How Do Prescription Drug PRICES Relate to Their VALUE?

Wednesday, September 11, 2019

12:00 – 1:00 PM

This webinar is being recorded. The recording and presentation will be shared with attendees.

Do We Really Need to Choose Between Affordability and Access?

Sarah K. Emond, MPP

September 11, 2019

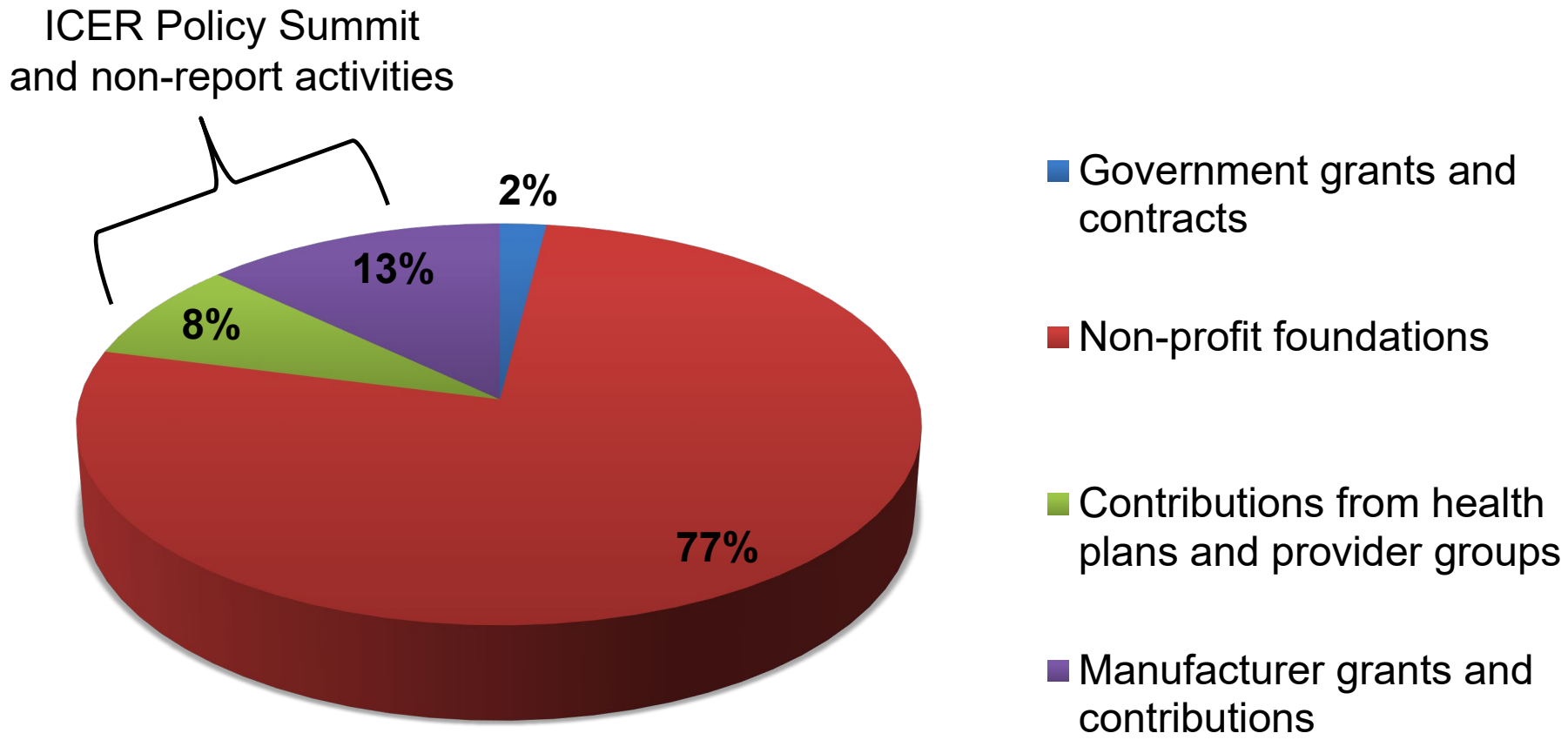
No.

