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# ICER Value Framework

Overview of Conceptual Elements and Procedures  
Related to Value Assessment Reports and Appraisal  
Committee Voting at Public Meetings

January, 2018



INSTITUTE FOR CLINICAL  
AND ECONOMIC REVIEW

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# Institute for Clinical and Economic Review (ICER)

- **Independent** health technology assessment group
- Develop **publicly available value assessment reports** on medical tests, treatments, and delivery system innovations
  - [www.icer-review.org](http://www.icer-review.org)
- Convene regional independent **appraisal committees** for public hearings on each report

# Appraisal Committees Meet ~ 3x / Year



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# Guiding Principles

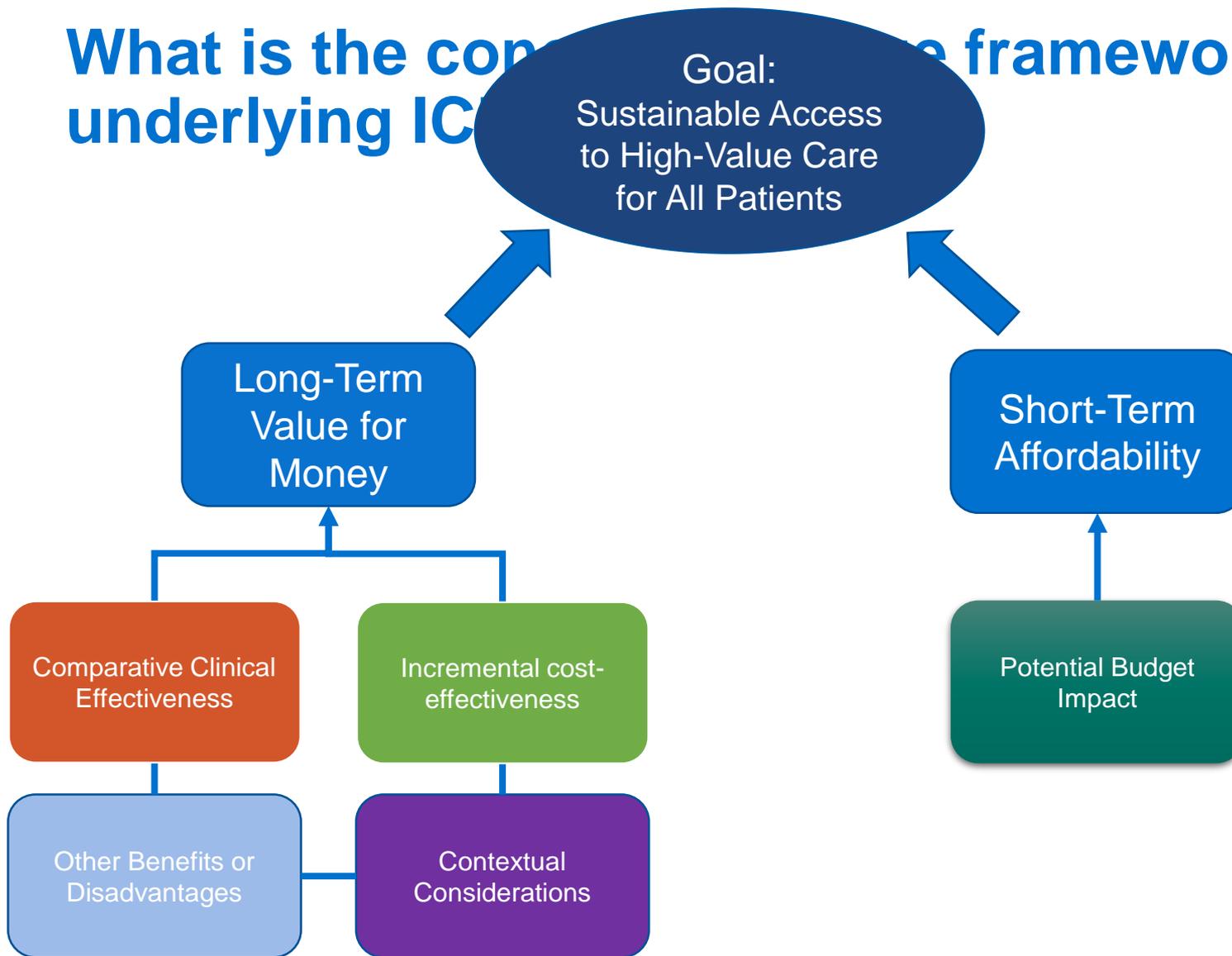
- Rigorous thinking about evidence can prevent the kind of waste that strains our ability to provide affordable care for all patients.
- Patients and the public should be more involved in assessments of value and discussions of how to apply evidence across the health system
- Evidence on added benefit, price, and insurance coverage
  - Alignment of these represents the grand bargain needed to foster innovation and create sustainable access to high-value care for all patients

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# The ICER Value Framework: Purpose

- Intended to make transparent how “value” is conceived of and evaluated in ICER reports
- Takes a “population” level perspective as opposed to trying to serve as a shared decision-making tool to be used by individual patients and their clinicians
- Even with its population-level focus, the ICER value framework seeks to encompass and reflect the experiences and values of patients

# What is the conceptual framework underlying ICER?



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# Summary of Key Features of ICER Value Assessment Framework

