Do Specialty Drugs Offer Value for Money?

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Despite High Costs, Specialty Drugs May Offer Value For Money Comparable To That Of Traditional Drugs

ABSTRACT Specialty drugs are often many times more expensive than traditional drugs, which raises questions of affordability and value. We compared the value of specialty and traditional drugs approved by the Food and Drug Administration (FDA) in the period 1999–2011. To do this, we identified published estimates of additional health gains (measured in quality-adjusted life-years, or QALYs) and increased costs of drug and health care resource use that were associated with fifty-eight specialty drugs and forty-four traditional drugs, compared to preexisting care. We found that specialty drugs offered greater QALY gains (0.183 versus 0.002 QALYs) but were associated with greater additional costs ($12,238 versus $784), compared to traditional drugs. The two types of drugs had comparable cost-effectiveness. However, the distributions across the two
Specialty drugs

“Large molecule”
Produced using advanced biotechnology
Physician-administered

“Small-molecule”
Manufactured using simpler processes
Self-administered
Are specialty drugs good value for money?
What did we do?

- FDA drug approvals (1999–2011)
- Examined if costs of specialty drugs are worth the clinical benefits
- Compared with traditional drugs
How we did it

FDA drug approvals
(1999–2011)
How we did it

Literature search to identify $\Delta$ QALYs and $\Delta$ costs
Cost-effectiveness

Cost-effectiveness expressed using an incremental cost-effectiveness ratio (ICER)

An ICER is the ratio of the difference in costs to the difference in effects between competing interventions

\[
ICER = \frac{Cost_B - Cost_A}{Effect_B - Effect_A}
\]
Quality-Adjusted Life Years

Quality-adjusted life years gained

PD diagnosis

QALY with treatment

QALY without treatment

Quality of life

Lifeyears

PD = Parkinson's Disease
How we did it

Literature search to identify $\Delta$ QALYs and $\Delta$ costs

Specialty drugs
$\Delta$ QALYs
$\Delta$ Costs

VS.

Traditional drugs
$\Delta$ QALYs
$\Delta$ Costs
Specialty drugs
$12,238

Traditional drugs
$784

p < 0.01
## Largest added costs

<table>
<thead>
<tr>
<th>Drug name</th>
<th>Indication</th>
<th>Specialty drug?</th>
<th>Added cost ($)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Agalsidase beta</td>
<td>Fabry disease</td>
<td>✓</td>
<td>3,663,000</td>
</tr>
<tr>
<td>Deferasirox</td>
<td>Hemosiderosis</td>
<td>✓</td>
<td>168,000</td>
</tr>
<tr>
<td>Imatinib mesylate</td>
<td>Chronic myeloid leukemia</td>
<td>✓</td>
<td>152,000</td>
</tr>
<tr>
<td>Omalizumab</td>
<td>Moderate to severe asthma</td>
<td>✓</td>
<td>101,000</td>
</tr>
<tr>
<td>Dasatinib</td>
<td>Chronic myeloid leukemia</td>
<td>✓</td>
<td>98,000</td>
</tr>
<tr>
<td>Crizotinib</td>
<td>Metastatic NSCLC</td>
<td>✓</td>
<td>97,000</td>
</tr>
<tr>
<td>Cabazitaxel</td>
<td>Metastatic prostate cancer</td>
<td>✓</td>
<td>78,000</td>
</tr>
<tr>
<td>Enfuvirtide</td>
<td>HIV-1 infection</td>
<td>✓</td>
<td>72,000</td>
</tr>
<tr>
<td>Ranibizumab</td>
<td>Macular degeneration</td>
<td>✓</td>
<td>70,000</td>
</tr>
<tr>
<td>Fingolimod</td>
<td>Multiple sclerosis</td>
<td>✓</td>
<td>63,000</td>
</tr>
</tbody>
</table>
Added health benefit

**Specialty drugs**
0.183

**Traditional drugs**
0.002

p < 0.01

Chambers et al.  
*Health Affairs* 2014 Oct;33(10):1751-60
## Largest QALY gains

<table>
<thead>
<tr>
<th>Drug name</th>
<th>Indication</th>
<th>Specialty drug?</th>
<th>QALY gain</th>
</tr>
</thead>
<tbody>
<tr>
<td>Deferasirox</td>
<td>Hemosiderosis</td>
<td>✔</td>
<td>4.2</td>
</tr>
<tr>
<td>Imatinib</td>
<td>Chronic myeloid leukemia</td>
<td>✔</td>
<td>4.1</td>
</tr>
<tr>
<td>Linezolid</td>
<td>Bacterial infections</td>
<td>✗</td>
<td>1.1</td>
</tr>
<tr>
<td>Ranibizumab</td>
<td>Macular degeneration</td>
<td>✔</td>
<td>1.1</td>
</tr>
<tr>
<td>Bosentan</td>
<td>Pulmonary arterial hypertension</td>
<td>✔</td>
<td>1.0</td>
</tr>
<tr>
<td>Oxaliplatin</td>
<td>Metastatic colon cancer</td>
<td>✔</td>
<td>0.9</td>
</tr>
<tr>
<td>Dasatinib</td>
<td>Chronic myeloid leukemia</td>
<td>✔</td>
<td>0.8</td>
</tr>
<tr>
<td>Enfuvirtide</td>
<td>HIV-1 infection</td>
<td>✔</td>
<td>0.8</td>
</tr>
<tr>
<td>Entecavir</td>
<td>Chronic hepatitis B</td>
<td>✔</td>
<td>0.8</td>
</tr>
<tr>
<td>Agalsidase beta</td>
<td>Fabry disease</td>
<td>✔</td>
<td>0.7</td>
</tr>
</tbody>
</table>
Cost-effectiveness

Similar cost-effectiveness

$p = 0.35$
What does this mean?

- Specialty drugs more expensive, but offer larger benefits
- In many cases offer reasonable value for money
- Study does not address affordability
Limitations

- Study relied on published literature
- Caution with incremental cost data
- Choice of comparator
- Does not address affordability
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Thank you

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