Ameet Sarpatwari, PhD, JD

Assistant Professor of Medicine

Harvard Medical School

Brigham and Women's Hospital

@ameetsarpatwari

Paying for Value

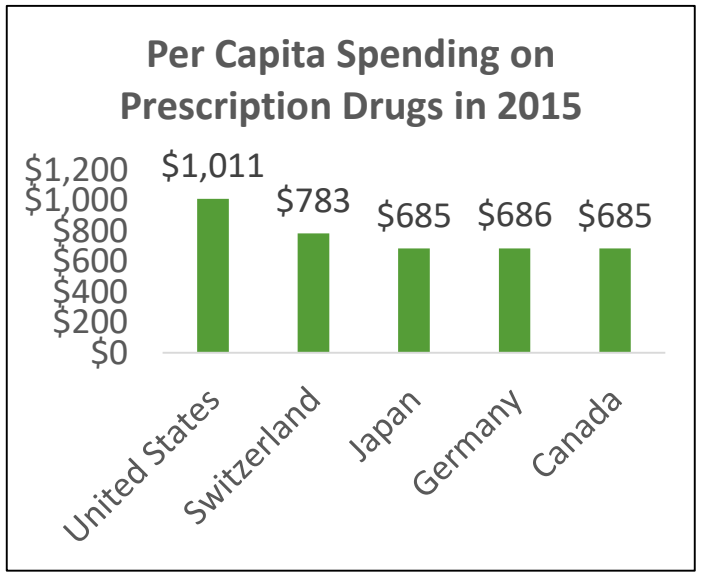
Impetus for Value-Based Drug Pricing

- Rising net US prescription drug spending
  - 2016: retail and non-retail spending=$472 billion
    -Altarum Institute (2018).
  - 2017-2026: projected 6% annual increase in net retail spending
    - Faster than any other major health care good or service
      -Cuckler et al. Health Aff (2018).
- Driven by rising drug prices
  - Median annual list price of new cancer medication
    - 2013→2017: $87,000 (2017 dollars) → >$160,000
      -IQVIA (2018).

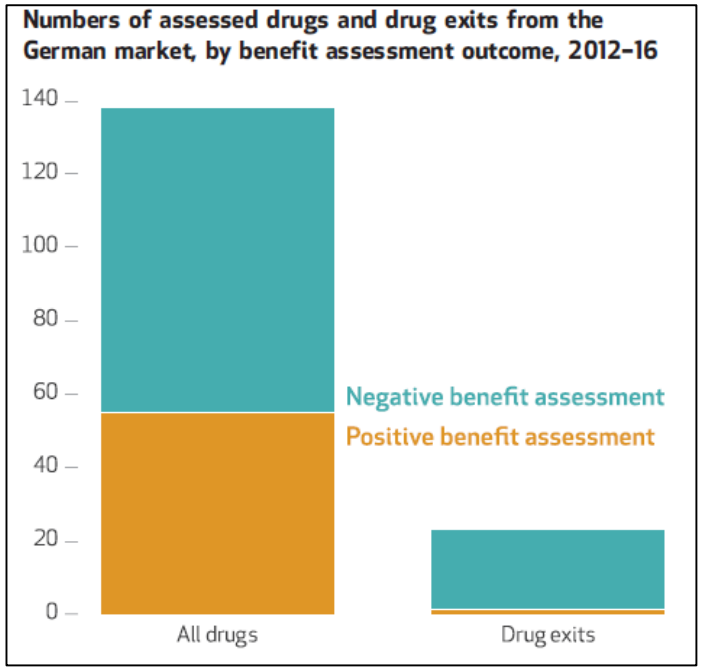
CNBC

FDA approves Novartis' $2.1 million gene therapy — making it the world's most expensive drug

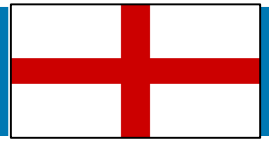
- Little correlation between price and clinical benefit
  - Of 138 new drugs evaluated between 2012 and 2016 under the German AMNOG process, 83 (60%) had a negative benefit assessment
    -Stern et al. Health Aff (2018).
- Goals: efficient spending, signaling to would-be innovators



-OECD (2017).



European Approaches to Value-Based Drug Pricing

	 England	 Germany

Value Measure	Comparative cost-effectiveness
	• Quality-adjusted life year (QALY)
	  • 1 QALY=1 year of perfect health
	• Incremental cost-effectiveness ratio
	  • $(Cost_1-Cost_o)/(QALY_1-QALY_o)$
	• Added cost per QALY: loose thresholds
	  • Generally: £20,000-£30,000
	  • End-of-life care: £50,000
	  • Rare diseases: £100,000
		Comparative effectiveness
		• Categories
		  • Major added benefit*
		  • Considerable added benefit*
		  • Minor added benefit*
		  • Nonquantifiable added benefit*
		  • No evidence of added benefit
		  • Less benefit
		*=positive benefit assessment
		• Price premium negotiated for positive assessment; reference pricing for negative assessment

Arbitration Mechanism	No	Yes
Timing	Pre-market entry	Post-market entry (effective after first year)
Assessor	National Institute for Health and Care Excellence	Institute for Quality and Efficiency in Health Care