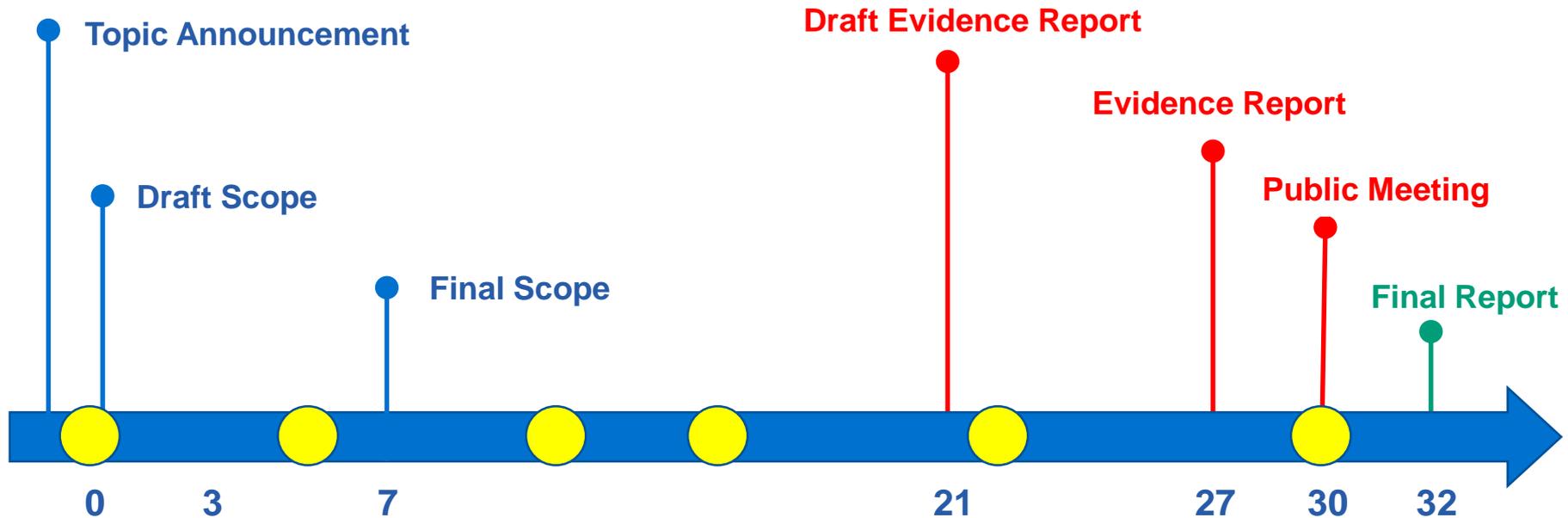
- Anchoring in long-term perspective on value
- Foundation in evidence on comparative clinical effectiveness
- Acceptance of multiple forms of evidence
- Recognition that what matters to patients is not limited to measured “clinical” outcomes
- Acknowledgment of the role of contextual considerations in value judgments
- Inclusion of information on potential budget impact

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# ICER report development process: 8 months

- Topic selection
- Scoping phase with Open Input
- Report development x 4 months
- Early feedback of draft results to manufacturers and patient groups
- Draft Evidence Report for public comment
- Evidence Report distributed to CEPAC/CTAF
- Public Meeting
- Final Evidence Report with votes, discussion, policy roundtable recommendations

