Session 3: Communication of HTE to Key Stakeholders

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Central Office Org Chart Text Version

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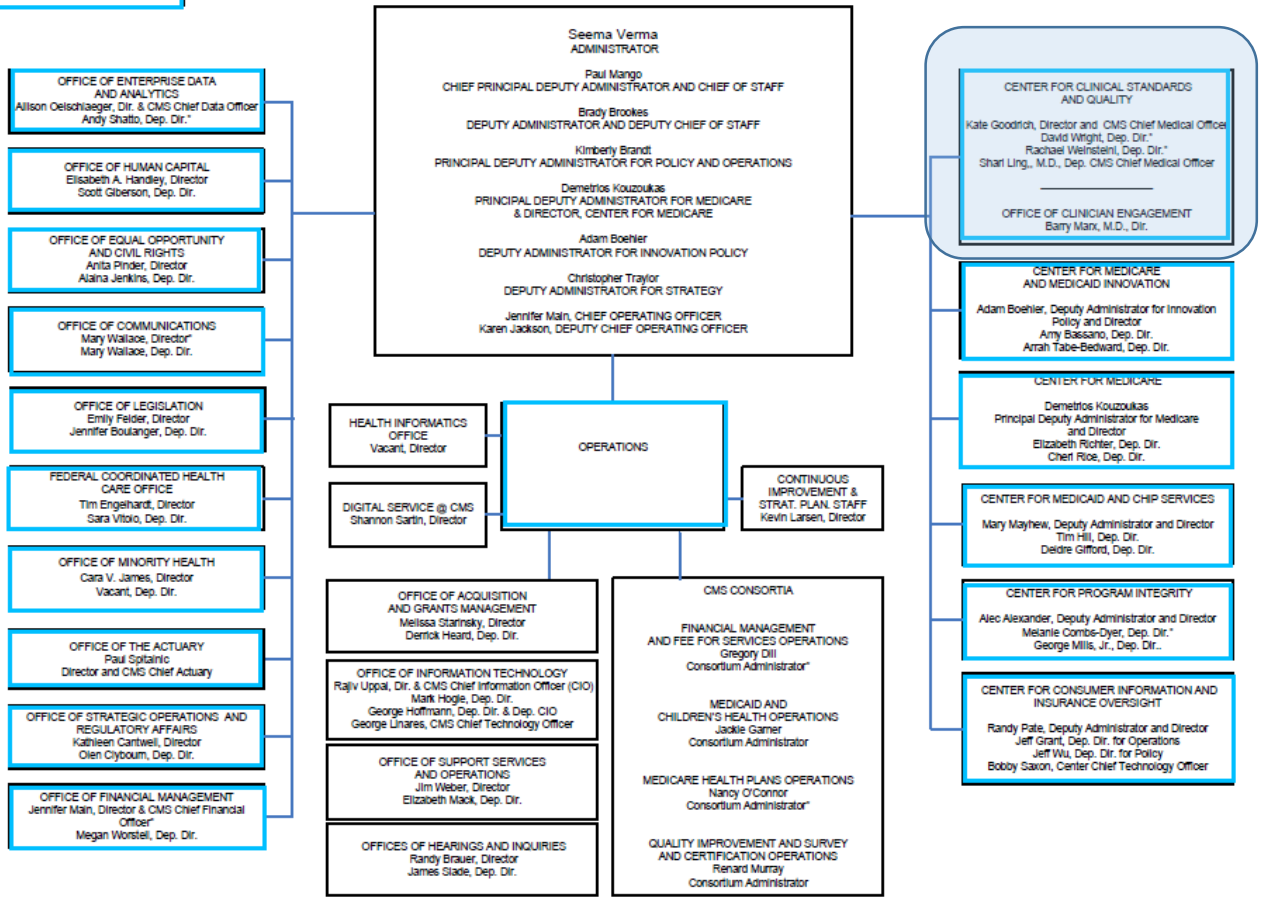
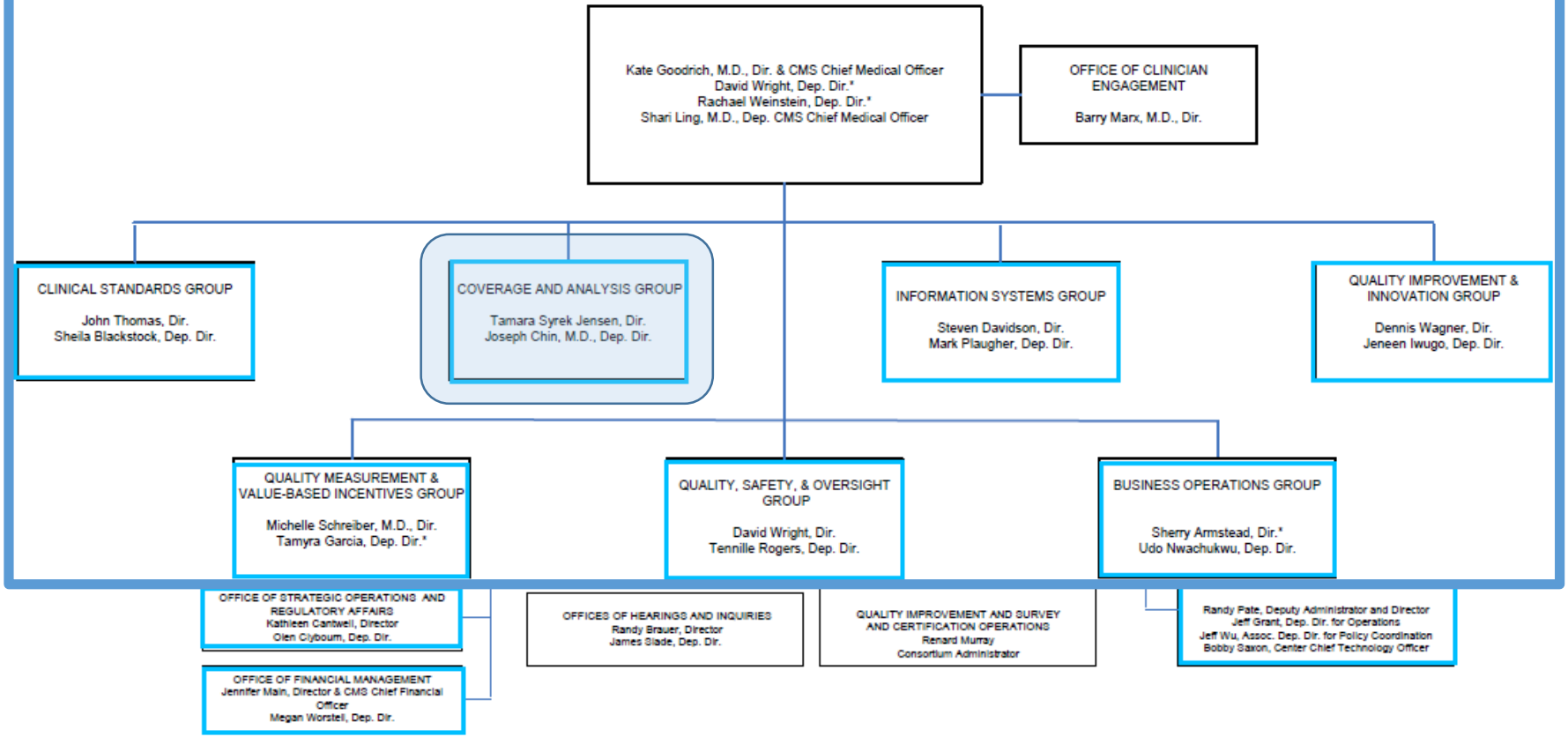
DEPARTMENT OF HEALTH AND HUMAN SERVICES
CENTERS FOR MEDICARE & MEDICAID SERVICES

