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
  - 2017-2026: projected 6% annual increase in net retail spending
    - Faster than any other major health care good or service
  - Driven by rising drug prices

- Median annual list price of new cancer medication
  - 2013→2017: $87,000 (2017 dollars) → >$160,000

- 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

- Goals: efficient spending, signaling to would-be innovators

Per Capita Spending on Prescription Drugs in 2015


Numbers of assessed drugs and drug exits from the German market, by benefit assessment outcome, 2012-16

## European Approaches to Value-Based Drug Pricing

<table>
<thead>
<tr>
<th>Value Measure</th>
<th>England</th>
<th>Germany</th>
</tr>
</thead>
</table>
| **Comparative cost-effectiveness** | - Quality-adjusted life year (QALY)  
  • 1 QALY = 1 year of perfect health  
  - Incremental cost-effectiveness ratio  
    • \((\text{Cost}_1 - \text{Cost}_0) / (\text{QALY}_1 - \text{QALY}_0)\)  
  - 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

<table>
<thead>
<tr>
<th>Model</th>
<th>Manufacturer</th>
<th>Payer</th>
<th>Drug</th>
<th>Details</th>
</tr>
</thead>
</table>
| 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

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

-Chandra & Garthwaite. NEJM (2017).

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