# Typical 32-week report timeline



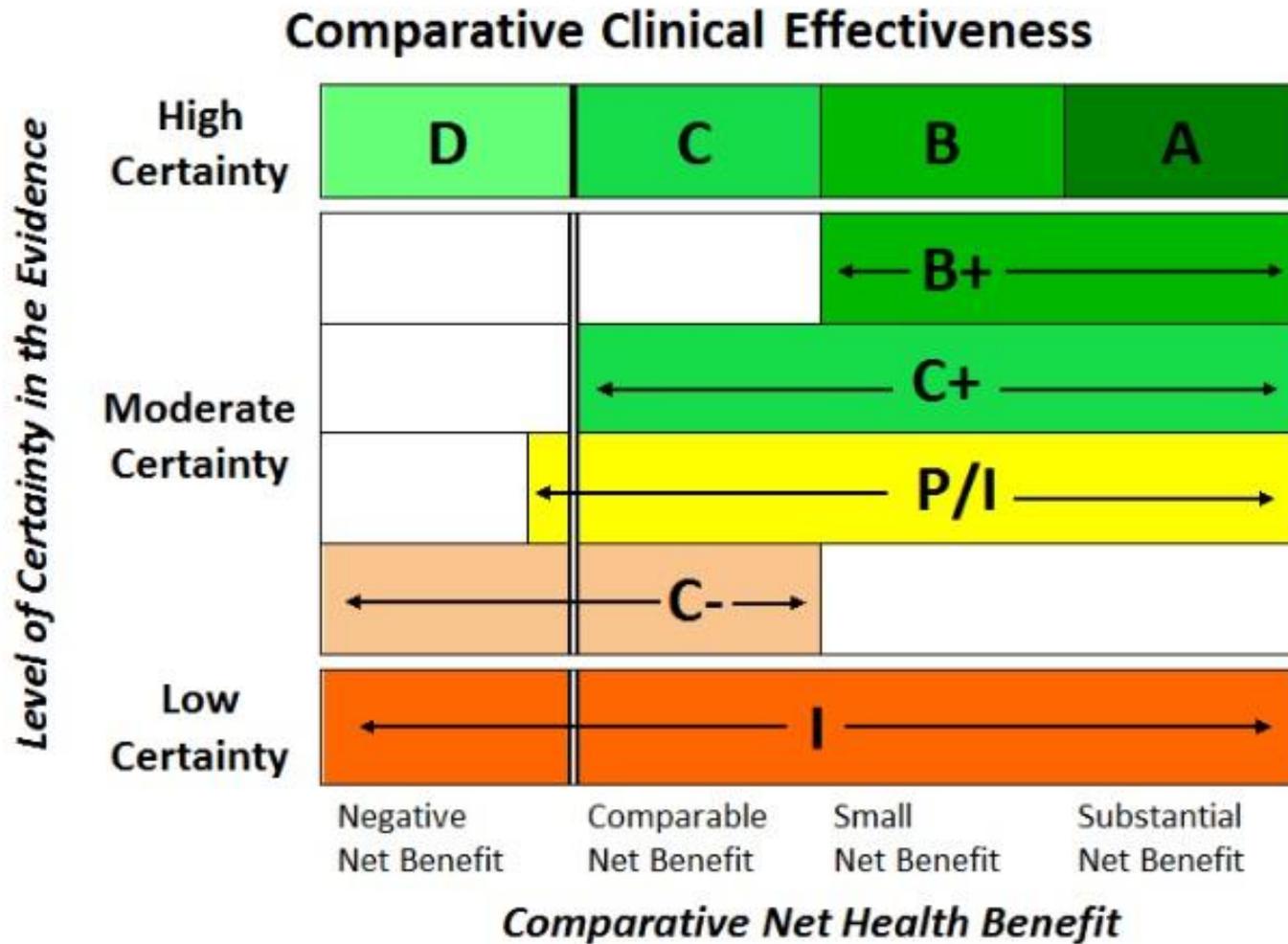
 = Touchpoints with Patient Groups

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# Comparative Clinical Effectiveness

- Systematic review of evidence
  - Patient groups inform what outcomes are important, differences across severity, time in disease course, etc.
  - Publicly available material and option for in-confidence material from manufacturers
  - Evidence sought from multiple sources, not just randomized controlled trials (RCTs)
    - Patient groups inform re: opportunities for using or generating real-world evidence
  - Indirect comparisons through network meta-analysis
  - Examination of heterogeneity of treatment effect
  - ICER EBM rating matrix to present prelim judgments

# ICER EBM Matrix



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# Incremental Cost-effectiveness

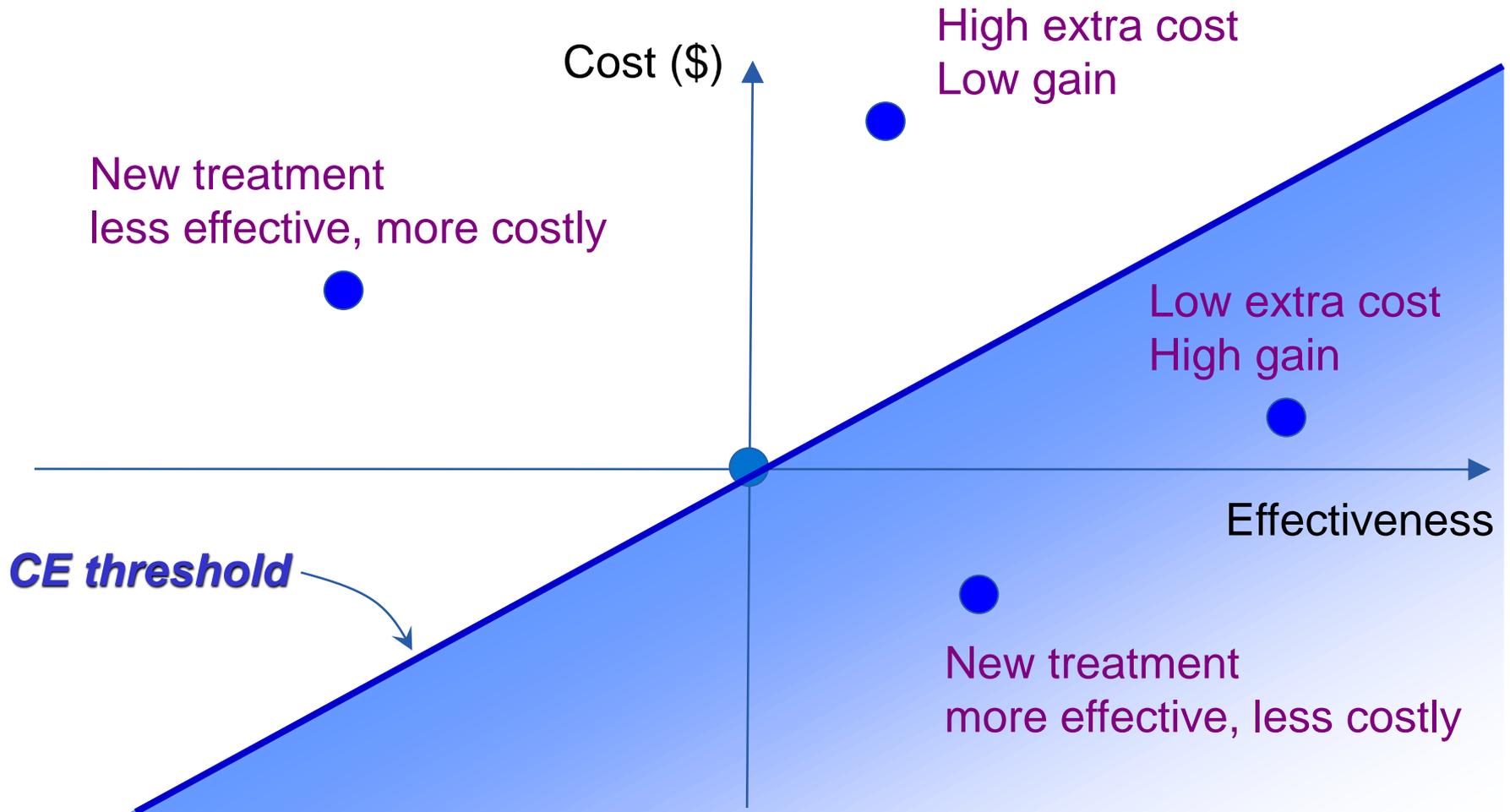
- Computer modeling comparing long-term clinical outcomes and costs for different treatment options
- Costs from health system perspective – all health care costs
  - Costs of drugs from estimates of discounted US prices
  - “Societal” perspective analyses presented when feasible
- Standard measures of health outcomes
  - Functional improvement, prevented negative outcomes
  - Additional life-years gained
  - Improvement in quality of life
  - Summing these for comparison across treatments:
    - Cost per quality-adjusted life year gained (QALY)