APPROVED as of November 13, 2018
* Acting



DEPARTMENT OF HEALTH AND HUMAN SERVICES
CENTERS FOR MEDICARE & MEDICAID SERVICES
CENTER FOR CLINICAL STANDARDS AND QUALITY

APPROVED LEADERSHIP
As of November 1, 2018
*Acting



Challenge Question

- How does CMS promote the assessment of heterogeneity of treatment effects while clinical evidence is being developed?
- What sub-groups are of interest to Medicare when generalizability is considered for National Coverage Determinations?

42 CFR § 405.212 Medicare Coverage IDE study criteria

(1) The principal purpose of the study is to test whether the device improves health outcomes of appropriately selected patients.

(2) The rationale for the study is well supported by available scientific and medical information, or it is intended to clarify or establish the health outcomes of interventions already in common clinical use.

(3) The study results are not anticipated to unjustifiably duplicate existing knowledge.

(4) The study design is methodologically appropriate and the anticipated number of enrolled subjects is adequate to confidently answer the research question(s) being asked in the study.

(5) The study is sponsored by an organization or individual capable of successfully completing the study.

(6) The study is in compliance with all applicable Federal regulations concerning the protection of human subjects found at 21 CFR parts 50, 56, and 812 and 45 CFR part 46.

(7) Where appropriate, the study is not designed to exclusively test toxicity or disease pathophysiology in healthy individuals. Studies of all medical technologies measuring therapeutic outcomes as one of the objectives may be exempt from this criterion only if the disease or condition being studied is life threatening and the patient has no other viable treatment options.

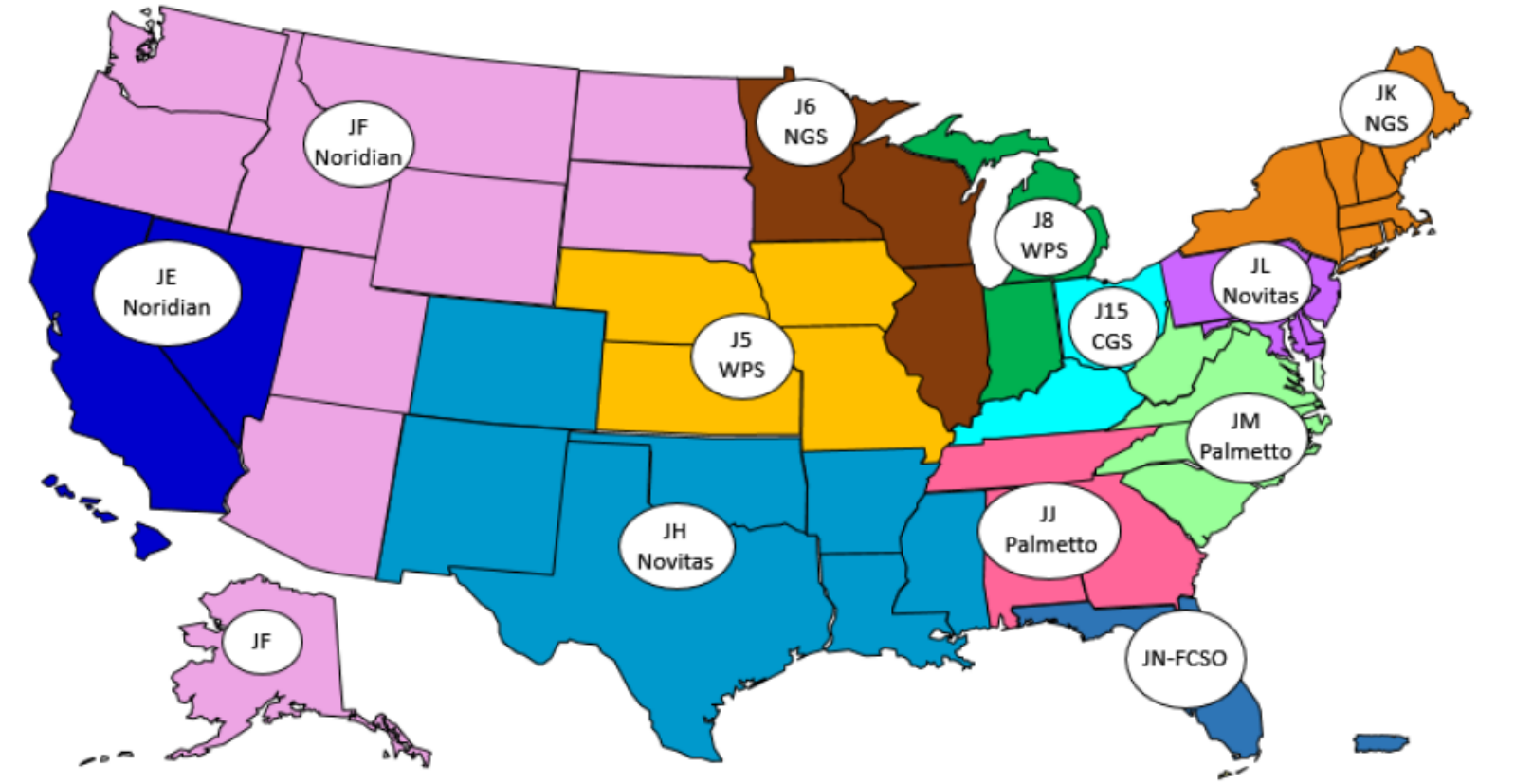
(8) The study is registered with the National Institutes of Health's National Library of Medicine's ClinicalTrials.gov.

(9) The study protocol describes the method and timing of release of results on all pre-specified outcomes, including release of negative outcomes and that the release should be hastened if the study is terminated early.

