Is the “Cure” Worth the Cost?
Multiple myeloma survival improvements

5- & 10-YEAR RELATIVE SURVIVAL

Pulte et al. Recent improvement in survival of patients with multiple myeloma: variation by ethnicity. Leukemia & Lymphoma Vol. 55, Iss. 5, 2014
The Increasing Costs of Health Care Squeeze Out Other Public Spending Priorities, Too

STATE BUDGET, FY2001 VS. FY2011 (BILLIONS OF DOLLARS)

NOTE: Dollar figures are inflation adjusted using a measure specific to government spending as developed by the U.S. Bureau of Labor and Statistics.
SOURCE: Massachusetts Budget and Policy Center Budget Browser.
Conceptual Approaches to “Fair” Pricing

• “Free market”/supply and demand

• Costs of development and production plus “reasonable” profit

• Added “value” to patients and health systems
ICER Value Assessment Framework

<table>
<thead>
<tr>
<th>Comparative clinical effectiveness</th>
<th>Incremental cost for better clinical outcomes (long-term)</th>
<th>Other benefits or disadvantages</th>
<th>Contextual considerations</th>
<th>“Long-Term Value for Money”</th>
</tr>
</thead>
<tbody>
<tr>
<td>Public discussion and vote</td>
<td>Possible need for extra steps to improve affordability</td>
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<td>HIGH</td>
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<td>INTERMEDIATE</td>
<td>LOW</td>
<td></td>
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</tbody>
</table>

Potential Health System Budget Impact (short-term)

- Possible need for extra steps to improve affordability

Maximizing “Health System Value”

- Price reduction
- Different payment mechanisms
- Prioritizing patient access
- Reallocating health system resources
- Obtaining outside resources

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Cost per QALY Thresholds

- **Societal “willingness to pay”**
  - World Health Organization 1-3x per capita GDP ($50,000-$150,000)

- **Individual “willingness to pay”**
  - ~2 times annual salary ($100,000)

- “Opportunity cost” for the health system
  - ~1x per capita GDP at most in UK, Latin America
  - Extrapolated ~$50,000 per QALY in the US

- **ICER:** $100,000-$150,000 per QALY
# ICER Value Assessment Framework

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<tr>
<td>Public discussion and vote</td>
<td>? Possible need for extra steps to improve affordability</td>
<td>Public and Policy Roundtable to Consider Policy Options</td>
</tr>
<tr>
<td>HIGH</td>
<td></td>
<td>Price reduction</td>
</tr>
<tr>
<td>INTERMEDIATE</td>
<td></td>
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<td>Reallocating health system resources</td>
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<tr>
<td></td>
<td></td>
<td>Obtaining outside resources</td>
</tr>
</tbody>
</table>

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Potential Budget Impact and Value

• New treatments of good long-term value may still not be affordable in the short term
  – Could displace more valuable services (opportunity cost)
  – Could lead to short-term increases in overall health spending that push insurance premiums beyond affordable range
Potential Budget Impact Threshold

• What level of potential budget impact suggests a possible concern about short-term affordability?

• Theoretical basis of a potential budget impact threshold based on state legislation and ACA

  – The amount of net cost increase per individual new drug that would contribute to growth in overall health care spending greater than the anticipated growth in national GDP + 1%

  – A potential budget impact greater than this level serves as an “alarm bell” for consideration of whether utilization management, lower prices, reallocation of resources, etc. are needed
# Summary of 2015-2016 Potential Budget Impact Threshold Calculations