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# The core measure of health gain for patients: The Quality Adjusted Life Year (QALY)

- The **QALY** is recommended by US and international bodies
- A measure of improvement in patients' lives by measuring years of life weighted by an index of quality of life
- Numerical weights:
  - 1 = perfect health
  - 0 = dead
- Quality of life estimates for different health “states” derived from studies of patients, caregivers, or community members
- Sum of quality weight x duration = QALY

# Incremental cost-effectiveness ratios



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# Cost per QALY Thresholds

- Societal “willingness to pay”
  - WHO and ACC 1-3x per capita GDP
- Individual “willingness to pay”
  - ~2 times annual salary
- “Opportunity cost” for the health system
  - ~1x per capita GDP in UK, Latin America
- ICER: \$50,000-\$150,000 per QALY
  - Value-based price benchmark \$100-\$150K/QALY

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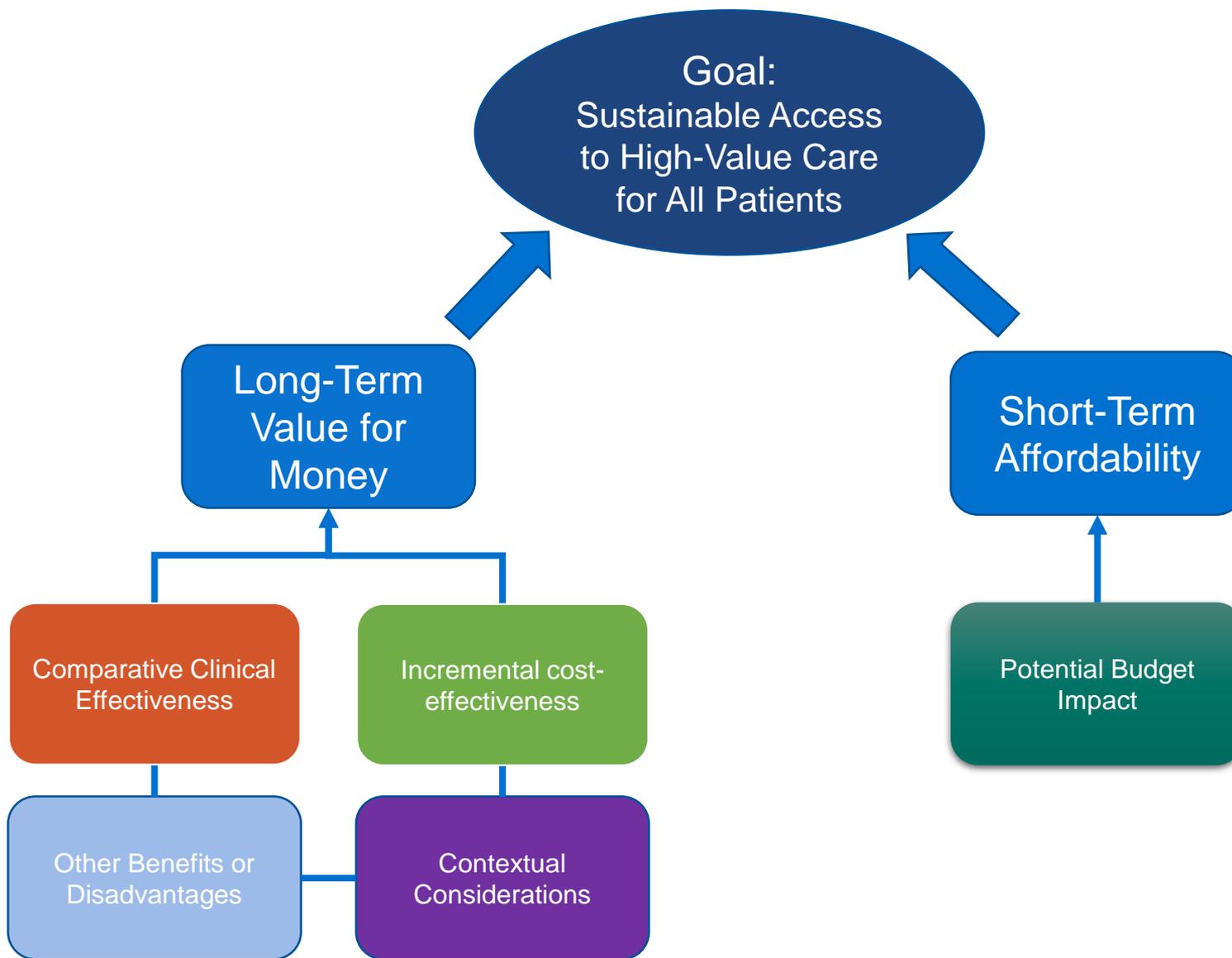
# Potential Other Benefits

