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The Policy Pincer: Rising Drug Costs and Declining Access
Innovation incentives and the role of ‘value’

• Drug development is risky and expensive
  • Long time horizon
  • Low success rate
  • Large capital outlays
  • Only makes sense if return for successes is large
• For drug development we have created a structure that provides this
  • Temporary monopoly rights to drug innovators when successful
The Reward Box
Revenue associated with pharmaceutical innovation
Reward Box is routinely changed by Policy

**Policy: Orphan Drug Act**

- **Lengthens exclusivity period**
- **Reduces FDA approval requirement**
- **Provides research tax credit**

**Policy: 21st Century Cures Act**

- **Enables off-label promotion**
- **Reduces FDA approval requirement**
But while monopoly duration addressed in policy, prices during monopoly are not
Prices rising more than ‘value’
How does ‘value’ connect with drug prices?

• Basic principles, and a tough needle to thread
  • Drug innovation matters
    • So incentives for it are desirable
  • Access to innovative drugs matters
    • So a system in which restricting access or giving ‘skin in the game’ is the go-to check on prices undermines its own purpose
  • Money is not infinite – there are always productive alternative uses (like letting people keep it)

• Determining fair prices, or ‘value based prices’, is a means to balance these objectives
This thing about other uses of money

- Remember, we are trying to thread a tough needle
- Allocate enough to encourage innovation
- But not so much we cannot afford other priorities

$475,000

OR?

- One year of commercial insurance for 17 families of four
- One year of salary for 6 US nurses
- A dozen full treatment course for Hep C
- > 500,000 doses of pentavalent vaccine (DPT, HAV, Haemophilus)
How may ‘value’ affect drug prices?

• Tech assessment produces a threshold price –
  • The treatment at that price might deliver one quality adjusted life year per $100,000 (for instance)
  • Using $100,000 per QALY means a decision that health is worth that much for society

• These prices are not what is needed to incentivize innovation
  • That number is not known, but it is almost certainly higher for rare diseases than common ones

• When people say ‘fair’ prices, they mean (or should mean) fair to society, not to drug companies
The approach is called “Value-based pricing”

• Two prongs:
  1) Determines price based on treatment’s benefit (i.e. manage the height of the box along with standards for its commencement, termination and downward slope)
  2) With price managed, require payers (including Medicare and Medicaid) to cover with reduced copayments
     1) Those are there to manage price (which they do poorly)

• Value based pricing is not:
  • Perfect, but it is meaningfully better than current
  • Recognizes that ensuring access to new innovation is not something markets do well
  • Outcomes based contracting
Value-based pricing concepts and approaches
These analyses reveal how misaligned prices can be

Table ES5. Incremental Cost-Effectiveness Ratios Compared to Best Supportive Care (BSC) for the Base Case

<table>
<thead>
<tr>
<th>Treatment vs. BSC</th>
<th>Cost Per LY Gained</th>
<th>Cost Per QALY Gained</th>
<th>Cost Per PEx Averted</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>CF Individuals with a Gating Mutation</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Kalydeco Plus BSC</td>
<td>$1,476,543</td>
<td>$956,762</td>
<td>$463,571</td>
</tr>
<tr>
<td></td>
<td>CF Individuals Homozygous for F508del Mutation</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Orkambi Plus BSC</td>
<td>$1,280,892</td>
<td>$890,739</td>
<td>$334,495</td>
</tr>
<tr>
<td>Symdeko Plus BSC</td>
<td>$1,367,400</td>
<td>$974,348</td>
<td>$424,212</td>
</tr>
<tr>
<td></td>
<td>CF Individuals Heterozygous for F508del Mutation and Residual Function Mutation</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Kalydeco Plus BSC</td>
<td>$1,340,171</td>
<td>$941,110</td>
<td>$373,541</td>
</tr>
<tr>
<td>Symdeko Plus BSC</td>
<td>$1,174,508</td>
<td>$840,568</td>
<td>$390,600</td>
</tr>
</tbody>
</table>

BSC: best supportive care; LY: life year; QALY: quality adjusted life years; PEx: pulmonary exacerbation
And sometimes show that prices are probably not far off