Institute for Clinical and Economic Review (ICER)

- **Independent** health technology assessment group whose reviews are funded by non-profit foundations
- Develop **publicly available value assessment reports** on medical tests, treatments, and delivery system innovations for over 12 years
- Use cost-effectiveness analysis to determine **value-based price benchmarks**
- Convene regional independent **appraisal committees** for public hearings on each report

Independent Appraisal Committees

Sources of Funding, 2019



Why we do what we do

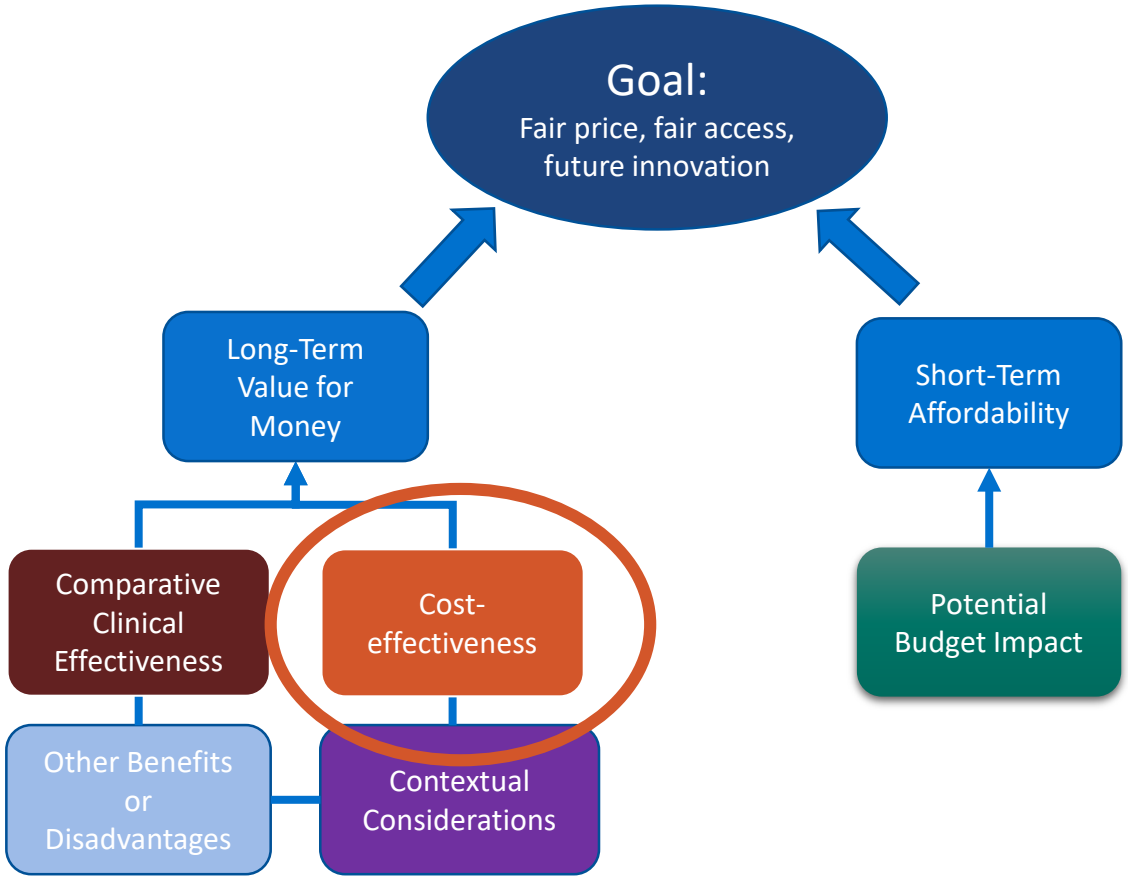
"I do not want to have to choose to help send my boys to college or have access… At what point will I, or other ATTR families, have to decide the best option for them as a family, is to stop taking (or not take) the drug and let the disease take it's natural progression." *Dustin, amyloidosis patient*