- This intervention provides significant direct patient health benefits that are not adequately captured by the QALY.
- This intervention offers reduced complexity that will significantly improve patient outcomes.
- This intervention will reduce important health disparities across racial, ethnic, gender, socio-economic, or regional categories.
- This intervention will significantly reduce caregiver burden.
- This intervention offers a novel mechanism of action or approach that will allow successful treatment of many patients who have failed other available treatments.
- This intervention will have a significant impact on improving return to work and/or overall productivity.
- Other important benefits or disadvantages not otherwise described.

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# Contextual Considerations

- This intervention is intended for the care of individuals with a condition of particularly **high severity** in terms of impact on length of life and/or quality of life.
- This intervention is intended for the care of individuals with a condition that represents a particularly **high lifetime burden of illness**.
- This intervention is the **first to offer any improvement for patients** with this condition.
- Compared to “the comparator,” there is **significant uncertainty about the long-term risk of serious side effects** of this intervention.
- Compared to “the comparator,” there is **significant uncertainty about the magnitude or durability of the long-term benefits** of this intervention.
- There are additional contextual considerations that should have an important role in judgments of the value of this intervention.



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# Potential Budget Impact Threshold

- The purpose is to signal to stakeholders and policy makers when the amount of added health care costs associated with a new service – even one with good long-term value -- may be difficult for the health system to absorb over the short term without displacing other needed services or contributing to rapid growth in health care insurance costs that threaten sustainable access to high-value care for all patients.
- Example: Sovaldi for Hepatitis C
- NOT A BUDGET CAP!

# Potential Budget Impact threshold 2017-2018

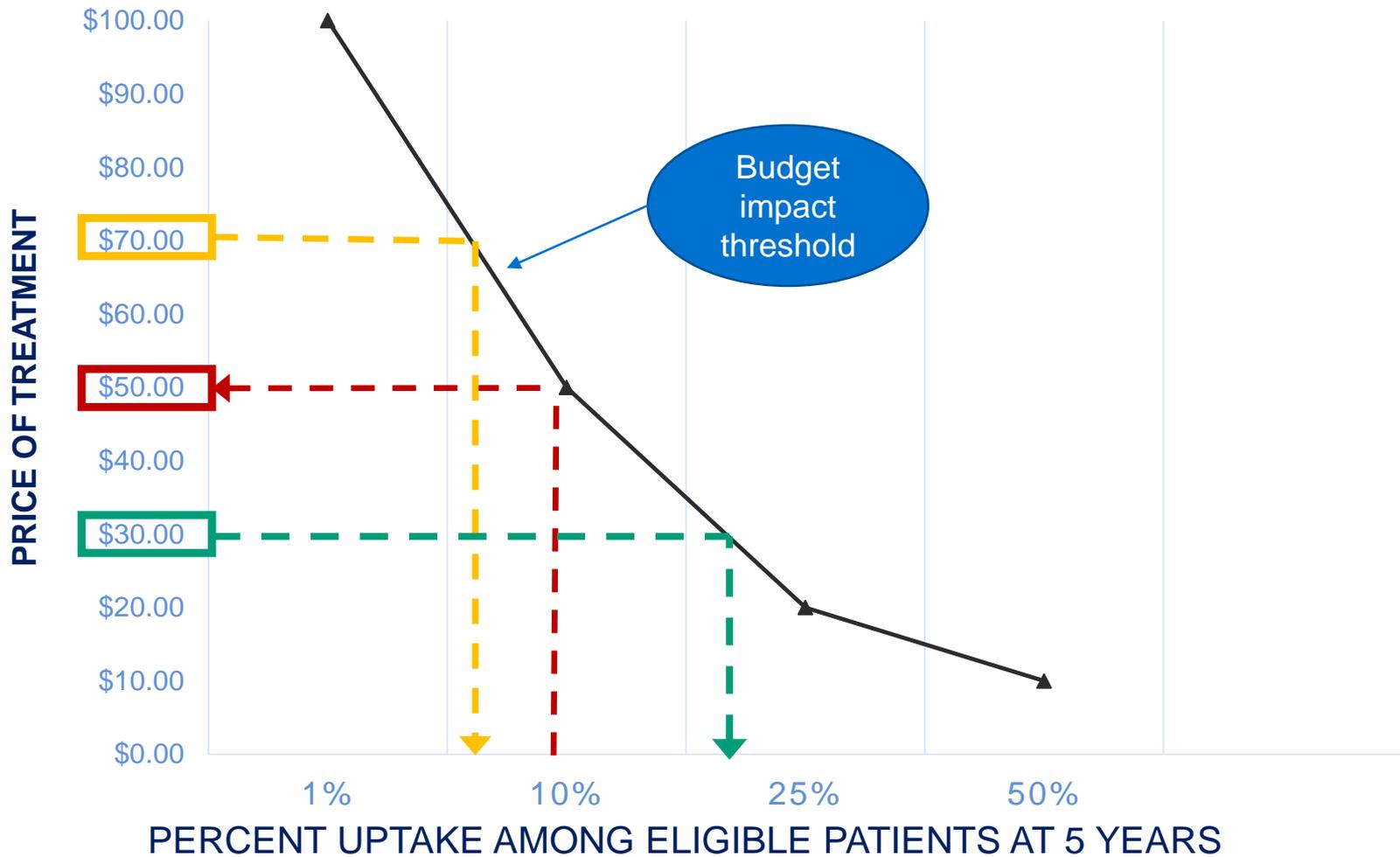
**NOTE: ICER's Potential Budget Impact threshold has been updated for 2018-2019 and will be applied to all reviews for which a draft report has not been published by May 2018. The new thresholds and calculations are available on the following slide.**

