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# **Informing Coverage with Evidence Development: Private Sector Perspectives**

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## Overview:

- Questions we've been asked to address
- Overview of UnitedHealthcare's approach to evidence review, coverage/benefit design, and programs to promote high quality, cost-effective care and support clinical innovation
- Perspectives to inform Medicare CED policy and program

## Questions We've Been Asked to Address:

### Question 3

How would an evidentiary threshold to invoke CED be influenced by the following?

- Whether the item or service is a diagnostic v. a therapeutic technology
- The severity of the disease
- The safety profile of the technology
- The availability of acceptable alternatives for the same disease/condition
- Other factor(s)
- A combination or tradeoff involving two or more of the above

## Questions We've Been Asked to Address:

### Question 4

How would an evidentiary threshold to invoke CED be influenced if the outstanding questions focused only on the generalizability of a strong but narrow evidence base to

- Additional settings
- Additional practitioners
- Broader clinical indications for related or unrelated diseases
  - An example of a related condition might include a different stage of the same cancer. An example of an unrelated condition might include the use of a cancer drug for a rheumatologic disease.

# UnitedHealth Group: A Diversified Health and Well-Being Company With A Mission of Helping People Live Healthier Lives

UnitedHealth Group

## Innovation, Transparency and Consumerism



UnitedHealthcare®

Data



OPTUM™

Health  
Benefits

← Services →

Health Services

Partnership

## Foundational Competencies

Clinical care management

Advanced technology

Health data and informatics

- UnitedHealth Group serves more than 75 million Americans each year
- We process more than \$115 billion in health care spend per year
- We partner with more than 650,000 physicians and other care providers, 6,000 hospitals, 80,000 dentists, and 65,000 pharmacies in all 50 states

## UnitedHealthcare:

- Provides coverage and benefits to 35 million Americans
- Develops and deploys comprehensive, integrated programs to promote high quality, cost effective care, based on the most advanced clinical science and informed guidance
- Utilizes a flexible, evolving “toolbox” including:
  - Benefit design
  - Coverage policy
  - Network configurations
  - Care facilitation
  - Consumer engagement and activation
  - Transparent performance assessment and feedback
  - Shared accountability with physicians, hospitals
    - Performance-based contracting
- Strong supporter of clinical innovation and advances in the art and science of medicine

# Coverage Considerations for Medicare Beneficiaries (and for CED):

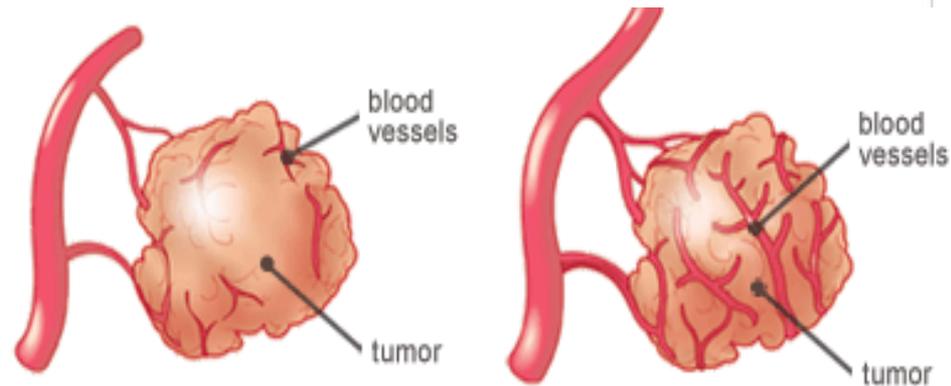


- Ideal characteristics of health services available to Medicare beneficiaries
  - At least as safe as other services treating the same condition
  - At least as effective in real world practice as other services treating the same condition
  - Appropriate to subpopulations accessing the service
  - Cost-effective on an episode basis relative to other treatments
  - Performed by physicians and in facilities with experience and expertise
  - Ongoing measurements of clinical quality and cost effectiveness

# New Medical Technologies Offer Both Opportunities and Challenges



# Eloxatin™



**AVASTIN™**  
(bevacizumab)

## What's Wrong with the Current State?

- Systematic reviews of clinical evidence and clinical practice guidelines often lack scientific rigor
  - How to assess safety and effectiveness
- Body of clinical evidence for services in which physicians treating Medicare beneficiaries are interested may be weak or totally lacking
  - Example: Treatment of chronic wounds
  - Example: Transcutaneous Electrical Nerve Stimulation (TENS)
- Difficult for physicians to connect recommendations to clinical evidence
  - How to identify appropriate sub-populations
  - Cost-effectiveness frequently not addressed at all
- Conflicting recommendations from different stakeholders
  - How to identify physicians and facilities with experience and expertise
  - How to measure clinical quality and cost effectiveness

# What's Wrong with the Current State (cont.)?



- FDA does not have a requirement to provide information regarding a medical device's comparative effectiveness in the device label.
  - In the absence of comparative data, should medical device labeling be required to state that there is no evidence of the product's superiority to other products?
- Should the FDA require active comparator trials as part of the approval process and provide strict oversight of the process of study design and analysis?

# UnitedHealthcare Approach to Coverage Policy:



- **Scientific Evidence**
  - Standards based on credible published scientific evidence, supported by controlled clinical trials or observational studies.
- **Clinical Appropriateness**
  - Services must be clinically appropriate for our members in terms of type, frequency, extent and duration.
- **Cost Effectiveness**
  - Services must not be more costly than an alternative services that is at least as likely to produce equivalent therapeutic and diagnostic results.