US Examples of Value-Based Drug Pricing

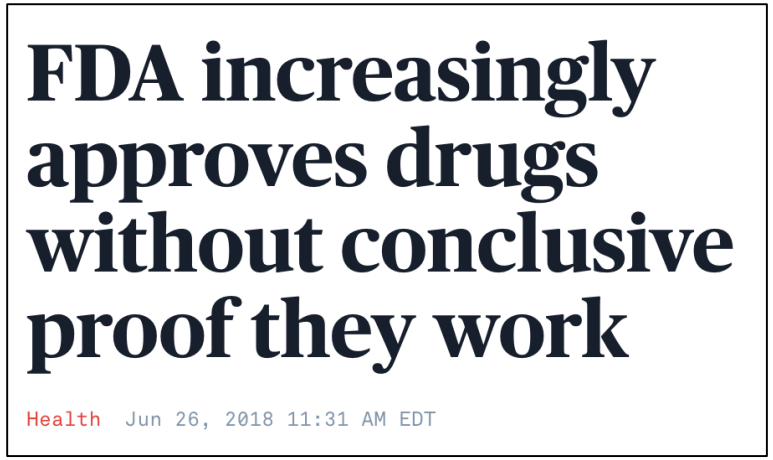
Model	Manufacturer	Payer	Drug	Details
Comparative cost-effectiveness	Regeneron & Sanofi	Market-wide	Dupilumab (Dupixent)	• Priced at $37,000 per year based on Institute for Clinical and Economic Review (ICER) assessment • Ensured limited utilization management • 2018 sales=$922 million
Indication-specific pricing*	Multiple	Express Scripts	Oral anticancer drugs	• Weighted-average based on estimates of indication-specific use
Outcomes-based pricing	Amgen	Harvard Pilgrim	Evolocumab (Repatha)	• Refunds payment for patients who have a heart attack or stroke after at least six months of taking the drug as prescribed

*=Can be a subset of comparative cost-effectiveness pricing.

Logistical Challenges

- Purported challenge: Medicaid best price rule
  - Mandatory 23.1% rebate off list price or best price, whichever offers greater savings
  - Concern for indication-specific pricing: best price for one indication applied to all indications
    - BUT weighted-average pricing possible
  - Concern for outcomes-based pricing: best price=$0
    - BUT rebating based on performance across population possible

-Sachs et al. JHPPL (2018).


FDA increasingly approves drugs without conclusive proof they work

Health Jun 26, 2018 11:31 AM EDT

-PBS News Hour (2018).

- Other challenges
  - Limited evidence-base for many new drugs, especially gene therapy “cures”
    - Of 68 cancer indications approved by the EMA between 2009 and 2013, only 35 (51%) had shown improvement in overall survival or quality of life (median 5.4 years of follow-up time)

-Davis et al. BMJ (2017).

  - Limited infrastructure to track relevant outcomes (e.g., information available through claims)

-Kesselheim & Seeley et al. Commonwealth Fund Issue Brief (2017).

Theoretical Considerations

- Appropriateness of paying for drugs on the basis of value
  - We do not pay physicians based on the value of their care
  - Are drugs unique compared to other health care goods and services?
- Appropriateness of allowing manufacturers to extract entirety of value
  - About 25% of small-molecule drugs approved between 2008 and 2017 were based in part on patents or other late-stage contributions from publicly-supported research institutions.

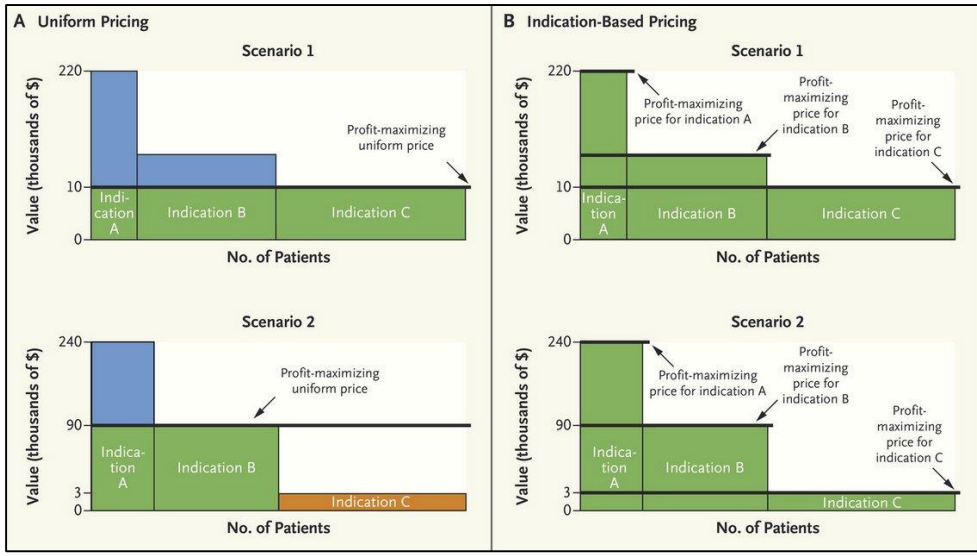
-Nayak et al. BMJ (In Press).

- Comparative cost-effectiveness
  - Problematic if comparator offers little benefit but is highly priced
- Indication-specific pricing
  - "…indication-based pricing results in higher prices for patients who benefit the most, higher utilization by patients who benefit least, higher overall spending, and higher manufacturer profits."
  - But assumes prices not anchored to value
- Outcomes-based pricing
  - Possibility of illusory savings if price not anchored to value
  - Possibility of gaming of outcomes

FIRST OPINION

My hand surgeon should have been paid $4.5 billion for fixing my broken wrist, not $1,000

-Stat News (2019).



-Chandra & Garthwaite. NEJM (2017).