Item	Parameter	2017-2018 Estimate	Source
1	Growth in US GDP, 2017 (est.) +1%	3.20%	World Bank, 2016
2	Total personal medical health care spending	\$2.71 trillion	CMS NHE, 2016
3	Contribution of drug spending to total health care spending	17.7%	CMS NHE, 2016; Altarum Institute, 2014
4	Contribution of drug spending to total health care spending	\$479 billion	Calculation (Row 2 x Row 3)
5	Annual threshold for net health care cost growth for ALL drugs	\$15.3 billion	Calculation (Row 1 x Row 4)
6	Average annual number of new molecular entity approvals	33.5	FDA, 2016
7	Annual threshold for average cost growth per individual new molecular entity	\$457.5 million	Calculation (Row 5 ÷ Row 6)
8	Annual threshold for estimated potential budget impact for each individual new molecular entity	\$915 million	Calculation (doubling of Row 7)

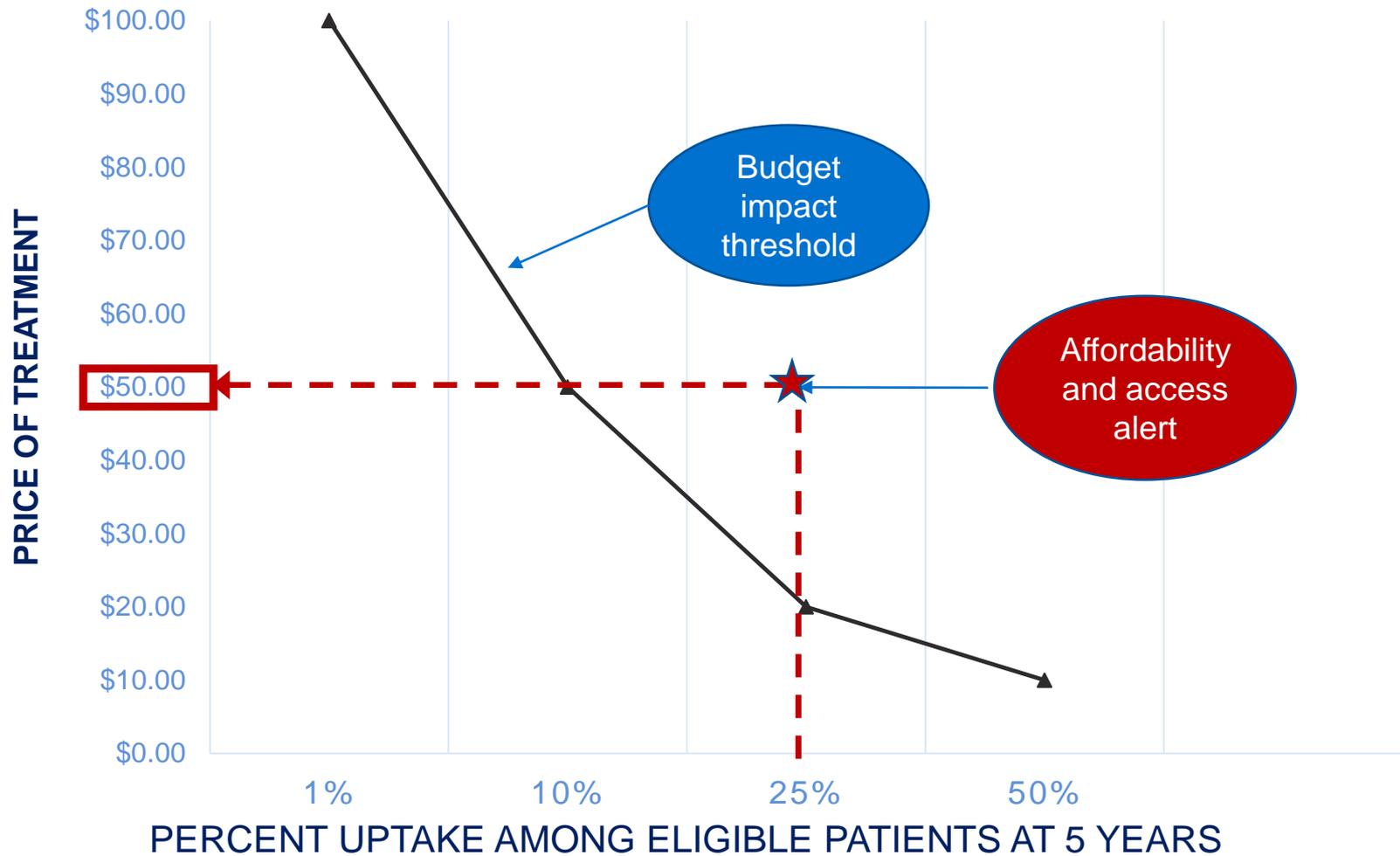
# NEW: Potential Budget Impact threshold 2018-2019