<table>
<thead>
<tr>
<th>Item</th>
<th>Parameter</th>
<th>Estimate (Drugs)</th>
<th>Estimate (Devices)</th>
<th>Source</th>
</tr>
</thead>
<tbody>
<tr>
<td>1</td>
<td>Growth in US GDP, 2015-2016 (est.) +1%</td>
<td>3.75%</td>
<td>3.75%</td>
<td>World Bank, 2015</td>
</tr>
<tr>
<td>2</td>
<td>Total health care spending ($)</td>
<td>$3.08 trillion</td>
<td>$3.08 trillion</td>
<td>CMS NHE, 2014</td>
</tr>
<tr>
<td>3</td>
<td>Contribution of drug/device spending to total health care spending (%)</td>
<td>13.3%</td>
<td>6.0%</td>
<td>CMS NHE, Altarum Institute, 2014</td>
</tr>
<tr>
<td>4</td>
<td>Contribution of drug spending to total health care spending ($) (Row 2 x Row 3)</td>
<td>$410 billion</td>
<td>$185 billion</td>
<td>Calculation</td>
</tr>
<tr>
<td>5</td>
<td>Annual threshold for net health care cost growth for ALL new drugs (Row 1 x Row 4)</td>
<td>$15.4 billion</td>
<td>$6.9 billion</td>
<td>Calculation</td>
</tr>
<tr>
<td>6</td>
<td>Average annual number of new molecular entity or device approvals, 2013-2014</td>
<td>34</td>
<td>23</td>
<td>FDA, 2014</td>
</tr>
<tr>
<td>7</td>
<td>Annual threshold for average cost growth per individual new molecular entity (Row 5 ÷ Row 6)</td>
<td>$452 million</td>
<td>$301 million</td>
<td>Calculation</td>
</tr>
<tr>
<td>8</td>
<td>Annual threshold for estimated potential budget impact for each individual new molecular entity (doubling of Row 7)</td>
<td>$904 million</td>
<td>$603 million</td>
<td>Calculation</td>
</tr>
</tbody>
</table>
ICER Value-Based Price Benchmark

• Two components:
  – Step 1: Long-term cost-effectiveness
    • Price at which the cost per quality-adjusted life year gained = $100,000-$150,000
    • Range leaves room for the role of other factors
  – Step 2: Potential short-term budget impact
    • $904 million NET per year per new drug = affordability “alarm bell”
## ICER Reports and Value-Based Pricing

<table>
<thead>
<tr>
<th>Drug</th>
<th>Long-term Value-based Price</th>
<th>Affordability Alarm Bell?</th>
</tr>
</thead>
<tbody>
<tr>
<td>Harvoni</td>
<td>(150%-200%)</td>
<td>Yes (60%)</td>
</tr>
<tr>
<td>PCSK9 inhibitors</td>
<td>(46%-62%)</td>
<td>Yes (85%)</td>
</tr>
<tr>
<td>Entresto</td>
<td>(200%-300%)</td>
<td>Yes (9%)</td>
</tr>
<tr>
<td>Kyprios</td>
<td>(32%-64%)</td>
<td>No</td>
</tr>
<tr>
<td>Empliciti</td>
<td>(75%-89%)</td>
<td>No</td>
</tr>
<tr>
<td>Ninlaro</td>
<td>(80%-94%)</td>
<td>No</td>
</tr>
<tr>
<td>Mepolizumab</td>
<td>(63%-76%)</td>
<td>No</td>
</tr>
<tr>
<td>Tresiba</td>
<td>(8%-10%)</td>
<td>No</td>
</tr>
<tr>
<td>Obeticholic acid for PBC</td>
<td>(64%-73%)</td>
<td>No</td>
</tr>
<tr>
<td>TKIs for lung cancer</td>
<td>(100%-125%)</td>
<td>No</td>
</tr>
<tr>
<td>PD-1s for lung cancer</td>
<td>(25%-50%)</td>
<td>No</td>
</tr>
</tbody>
</table>

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Conceptual Approaches to “Fair” Pricing

• “Free market”/supply and demand

• Costs of development and production plus “reasonable” profit

• Added “value” to patients and health systems
  – Needs to be sensitive to the potential tension between long-term value for money and problems with short-term affordability
Trends in Specialty Drug Pricing

Peter B. Bach, MD, MAPP
Memorial Sloan Kettering Cancer Center
The Evidence Driven Drug Pricing Project (www.drugabacus.org)
bachp@mskcc.org
@peterbachmd
Evidence Driven Drug Pricing Project

The Evidence Driven Drug Pricing Project was established in an effort to highlight the on-going problem of rising drug prices.
Major work streams

- Research on pricing trends and policy solutions that tie drug prices to their drug attributes
- Coordination and evaluation of pilot programs
- Dissemination of findings in academic and lay press
Recent Projects

• Indication Specific Pricing
• Medicare Part B Payment Pilot
• Excess cost from drug waste
• Tracking of Recent Pricing Trends
The Problem
Cost for an additional year of life goes up each year in cancer
The graph I show the most often
The Next Big Debate in Health Care

Patient Spending on Deductibles Outpacing Wages

At least we have access?
The problem is the FDA?
Research

Trends in utilization of FDA expedited drug development and approval programs, 1987-2014: cohort study

Aaron S Kesselheim, associate professor of medicine, Bo Wang, medical student, Jessica M Franklin, assistant professor of medicine, Jonathan J Darrow, research fellow
The FDA Is Basically Approving Everything. Here's The Data To Prove It
Competition will solve this?
Exhibit 3
Change in costs for a thirty-day supply of oral anticancer drugs recently approved by the FDA, 2007–13

Effect of “competition”??