Fair Price, Fair Access, Future Innovation

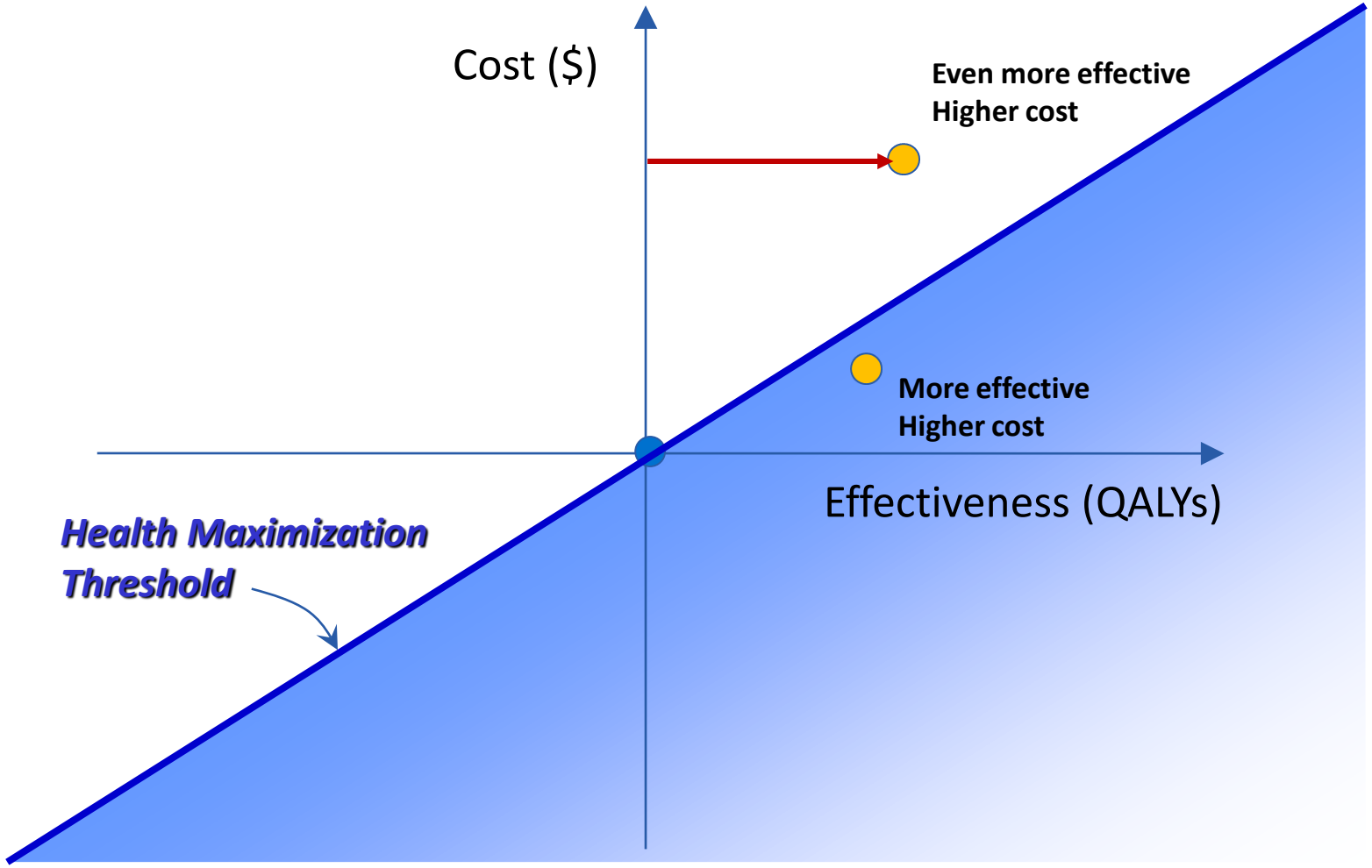
Why Do Comparative Effectiveness Research?