Item	Parameter	Estimate	Source
1	Growth in US GDP, 2018 (est.) +1%	3.5%	World Bank, 2018
2	Total personal medical health care spending, 2017 (\$)	\$2.88 trillion	CMS NHE, 2018
3	Contribution of drug spending to total health care spending (%)	17.0%	CMS National Health Expenditures (NHE), 2018; Altarum Institute, 2017
4	Contribution of drug spending to total health care spending, 2016 (\$) (Row 2 x Row 3)	\$481 billion	Calculation
5	Annual threshold for net health care cost growth for ALL drugs (Row 1 x Row 4)	\$16.8 billion	Calculation
6	Average annual number of new molecular entity approvals, 2016-2017	34	FDA, 2018
7	Annual threshold for average cost growth per individual new molecular entity (Row 5 ÷ Row 6)	\$495.3 million	Calculation
8	Annual threshold for estimated potential budget impact for each individual new molecular entity (doubling of Row 7)	\$991 million	Calculation

# POTENTIAL BUDGET IMPACT SCENARIOS

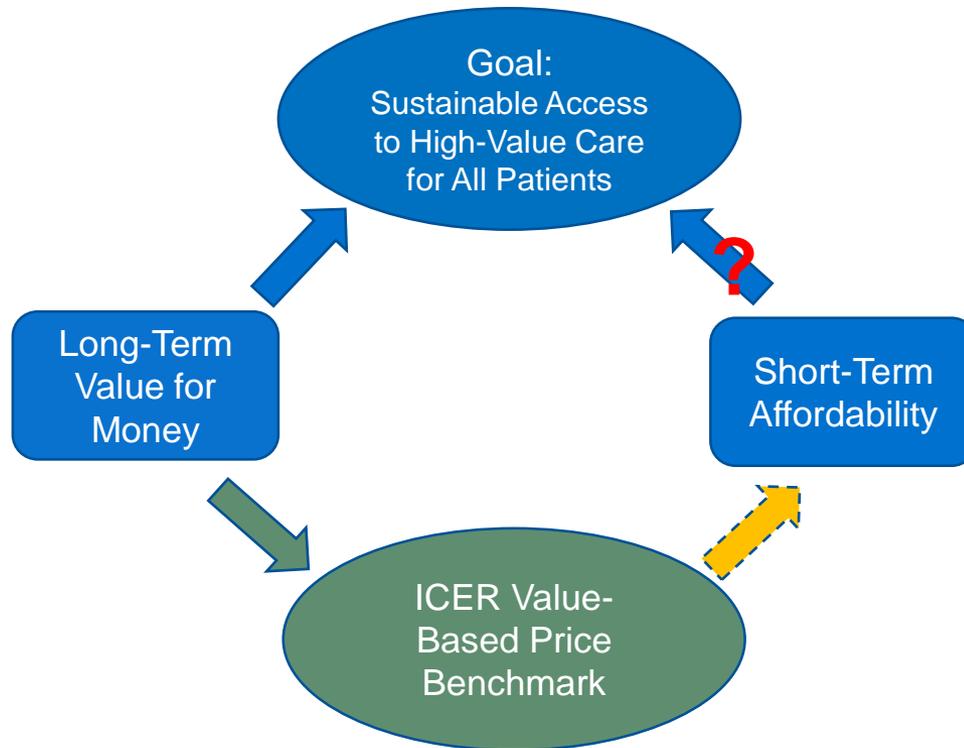


# POTENTIAL BUDGET IMPACT SCENARIOS



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# ICER value-based price benchmark



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# ICER Reports and Value-Based Pricing

Drug category	Discount from Prices After Rebate to Meet ICER Value-based Price Range
Psoriasis	5%
Multiple sclerosis	25%
Rheumatoid arthritis	15%
Atopic dermatitis	0%
Osteoporosis	50%-80%
TKIs for lung cancer	0%
PD-1s for lung cancer	50%
Abuse-deterrent opioids	40%
Ovarian cancer PARP drugs	50% for maintenance therapy

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# Modifications for “Treatments of Ultra-rare Disorders (URDs)”

- Why any modification?
  - Smaller populations and less well known natural history often leads to more uncertainty in clinical evidence
  - Frequently conditions of children associated with very high severity
  - Questions of whether traditional cost-effectiveness thresholds provide enough ROI

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# Key modifications for treatments of URDs

- Eligible treatments
  - Condition/label for less than approximately 10,000 individuals (higher than EU definition)
  - No ongoing or planned clinical trials for patient populations greater than 10,000
- No explicit change to different “standard” of evidence for judgments of comparative clinical effectiveness
- Instead, ICER will provide specific context regarding the potential challenges of generating evidence for these treatments
  - Conducting RCTs
  - Validating surrogate outcome measures
  - Obtaining long-term data on safety and on the durability of clinical benefit.
- Commonly used approach of evaluating treatments for URDs against historical controls will be highlighted.

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## Key modifications for treatments of URDs

- Cost-effectiveness thresholds included in the report expanded from \$50,000 per QALY to \$500,000 per QALY
- Value-based price benchmark remains \$100-\$150K/QALY but specific language to be included about decision-makers history of accepting higher thresholds
- When impact of treatment on benefits and costs outside the health system are substantial in relation to health system costs, societal perspective analyses will be presented in tandem with results from the health system perspective.