<table>
<thead>
<tr>
<th>Change after:</th>
<th>Change in costs</th>
<th>95% CI</th>
<th>P-value</th>
</tr>
</thead>
<tbody>
<tr>
<td>Additional supplemental indication</td>
<td>+9.2%</td>
<td>4.3, 14.3</td>
<td>0.001</td>
</tr>
<tr>
<td>Additional compendium-recommended off-label indication</td>
<td>+4.3%</td>
<td>0.0, 8.9</td>
<td>0.061</td>
</tr>
<tr>
<td>FDA approval of a competitor drug</td>
<td>−1.8%</td>
<td>−3.1, −0.4</td>
<td>0.003</td>
</tr>
<tr>
<td>One year</td>
<td>+4.4%</td>
<td>3.1, 6.0</td>
<td>&lt;0.001</td>
</tr>
</tbody>
</table>
Generics will solve everything?
It is important to know that no two drugs in one class are exactly the same. In fact, not all medications work the same for all patients, including the generic equivalents. When your doctor prescribes a medication, it is because he/she has decided it is the right one for you.

**Ask your doctor about continuing on brand-name CRESTOR.**

Brand-name CRESTOR—manufactured by AstraZeneca—will continue to be readily available. If you prefer to keep taking the brand, talk with your doctor. He or she can request brand-name CRESTOR on your prescription.¹

Also, remember that the $5 savings card can only be used with brand-name CRESTOR. Pay as low as $3 every time you fill, for as long as you're prescribed CRESTOR.

**Step 3**

If it's not CRESTOR, let the pharmacist know. At the pharmacy, your brand-name CRESTOR prescription may be switched automatically to a generic. That's why you need to remember to specifically ask your pharmacist for CRESTOR, as your doctor prescribed.

Only CRESTOR is CRESTOR.
Value-based pricing

• Core notion is to calculate prices for drugs that are “fair” or “appropriate”

• This looks like two things it isn’t
  – Price Fixing
  – European

• The difference is it is optional
Finding value-based prices:
The DrugAbacus project
Amgen’s analyses of Blincyto put back into the DrugAbacus
Note at these settings spending on the 54 drugs is about the same as current spending in US.
Under these settings spending on the 54 drugs is about six times current spending in US
Under these settings spending on the 54 drugs is about twenty times current spending in US.
Under these settings, the price of Gleevec increases from $10,262 to $211,994
If we had value based prices, then what?

Table. Potential Policy Options for Incorporating Value-Based Pricing for Drugs Into Regulation and Payment

<table>
<thead>
<tr>
<th>Actor</th>
<th>For Drugs With Value-Driven Prices</th>
<th>For Drugs Priced Above Value-Driven Prices</th>
</tr>
</thead>
<tbody>
<tr>
<td>Private payers</td>
<td>Guarantee formulary inclusion in first tier</td>
<td>Allow exclusion, placement in higher tier, or &quot;step&quot; therapy</td>
</tr>
<tr>
<td></td>
<td>Attach zero or nominal copayment</td>
<td>Attach coinsurance or apply reference pricing</td>
</tr>
<tr>
<td>Medicare formulary</td>
<td>Require inclusion in all Part D formularies in first tier</td>
<td>Allow exclusion or high-tier placement in Part D formularies</td>
</tr>
<tr>
<td></td>
<td>Set Part B coinsurance at low level, such as 5% or flat fee</td>
<td>Keep Part B coinsurance at 20% or reference pricing above value-based price</td>
</tr>
<tr>
<td>Medicare add-ons</td>
<td>Include entire price in new technology and pass-through payments</td>
<td>Include only portion of cost of new drugs up to value-based price in new technology and pass-through payments</td>
</tr>
<tr>
<td></td>
<td>Include entire price in projection of costs for bundle and gain share calculations</td>
<td>Include only portion of cost up to value-based price in calculating the cost of bundles and other performance payments</td>
</tr>
<tr>
<td>HRSA</td>
<td>Exclude drug from 340B drug discount program</td>
<td>Include in 340B program mandatory discounts</td>
</tr>
<tr>
<td>FDA</td>
<td>Increase exclusivity period</td>
<td>Decrease exclusivity period. Grant priority review to speed competitors to market</td>
</tr>
</tbody>
</table>

Abbreviations: FDA, Food and Drug Administration; HRSA, Health Resources and Services Administration.