<table>
<thead>
<tr>
<th>Trial</th>
<th>Therapy</th>
<th>Event-free Survival at 6 Months*</th>
<th>Overall Survival at 12 Months</th>
</tr>
</thead>
<tbody>
<tr>
<td>B210114</td>
<td>Tisagenlecleucel</td>
<td>58%</td>
<td>81%</td>
</tr>
<tr>
<td>B220514</td>
<td>Tisagenlecleucel</td>
<td>46%</td>
<td>62%</td>
</tr>
<tr>
<td>B2202 / ELIANA19</td>
<td>Tisagenlecleucel</td>
<td>60%</td>
<td>62%</td>
</tr>
<tr>
<td>Jeha 200617</td>
<td>Clofarabine</td>
<td>11%</td>
<td>20%</td>
</tr>
<tr>
<td>Hijiya 201117</td>
<td>Clofarabine/etoposide / cyclophosphamide</td>
<td>35%</td>
<td>35%</td>
</tr>
<tr>
<td>Von Stackelberg 201617</td>
<td>Blinatumomab</td>
<td>16%</td>
<td>38%</td>
</tr>
<tr>
<td>Locatelli 201719</td>
<td>Blinatumomab</td>
<td>NR</td>
<td>NR</td>
</tr>
</tbody>
</table>

*Based on the number enrolled, not the number receiving the infusion with CAR-T cells or the number responding to treatment

### Table ES8. Objective Response Rates Reported for Tisagenlecleucel for Relapsed or Refractory Adult B-Cell Lymphoma Compared with SCHOLAR-1

<table>
<thead>
<tr>
<th>Trial</th>
<th>Therapy</th>
<th>ORR</th>
<th>CR</th>
</tr>
</thead>
<tbody>
<tr>
<td>JULIET16</td>
<td>Tisagenlecleucel</td>
<td>53%</td>
<td>40%</td>
</tr>
<tr>
<td>NCT092432617</td>
<td>Tisagenlecleucel</td>
<td>64%</td>
<td>57%</td>
</tr>
<tr>
<td>SCHOLAR-117</td>
<td>Mix of salvage therapies</td>
<td>26%</td>
<td>7%</td>
</tr>
</tbody>
</table>

CR: complete remission, ORR: objective response rate

### Table ES19. Value-Based Price Benchmarks for Tisagenlecleucel and Axicabtagene Ciloleucel

<table>
<thead>
<tr>
<th>Therapy</th>
<th>WAC</th>
<th>Net Price (with Mark-Up)</th>
<th>Price* to Achieve $100,000 per QALY</th>
<th>Price* to Achieve $150,000 per QALY</th>
<th>Discount from WAC with Mark-Up to Reach Threshold Prices</th>
</tr>
</thead>
<tbody>
<tr>
<td>Tisagenlecleucel (B-ALL)</td>
<td>$475,000</td>
<td>$575,000</td>
<td>$1,162,563</td>
<td>$1,688,232</td>
<td>+102% to +194%</td>
</tr>
<tr>
<td>Axicabtagene Ciloleucel (B-cell Lymphoma)</td>
<td>$373,000</td>
<td>$473,000</td>
<td>$340,797</td>
<td>$524,015</td>
<td>28% to +11%</td>
</tr>
</tbody>
</table>

Payment assumed for tisagenlecleucel was payment for responders at one month. Payment assumed for axicabtagene ciloleucel was payment at infusion.

*Price needed to achieve the thresholds includes both the acquisition cost and associated mark-up.

B-ALL: B-cell acute lymphoblastic leukemia, QALY: quality-adjusted life year

+Indicates premium

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INSTITUTE FOR HEALTH POLICY FORUM  #DRUGPRICES  #KPIHP  KAISER PERMANENTE.
That word ‘value’, it’s getting around
That word ‘value’, it’s getting around

• Once analysts started talking about value based pricing, everyone started calling every pricing agreement value based

  • Mortgages, outcomes arrangements, the Netflix model, out of pocket caps
  • Nope – value pricing is when the benefits of a treatment are mathematically aligned with its price
Pricing is not the only needed fix – prescriber incentives

