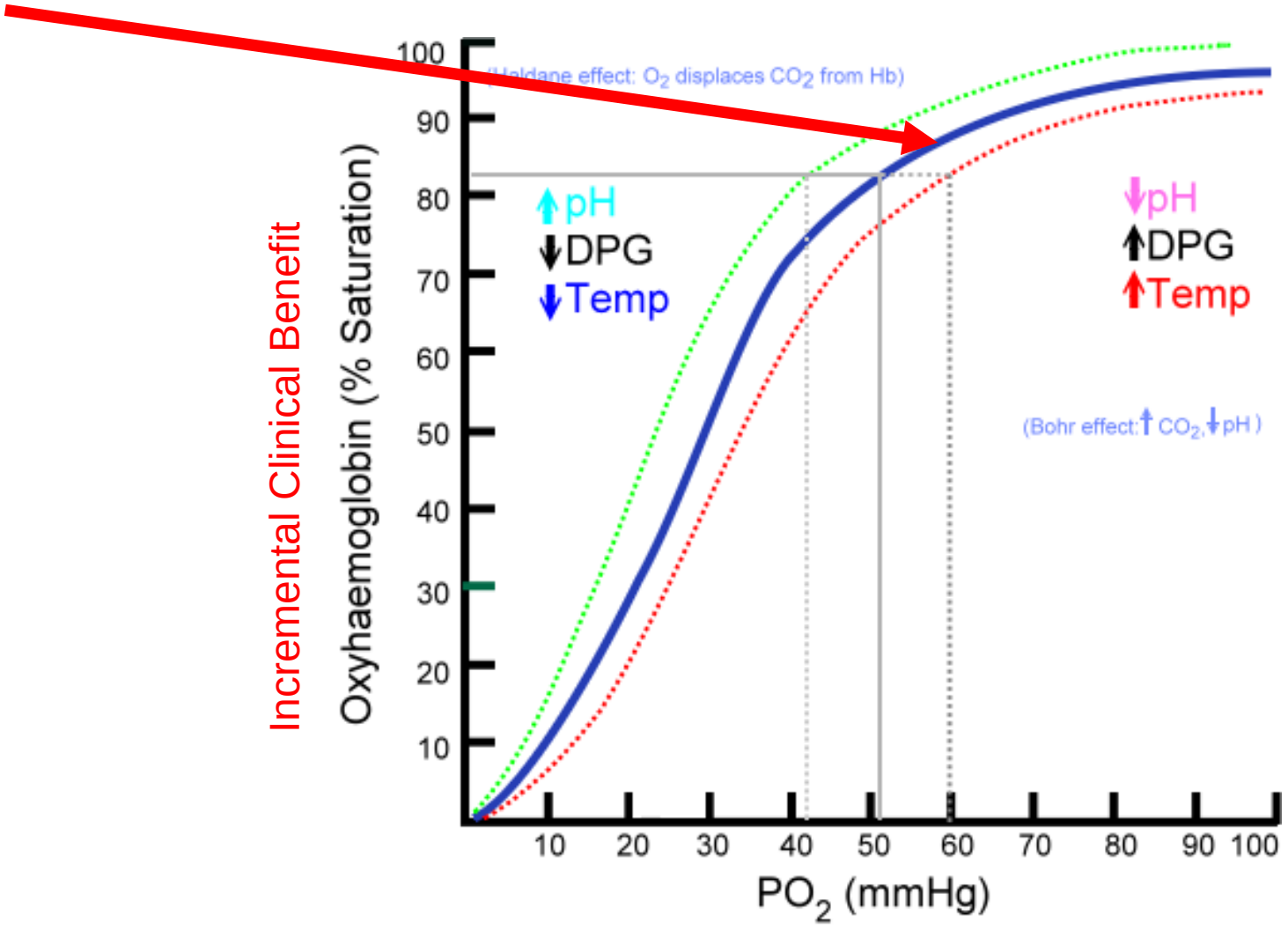
You Are Here



Clinical Trial Size and Duration

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The Plumbing Paradigm of Progress in Revascularization

1. How many plugged drains do you need to study to prove that the red one is better than the blue one?

2. If the red one costs 4X as much as the blue one, how much better does it have to be for you to buy it?

3. Suppose you already have a blue one at home?

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Comparison of Effects as Evidence Evolves From Single Trials to High-Quality Bodies of Evidence

Structured Abstract

Objective. The objective of our methods project was to use a diverse sample of medical interventions to assess empirically whether first trials rendered substantially different treatment effect estimates than reliable, high-quality bodies of evidence.