- FDA approval means “efficacy” not “effectiveness”
  - Little to no comparative data, often
- Patients and health systems struggle to know what options are of the highest value
  - Little to no info available on costs at point of service
- Health care spending continues to crowd-out other social spending priorities

ICER Value Assessment Framework



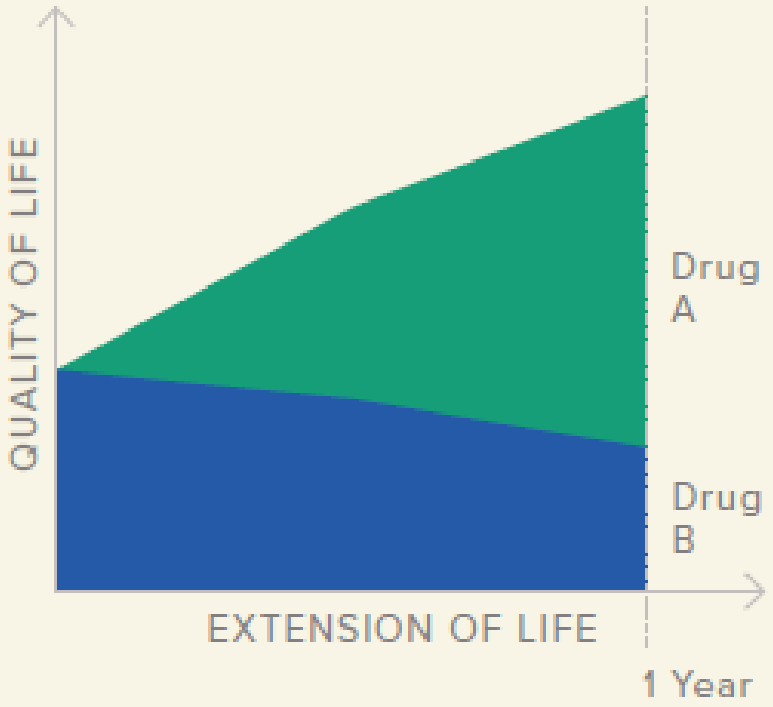
Determining value-based price benchmarks

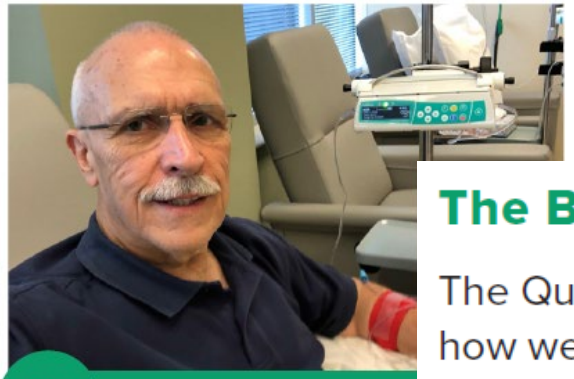


HOW THE QALY WORKS

On average, Drugs A & B both extend the lives of patients for one year, but Drug B has severe side effects.

Clinically superior, Drug A is credited with more QALYs gained and, many would argue, deserves a higher price.





I have an incurable blood
and rely on pharmaceutical in
to stay alive. ICER's independer
effectiveness analysis—built on
QALY—is one useful input to help us
think through what a fair price would be.
ICER works to ensure drugs are
according to the value they brin
patients, which helps us get affo
access to the care we need."

David Mitchell, President and I
Patients For Affordable Drugs

The Best Measure of Improved Outcomes for Patients

The Quality-Adjusted Life Year (QALY) is the gold standard for measuring how well a medical treatment improves and lengthens patients' lives, and therefore has served as a fundamental component of cost-effectiveness analyses in the U.S. and around the world for more than 30 years.

Supplementing the QALY, Not Replacing It

To maintain the ability of cost-effectiveness analyses to reflect the full benefits that treatments may have on quality of life, ICER will continue to calculate each treatment's QALY gained. The cost per QALY gained remains the best way for policymakers to understand how well the price of a treatment lines up with its benefits and risks for patients.