Thank you
Specialty Drug Pricing

AEI
September 12, 2016

Tomas Philipson
The University of Chicago
Common Questions Surrounding Debate

• Rising prices is the cause of increased spending?

• Will value based pricing cut drug spending?

• Are there abnormal profits in pharma?

• Are US prices too high relative to other countries?
Falling or rising prices cause increased spending?

- Price of health vs price of health care
- Innovation cuts the price of additional health
- Brands is in price cutting business, enablers of generics
- Health care spending is rising because price of health falling!
- Brands raises affordability of health regardless of launch prices!

Steve Jobs  
HIV
What Determines Price of Health Care?

- Elasticity of Demand
  - Too much Me-Too Innovation and Too High Prices (Hep C & EpiPen)

- Fixed R&D Costs: Prices Determine R&D Spending, not Vice Versa
  - VC and PE funding rounds look forward, not backward
  - Industry R&D claim misguided

- High Prices: Why do buyers pay? You can't live without life-saving drugs

Price determined by Marginal Costs & Demand
Mispricing in US Reimbursement System

• Mispricing of Private Payers
  – 1/3 of health care wasted.. Really?
  – Why still a policy wonk if you can save 1 trillion?

• Medicare Part A, C, D: Downward Pricing Pressure from Demand Side

• Medicare Part B: Cost Plus vs Cost Times
  – High Prices because that’s what customers want
  – Many lower priced drugs due to elastic demand (e.g. MSK)
Common Questions Surrounding Debate

• Rising prices is the cause of increased spending?

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Value-Based Pricing: Whose Values?

Old and new payer valuation frameworks: low value

Patients willing to go bankrupt: high value
Innovation and Quality Adjusted Prices

• Hult, Jaffe, Philipson (2016), “Technological Change and Quality Adjusted Prices in Health Care”, Becker Friedman Institute, The University of Chicago.

• Value: Benefits vs Costs

• Cost-Effectiveness=Quality adjusted prices in health care

• CEAR Data of Tufts: 7000 comparisons of innovators & standard of care

• Tufts: Lower established prices vs higher launch prices (Hep C)
Health Care Innovation and Quality Adjusted Prices
Hult, Jaffe, Philipson (2016)

Percent Change in Quality and Price

Percent Change in Price

Percent Change in Quality

45 degree

11% 11%

57%

19%
QAP Innovator / QAP Standard of Care
Hult, Jaffe, Philipson (2016)
Value Based Pricing

- Specialty drugs 3-4% of total health care spending
- Will this share rise or fall if ALL care value priced?
  - Evidence on Drugs vs Procedures: FDA and IP
- Value based reimbursement does not select on value in practice:
  - NICE and threshold pricing (Housing analog)
Budget Impact and Credit Markets

Forbes

Contributor

Tomas Philipson

University of Chicago economist with op-eds on the health care economy

Medical Breakthroughs And Credit Markets

By Tomas J. Philipson and Andrew C. von Eschenbach

Common Questions Surrounding Debate

- Rising prices is the cause of increased spending?
- Will value based pricing cut drug spending?
- Are there abnormal profits in pharma?
- Are US prices too high relative to other countries?
Abnormal Profits in Biopharma?
Product Pricing vs Portfolio Profits

• Would you pay $100 for a 10% chance to win $100?

  Peter Bach & Co: Yes  
  Tomas Philipson: No

• Wild guess: You would terminate your pension making negative returns

• Guess what? Your pensions funds Rx Industry

• Therefore: Asking industry to invest in ways you would not let them

• Portfolio Profits: Marketed drug must earn at least $1 billion if R&D has 10% success rate and $100m cost per program
Abnormal Profits in Biopharma?
Risk and Return

- Why not only hold pharma in pension?
- “Medical Innovation Premium” the last 4 decades
  - About 3-6% per year
- For what risk does this excess return compensate?
- Recent evidence suggests exogenous government risks
  - Innovative returns greatly exposed to government risks
    - Approval risks: scientific/bureaucratic risk *exogenous to firm*
    - Reimbursement risks: political risk surrounding fiscal imbalances
Common Questions Surrounding Debate

• Rising prices is the cause of increased spending?

• Will value based pricing cut drug spending?

• Are there abnormal profits in pharma?

• Are US prices too high relative to other countries?
International Pricing: Are US prices too high?