(10) The study protocol must describe how Medicare beneficiaries may be affected by the device under investigation, and how the study results are or are not expected to be generalizable to the Medicare beneficiary population. Generalizability to populations eligible for Medicare due to age, disability, or other eligibility status must be explicitly described.

A/B MAC Jurisdictions

as of October 2017



Evidence-based Medicare Coverage

National Coverage Determination (NCD):

- Sufficient evidence to conclude that the item or service improves clinically meaningful health outcomes for the Medicare population
- Based on a comprehensive review of published evidence

Medicare Beneficiaries in Clinical Studies

- Initial studies on new technologies may not include many older adults ≥ 65 years of age for several reasons including:
  - Heterogeneity – may have multiple comorbidities and/or be taking multiple medications
  - Non-adherence - may have difficulty following protocols and/or making all study follow-up visits
  - Other considerations – measurement issues, cognitive function

Coverage with Evidence Development (CED)

- Coverage in the context of approved clinical studies or with the collection of additional clinical data
- Allows for positive coverage when evidence is insufficient for a more favorable decision
  - Evidence gaps may be due to low number of beneficiaries in clinical studies, lack of meaningful health outcomes, limited generalizability, inconsistency of study findings.
- Without CED, the item or service would be non-covered
- May involve randomized controlled trials, observational studies and/or registries
  - specific intervention,
  - benefits and harms,
  - health outcomes

CMS & AHRQ General Requirements

a) The principal purpose of the study is to test whether the item or service meaningfully improves health outcomes of affected beneficiaries who are represented by the enrolled subjects.

b) The rationale for the study is well supported by available scientific and medical evidence.

c) The study results are not anticipated to unjustifiably duplicate existing knowledge.

d) The study design is methodologically appropriate and the anticipated number of enrolled subjects is sufficient to answer the research question(s) being asked in the National Coverage Determination.

e) The study is sponsored by an organization or individual capable of completing it successfully.

f) The research study is in compliance with all applicable Federal regulations concerning the protection of human subjects found in the Code of Federal Regulations (CFR) at 45 CFR Part 46. If a study is regulated by the Food and Drug Administration (FDA), it is also in compliance with 21 CFR Parts 50 and 56. In addition, to further enhance the protection of human subjects in studies conducted under CED, the study must provide and obtain meaningful informed consent from patients regarding the risks associated with the study items and/or services, and the use and eventual disposition of the collected data.

g) All aspects of the study are conducted according to appropriate standards of scientific integrity.

h) The study has a written protocol that clearly demonstrates adherence to the standards listed here as Medicare requirements.

i) The study is not designed to exclusively test toxicity or disease pathophysiology in healthy individuals. Such studies may meet this requirement only if the disease or condition being studied is life threatening as defined in 21 CFR §312.81(a) and the patient has no other viable treatment options.

j) The clinical research studies and registries are registered on the www.ClinicalTrials.gov website by the principal sponsor/investigator prior to the enrollment of the first study subject. Registries are also registered in the Agency for Healthcare Quality (AHRQ) Registry of Patient Registries (RoPR).

k) The research study protocol specifies the method and timing of public release of all prespecified outcomes to be measured including release of outcomes if outcomes are negative or study is terminated early. The results must be made public within 12 months of the study's primary completion date, which is the date the final subject had final data collection for the primary endpoint, even if the trial does not achieve its primary aim. The results must include number started/completed, summary results for primary and secondary outcome measures, statistical analyses, and adverse events. Final results must be reported in a publicly accessibly manner; either in a peer-reviewed scientific journal (in print or on-line), in an on-line publicly accessible registry dedicated to the dissemination of clinical trial information such as ClinicalTrials.gov, or in journals willing to publish in abbreviated format (e.g., for studies with negative or incomplete results).

l) The study protocol must explicitly discuss beneficiary subpopulations affected by the item or service under investigation, particularly traditionally underrepresented groups in clinical studies, how the inclusion and exclusion criteria effect enrollment of these populations, and a plan for the retention and reporting of said populations in the trial. If the inclusion and exclusion criteria are expected to have a negative effect on the recruitment or retention of underrepresented populations, the protocol must discuss why these criteria are necessary.

m) The study protocol explicitly discusses how the results are or are not expected to be generalizable to affected beneficiary subpopulations. Separate discussions in the protocol may be necessary for populations eligible for Medicare due to age, disability or Medicaid eligibility.

Guidance for the Public, Industry, and CMS Staff: Coverage with Evidence Development