<table>
<thead>
<tr>
<th>Article (Year)</th>
<th>Population studied</th>
<th>Comparison</th>
<th>Findings</th>
</tr>
</thead>
<tbody>
<tr>
<td>Elliott et al. (2009)²</td>
<td>Medicare beneficiaries with low risk and metastatic prostate cancer</td>
<td>Use of ‘androgen suppression therapy’ before and after a reimbursement change due to a law change that decreased the margin, compared between low risk and metastatic patients</td>
<td>Reduction in reimbursement of 64% associated with an OR of 0.61-0.70 reduction of use in low risk with no change in metastatic patients</td>
</tr>
<tr>
<td>Jacobson et al. (2010)³</td>
<td>Medicare beneficiaries with lung cancer</td>
<td>Use of five different drugs for lung cancer that all experienced shifts in margin due to a law change in 2005</td>
<td>Use of drugs with the largest decline in margin fell the most after the rule change. Use of drugs with unchanged margins increased.</td>
</tr>
<tr>
<td>Colla et al. (2012)⁴</td>
<td>Medicare decedents who had any cancer, treated in physician offices or hospital outpatient department</td>
<td>Utilization of chemotherapy in the months preceding death before and after a law change that decreased margins and comparing impact on two settings, where physician offices presumed to be more affected by incentives</td>
<td>Use of chemotherapy prior to death declined in physician offices following a reduction in margins, but did not decline in the hospital outpatient departments.</td>
</tr>
<tr>
<td>Epstein et al. (2013)⁵</td>
<td>Medicare beneficiaries with breast cancer (1992-2002)</td>
<td>Within treated population evaluation of prescribing frequency in relation to ‘margin’ (reimbursement – acquisition cost)</td>
<td>Increase margin of +10% led to an increase in prescribing likelihood of +10% - +177%.</td>
</tr>
<tr>
<td>Conti et al. (2012)⁶</td>
<td>Medicare beneficiaries with metastatic colorectal cancer</td>
<td>Use of two alternative drugs for colorectal cancer, one which went generic and as a result had a decline in margin compared to the other that did not</td>
<td>Use of the drug that went generic declined once the margin on the drug was reduced. Use of the alternative drug was maintained.</td>
</tr>
</tbody>
</table>

³ Colla et al. “Impact of Payment Reform on Chemotherapy at the End of Life”, Journal of Oncology Practice, 2012; e16-e13s
⁵ Conti et al. Journal of Oncology Practice. 2012; 8:35, e188-e23s
Pricing is not the only needed fix – supply chain mark-ups
Pricing is not the only needed fix – monopoly period has to end and doesn’t for biologics

Biologics Are Natural Monopolies (Part 1): Why Biosimilars Do Not Create Effective Competition
Preston Atteberry, Peter B. Bach, Jennifer A. Ohn, Mark Trusheim

Biologics Are Natural Monopolies (Part 2): A Proposal For Post- Exclusivity Price Regulation Of Biologics
Mark Trusheim, Preston Atteberry, Jennifer A. Ohn, Peter B. Bach

Time to Throw In the Towel on Biosimilars
Biologic drugs don’t face strong competition, and Washington’s preferred solution slows innovation.

By Peter B. Bach and Mark Trusheim
Aug 21, 2019 5:56 pm ET
Significant savings through regulated pricing

Proposal for post-exclusivity price regulations estimated to generate savings exceeding $200 billion.

EXHIBIT 1: Five-Year Savings (2018-22) Based On Various Estimates Of Post-Exclusivity Prices, Calculated From Projected US Revenue

<table>
<thead>
<tr>
<th>Percent of current price</th>
<th>Total savings</th>
<th>Savings to Medicare</th>
<th>Savings to Medicaid</th>
<th>Total savings</th>
<th>Savings to Medicare</th>
<th>Savings to Medicaid</th>
</tr>
</thead>
<tbody>
<tr>
<td>30%</td>
<td>258.1</td>
<td>63.7</td>
<td>23.7</td>
<td>213.5</td>
<td>52.7</td>
<td>19.6</td>
</tr>
<tr>
<td>20%</td>
<td>295.0</td>
<td>72.9</td>
<td>27.1</td>
<td>244.0</td>
<td>60.3</td>
<td>22.4</td>
</tr>
<tr>
<td>10%</td>
<td>331.8</td>
<td>82.0</td>
<td>30.5</td>
<td>274.5</td>
<td>67.8</td>
<td>25.3</td>
</tr>
</tbody>
</table>

Source: Authors’ analysis.
@pfizer’s Ian Read: It’s not drug pricing, that’s the problem, it’s drug affordability - that’s the problem. #politicohealth