Study design and setting. We employed a meta-epidemiological study design using 100 bodies of evidence from Cochrane reports that had been graded as high quality of evidence. To determine the concordance of effect estimates between first and subsequent trials, we applied both quantitative and qualitative approaches. For quantitative assessment, we used Lin's concordance correlation and calculated z-scores; to determine the magnitude of differences of treatment effects, we calculated standardized mean differences (SMDs) and ratios of relative risks. We determined qualitative concordance based on a 2-tiered approach incorporating changes in statistical significance and magnitude of effect.

Results. First trials both over- and under-estimated the true treatment effects in no discernible pattern. Nevertheless, depending on the definition of concordance, effect estimates of first trials were concordant with pooled subsequent studies in at least 33 percent but up to 50 percent of comparisons. The pooled magnitude of change as bodies of evidence advanced from single trials to high-quality bodies of evidence was 0.16 SMD (95% confidence interval [CI], 0.12 to 0.21). In 80 percent of comparisons the difference in effect estimates was smaller than 0.5 SMDs. In first trials with large treatment effects (>0.5 SMD), however, estimates of effect substantially changed as new evidence accrued (mean change 0.68 SMD, 95% CI, .50 to 0.86)

Conclusion. Results of first trials often change but the magnitude of change, on average, is small. Exceptions are first trials that present large treatment effects which often dissipate as new evidence accrues.

http://www.effectivehealthcare.ahrq.gov/ehc/products/549/2057/grades-predictive-values-evidence-150331.pdf

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Common Concerns

- Inadequate randomization, blinding, controls
- Unrealistic comparators
- Intermediates/surrogates don’t map rigorously to clinical utility outcomes
- Composite outcomes with asymmetry between arms
- Lack of generalizability to typical targeted Medicare beneficiary
- Conflicts of interest
- Bad results get buried

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The Challenges of Non-Inferiority

Why would CMS prefer superiority trial designs to inform coverage?

- Derivation of the delta
- Clinical creep – inferiority to placebo
- Assay sensitivity – bias toward N-I
- Blinding vulnerability– bias toward N-I

"Mrs. Jones, I am 80 percent confident that this new treatment is no more than 15 percent worse that what we would have done last year."

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Don’t Do Any of These…

- Exclude enrollment of subjects > 65 years old, unless disease is generally only found in younger patients.
- Exclude adequate numbers of women, unless the disease is in men only or the treatment would be inapplicable to women.
- Exclude subjects with comorbidities commonly found in the relevant beneficiary population.
- Tell different stories to FDA and CMS

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FDA-CMS Parallel Review

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FDA-CMS Parallel Review

- The anticipated benefits of parallel review include facilitating development of innovative new products and increased efficiency in the Agencies' review processes.

- Additional potential benefits include:

  - Reducing the time between FDA marketing approval or clearance decisions and Medicare NCDs;
  - Patients are expected to gain quicker access to innovative medical technologies if they are covered;
  - Sponsors/requesters gain timely insight to the information needs of CMS with respect to pursuing an NCD as well as a potentially shortened time to payment due to a streamlined multi-review process; and
  - FDA and CMS gain enhanced channels of communication. Specifically with regard to CMS, its early involvement will streamline the decision making process.

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FDA-CMS Parallel Review

- It will also focus attention on health outcomes of importance to Medicare, and provide early awareness of any remaining evidence gaps.

- If there are evidence gaps, CMS may address them by implementing coverage with evidence development (CED) or other policy vehicles.

- For example, if FDA approval or clearance is conditioned on a post-approval study, CMS could decide to cover the device within the parameters of the post-approval study under CED.

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