WITH evLYG, ONE ADDED YEAR
= ONE ADDED YEAR

Patients Engaged in Every Review

- Patient groups notified before review is announced
- Patients and patient groups give input into review scope – population, interventions, comparators, outcomes
- Patient input guides development of other benefits and contextual considerations
- Patients review the preliminary economic model, draft report, draft voting questions
- Patients front and center at entire public meeting, offer public comment, and contribute to the policy roundtable

ICER's Value-based Price Benchmarks

ICER's Value-based Price Benchmarks (2018)

Drug category	Recommended Discount*
Luxturna for childhood blindness	50-75%
Kymriah (CAR-T) for ALL	0%
Yescarta (CAR-T) for NHL	28%-11%
Hemlibra for hemophilia A	Cost-saving
Cystic Fibrosis	72%-77%
CGRPs for migraine prevention	25%-46%
Elagolix for endometriosis	15%-25%

Drug category	Recommended Discount*
Apalutamide, Xtandi, Abiraterone for prostate cancer	0% (apalutamide)
Psoriasis IL-23s and Cimzia	37%-57%
Inotersen, patisiran (amyloidosis)	90%-95%
Hereditary Angioedema	28%-68%
Opioid Use Disorder (new agents)	53%-69%
Eosinophilic asthma biologics	62%-80%

** For new drugs, discount from list price needed to meet common thresholds of cost-effectiveness. For drugs already in use, discount is from **post-rebate price***

Takeaways from RA, MS and Migraine reviews

- 2017 review of drugs for RA – all drugs offer clinical benefit to patients; no drugs had net prices in line that with clinical benefit; discounts needed ranged from 29% to 69% (update underway)
- 2017 review of drugs for MS – all drugs offered clinical benefit for patients; one drug had net price in line with that benefit, the other 14 did not; if price increases rolled back to 2011 levels, several drugs were high value
- 2018 review of drugs for chronic migraine – all manufacturers chose launch prices in line with ICER's evaluation of high value

Gene and Cell Therapies: The Future is Now

- By 2024, cost of gene therapies expected to reach more than $16 billion (EvaluatePharma)
- Health system currently set up to pay for chronic, long-term therapies, not one-time potential cures
- New approaches to value determination and payment needed
  - ICER is finalizing adaptations to its value assessment framework to account for these single or short-term transformative therapies
- Recent approval of Zolgensma for spinal muscular atrophy offers a case study

ICER's Review of Zolgensma

- One-time gene therapy for fatal childhood condition
- Adequate evidence to show significant net health benefit in patients, even with small number of patients studied
- Fair price, based on commonly-accepted health maximization thresholds, ranges from $1.1m to $2.1m
- Novartis cited ICER's analysis in its discussion of price
- Novartis offering pay-over-time arrangements
- Responsible pricing should lead to fair access for patients

Use of ICER assessments

- **For drug makers and payers:** helps negotiation over prices in conjunction with fair access
- **For payers and provider groups:** helps guide coverage decisions and pricing negotiations
- **For policymakers:** independent evaluation of value and suggested value-based prices figure in multiple proposals

Use of ICER Assessments: Drug Makers and Payers

- **Dupixent for severe atopic dermatitis, 2017**
- **Praluent for high cholesterol, 2018**
  - New data shared with ICER before public release
  - ICER updated its value-based price benchmarks
  - Drug makers commit publicly to ICER price range in conjunction with "streamlined" access from payers
  - Express Scripts and drug makers announce a deal
- **Vascepa for cardiovascular disease, 2019**
- **Zolgensma for spinal muscular atrophy, 2019**

Use of ICER Assessments: Payers and Providers

- **Medicaid programs: New York**
  - 2017 law establishing drug spend target
  - If spending ahead of trend allowed to identify drugs for evaluation of value
  - If companies and Medicaid cannot come to agreement on lower price Medicaid can trigger public process to determine specific target price for supplemental rebate
  - 2018 experience and Orkambi