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## Key modifications for treatments of URDs

- Broader framework for other benefits and contextual considerations to reflect potential for impact on family, school, and community as well as infrastructure for screening and care of affected individuals
- Manufacturers invited to supply information on costs of research and development if they desire

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# Summary

- Same standards for evidence review of clinical effectiveness but more context provided
- Same approach to cost-effectiveness but presentation of broader threshold ranges
- Same core list of other benefits and contextual considerations with some amplification
- Same value-based price benchmark but added context indicating information on research and development costs and history of acceptance of higher prices

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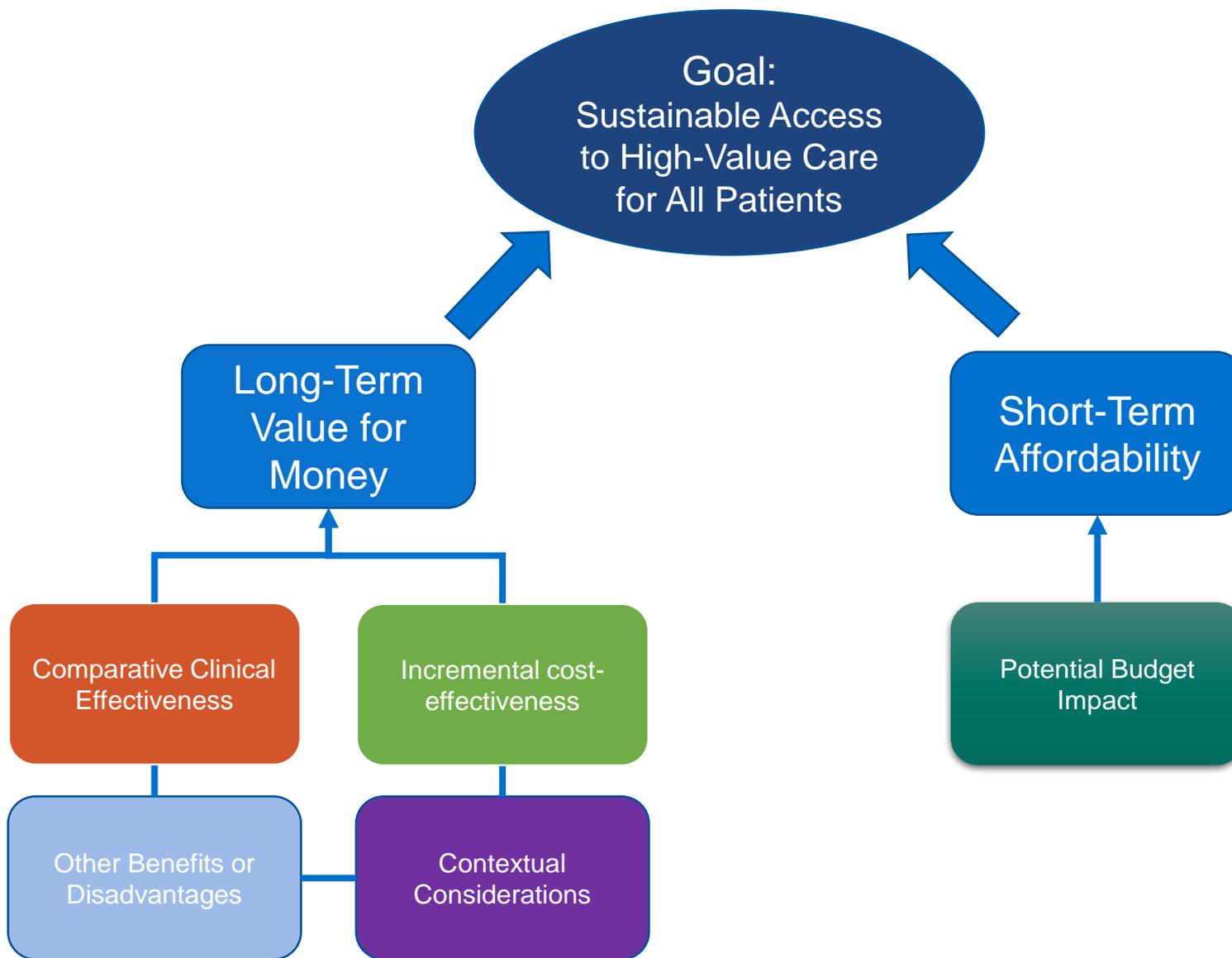
# Appraisal Committee meeting process

- CEPAC receives Evidence Report following public comment period on Draft Evidence Report
- Call with clinical experts 10-14 days before meeting
- Public meeting
  - Evidence synopsis on effectiveness and cost-effectiveness presented by lead reviewer
  - Patient representatives and clinical experts available to comment/respond to questions
  - Manufacturer comment
  - Patient and public comment
  - Discussion and voting on comparative clinical effectiveness, “other benefits and contextual considerations”, and value
  - Policy Roundtable discussion among key stakeholders and concluding statements from CTA/CEPAC

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# ICER Final Report

- Final report and associated summaries include
  - Results of appraisal committee voting with commentary
  - Synopsis of key themes of policy roundtable discussion
  - Policy perspectives and recommendations related to application of evidence to patient education, clinical care, insurance coverage, pricing, and future research needs



**Thank you**