# Assessment of Scientific Evidence: UnitedHealthcare Hierarchy of Evidence



- Statistically robust, well-designed randomized controlled trials
- Statistically robust, well-designed cohort studies
- Large, multi-site observational studies
- Single-site observational studies
- In the absence of incontrovertible scientific evidence, medical policies may be based upon national consensus statements by recognized authorities. The following stratification describes the hierarchy of use of medical policies and clinical guidelines within UnitedHealthcare:
  - Centers for Medicare and Medicaid Services (CMS) National Coverage Decisions (NCDs) (Medicare population)
  - Milliman Care® guidelines (commercial population)
  - National guidelines and consensus statements, e.g. United States Preventive Services Task Force (USPSTF), National Institutes of Health (NIH) clinical statements, Agency for Health Care Research and Quality (AHRQ) clinical statements.
  - Clinical position papers of professional specialty societies, e.g. American College of Physicians (ACP), American College of Cardiology (ACC), American College of Chest Physicians (ACCP), when their statements are based upon referenced clinical evidence.
- Expert opinion

# UnitedHealthcare's Approach to Supporting Evidence Generation and Clinical Innovation:



- UnitedHealthcare covers investigational and unproven services:
  - As part of well-designed and appropriately sponsored clinical trials
  - For treatment of life-threatening illnesses likely to cause demise within one year of proposed treatment when two separate studies from separate institutions conclude that the clinical advantages of such treatment outweigh the potential harms
  - For treatment of serious, rare diseases that occur so infrequently that a body of evidence is unlikely to accumulate within a reasonable period of time, when a three-physician SME panel concludes that the clinical advantages of such treatment outweigh the potential harms

# Acceptable Thresholds of Clinical Evidence:



- Must be high for diagnostic tests
  - Must directly impact physician decision-making
  - Must improve health outcomes
  - Example: PET scan for various cancers
    - PET must detect occult recurrent cancers that other markers miss
    - Comparative effectiveness with circulating tumor cell (CTC) technology?
  - Example: PET for dementia
    - How can PET impact physician decision-making when no effective treatment exists?
  - Example: Pharmacologic testing to predict warfarin responsiveness
    - Analytic validity
    - Clinical validity
    - Clinical utility
    - Retrospective observational data is currently available. Is a prospective RCT necessary to demonstrate clinical utility?

# Acceptable Thresholds of Clinical Evidence (cont.):



- Clinical evidence must be sufficient to conclude that treatment is safe, relative to other available treatments, including active observation. If evidence of safety is lacking, limited or conflicting, no coverage.
  - Example: lung volume reduction surgery to treat emphysema
- Threshold of clinical evidence is lower when
  - Other effective treatments are not available
  - Condition is life-threatening
  - Prevalence of condition is too low for development of a body of clinical evidence
  - Treatment does not lend itself to a randomized controlled trial
  - Cost-effectiveness data supports new technology
    - Example: ranibizumab vs. bevacizumab to treat age-related macular degeneration. \$150/dose vs. \$2,000/dose (approx.)

## Additional Issues to Consider:

- Coverage for certain services should be limited to physicians and facilities with demonstrated experience and expertise.
  - Organ and tissue transplants
  - Artificial hearts/left ventricular assist devices
    - Bridge to transplant
    - Destination therapy
  - Transcatheter aortic valve placement
- Threshold of evidence for new indications for existing technology should be high.
  - Example: bevacizumab to treat breast cancer
- Threshold of evidence for safety and effectiveness of existing technology in new site of service should be high.
  - Example: optical colonoscopy, minimally invasive treatments for BPH in physician office vs. facility

# Implications for Medicare Coverage Policy:



- Ensuring appropriate access to clinical innovation is not just a coverage issue
- It is important to have programs that manage the introduction and spread of clinical innovations, while also generating knowledge and advancing science
- Such programs involve multiple components and tools, working in concert, with defined accountabilities, timelines, and measures of success
- Evidentiary thresholds may vary when:
  - Other effective treatments are not available
  - Condition is life-threatening
  - Prevalence of condition is too low for development of a body of clinical evidence
  - Treatment does not lend itself to a randomized controlled trial
  - Cost-effectiveness data supports new technology

# Issues to Consider in Linking Coverage to Evidence Development:



- Are the research questions fully specified?
- Is there a research/analytic protocol that is likely to answer the question(s) in a reasonable timeframe?
- Are the accountabilities explicit regarding the role of the sponsor of coverage, the deliverer of the service, the recipient of the service, and the developer/proponent?
- Are the expectations realistic regarding the scope, timeline, budget, and success measures of such a program?
- Has there been consideration of the perspectives of various regulatory bodies, payers and other stakeholders in such a program?

# Additional Considerations in Developing Evidence for New Clinical Innovations:



- Ethical
  - Are effective treatments already available?
  - Should effective treatments be withheld to find out if new treatments work?
- Technical
  - Can invasive studies be blinded to physician and patient?
- Population
  - Who should be eligible for study enrollment?
  - Can the study results be generalized to a larger population?

## Summary:

- It is possible to develop and deploy evidence review, coverage and medical management programs that promote appropriate access to new clinical innovations
- Such programs may have varying evidentiary thresholds based on a number of factors
- Optimal care for individuals and populations requires a multi-component program, with coverage as only one element
- Critical elements of a coverage with evidence development program include: clear specification of the question(s); clear accountabilities to ensure the answer(s); realistic scope and performance expectations

# Questions?