Threshold Price Analysis for Orkambi

Annual cost at WAC	Annual Price to Achieve…					
	$50,000 /QALY	$100,000 /QALY	$150,000 /QALY	$200,000 /QALY	$300,000 /QALY	$500,000 /QALY
$272,886	$58,790	$70,991	$83,193	$95,394	$119,797	$168,604

- New York Medicaid DURB deliberation and vote

Use of ICER Assessments: New York Medicaid

Medicaid Is Right to Demand Lower Drug Prices

New York State can’t afford $250,000 a year for one cystic fibrosis medicine.

By Peter B. Bach

Bloomberg News

May 1, 2018

Use of ICER Assessments

- **Medicaid programs**
- **VA**
  - Monthly calls to debrief reports and potential applications
  - Pipeline discussion
  - Development of VA budget impact threshold
- **Private payers and PBMs**

Use of ICER Assessments: Payers and Purchasers

- Ongoing use by most payers to inform internal process
- CVS new benefit design for self-insured employers
    - Newly launched drugs, breakthrough drugs excluded
    - After negotiation, drugs that fail to reach a cost-effectiveness level of $100K/QALY can be designated a non-covered benefit
- Further details
    - CVS considers $100K/QALY as top of a range from $50-$100K/QALY
    - Will use $150K/QALY for treatments of ultra-orphan disorders
    - Experience to date: Two migraine prevention drugs ***added*** to formulary because pricing met ICER benchmarks
- Purchasers increasingly interested in new models for getting maximum value for the drug spend, including fair price/fair access, and empowering employees

Systematic Application of Value Assessment in Benefit Designs and Payment Policy

- Option 1 (private payers, purchasers): Special tier, step therapy, or exclusion for drugs whose best negotiated price remains above the value-based price benchmark; can be woven into rebate-free formulary structure
- Option 2 (private or public payers, purchasers): Include drugs on formulary but only **pay up to** the value-based price benchmark
- Option 3 (public payers): Allow CMS and/or Part D plans to **negotiate with price arbitration fallback**; value assessment reports used to create spectrum for proposals or as part of proposals to arbitrator

All options could be used for "all" drugs or only a subset

ICER as Part of the Policy Landscape

- States are laboratories of new approaches to negotiation, access, payment; ICER's research supports those efforts
- More and more manufacturers see value-based pricing as key to ensuring patient access; ICER's research is trusted, independent source of value-based pricing
- Purchasers will have more active participation in deciding benefit structures; ICER's research can inform formulary development
- Federal government efforts? Anyone's guess….

On the Horizon

- Trend of drugs focused on diseases affecting smaller patient populations will continue
- Cell and gene therapy “revolution”
- Unmet needs in hemophilia, sick cell disease may be met
- Prospects dim for Alzheimer’s

Unsupported Price Increases

- Why now?
- Annual report of up to 13 drugs that have experienced substantial price increases over a two-year time period
- Methods:
  - Identify drugs with price changes (WAC) over 2-year period in excess of Medical CPI
  - Adjust for net price increases
  - Narrow to top 10 in terms of budget impact
  - Add 3 additional drugs to list based on public comment
- ICER will review changes in the evidence base for these drugs, and report on whether potential evidentiary support for price increases was found
- Not intended to determine whether a price increase for a drug is fully justified by new clinical evidence
- Analysis is focused on whether or not substantial new evidence exists that could justify a price increase

Do we really need to choose between affordability and access?

- No:
  - Systematic application of independent analyses of fair pricing to formularies and benefit designs will lead to **fair access for patients**
  - Applications by **public insurers and the VA** expected to continue/grow
  - As **gene therapies** at >$1-2M arrive, public and political interest in value-based pricing will continue to gain momentum
  - **Manufacturers** that see value-based pricing as a core component of fair access for patients will look to independent assessments
  - Value-based pricing is core component of **policymaker efforts** to address drug pricing and value

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