- Innovation driven by world returns
- Example: Swedish firms don’t innovate for 9 million Swedes

**Key Implication**

What’s good for the UK is not good for the US
World returns to innovation as public good

- All countries benefit from profits provided by one country
- Inefficient under-provision of global profits and innovation
- EU prices too low as opposed to US prices too high

<table>
<thead>
<tr>
<th>Smaller Country</th>
<th>Larger Country</th>
</tr>
</thead>
<tbody>
<tr>
<td>- No access innovation tradeoff</td>
<td></td>
</tr>
<tr>
<td>- Will/Should provide small markups (Ex: CEA, Reference Pricing)</td>
<td></td>
</tr>
<tr>
<td>- Innovation access tradeoff</td>
<td></td>
</tr>
<tr>
<td>- US largest buyer with highest prices!</td>
<td></td>
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</table>

- The Horse Race of Future Returns to Innovation:
  - Emerging markets vs falling markups: Will US and China free-ride?
Real World Effectiveness of Interferon-based HCV Therapy

Represents a review of 25 studies in the real-world clinical care setting of 13,583 individuals diagnosed with HCV

Most common barriers:

- Medical ineligibilities
  - (e.g., substance use or psychiatric disorders, advanced liver disease and other co-morbidities)

- Patient barriers
  - (e.g., impact of side effects, lack of adherence, response to treatment)
Treatment Costs, Cost per SVR for HCV GT 1 Have Decreased Over Time

Cost and Cost Per SVR of Different Antiviral Regimens to Treat Patients with Hepatitis C Virus Genotype 1

Current Gilead discounts to Medicaid and the VA are greater than 50%

1 Chhatwal, J. et al. Why We Should Be Willing to Pay for Hepatitis C Treatment. Clinical Gastroenterology and Hepatology. Volume 13, Issue 10, October 2015, Pages 1711–1713. This price comparison does not imply that the treatments are clinically equivalent or that head-to-head clinical studies have been conducted.
Even Before Discounts, Harvoni Treatment Costs Lower Than Newer HCV Treatment Alternatives

Short-term 1-year total costs per sustained virologic response for GT 1 treatment-naive patients without cirrhosis

Source: Z.M. Younossi “Cost-effectiveness of all-oral ledipasvir/sofosbuvir regimens in patients with chronic hepatitis C virus genotype 1 infection”
Z. M. Younossi*, H. Park†, S. Saab‡, A. Ahmed §, D. Dieterich¶ & S. C. Dec 2014
Why Cures Disrupt the System

Chronic treatment for disease (e.g., HIV)
- Disease
- Chronic treatment of disease
- Payment and benefit aligned

Acute treatment for cure (e.g., HCV)
- Disease
- Cure
- Payment and benefit misaligned
Is the cure worth the cost?
The debate over the price of specialty drugs

JOSH OFMAN, M.D., M.S.H.S.
SVP, GLOBAL VALUE, ACCESS & POLICY
What is the real cost debate?

Is the cure worth the cost?

But...

What is the cost of not improving treatments?
We Need to Focus on the Rising Cost of the Most Common Diseases

- **Cardiovascular Disease**: #1 KILLER in the U.S. with 1 DEATH EVERY 40 SECONDS
  > $800B by 2030

- **Cancer**: 2nd LEADING CAUSE OF DEATH in the U.S.
  1% reduction in cancer-related death = $500B for society

- **Alzheimer’s Disease**: 5.3 MILLION AMERICANS IMPACTED
  > $1.1T by 2050
The challenge is clear

BIOTECHNOLOGY REVOLUTION

FOCUS ON SHORT-TERM SPENDING
Is the us spending too MUCH OR TOO LITTLE ON DRUGS?

Some of the most valuable interventions in healthcare address greatest unmet needs. Moderate drug spend projected at 6% growth. Cost offsets are clean. $1 = $3-10 saved. $750 billion annual healthcare system waste.
Patients deserve more appropriate methods for value assessment – not one size fits all

- No single answer
- Budget impact ≠ value
- Static vs Dynamic
- One approach to inform
- Cost-effectiveness thresholds = price control in disguise
There is no single answer to the question of value
Where we must focus – Patient Health

- Align around advanced methods and approaches to value - Balanced conversation
- Enable value-based partnerships through policy reform
- Ensure pharmaceutical benefit designs are non-discriminatory and value-based
- Ensure a robust, competitive generic marketplace to support the innovation ecosystem
- Align incentives