GLOBAL FORUM
DRIVING IDEAS TO ACTION

INNOVATION

WHAT IS HOT IN HAMBURG?
Thoughts on issues that will – and will not – be discussed at the 28th Annual EuroMeeting by John Lisman

Kemal Malik
Co-chair

Karl Broich
Co-Chair

28TH ANNUAL EUROMEETING:
INnovation: Do You Win by Being IN?
FDA’s Accelerated Pathways Are The New Normal (And They Work if You Work Them)

Barry Farrimond
Jonathan Fleming
Mark Mathieu

The FDA’s accelerated pathways (APs) – including Accelerated Approval (AA), Breakthrough Therapy Designation (BTD), Fast Track (FT), Orphan Drug (OD) status, and the Qualified Infectious Disease Product (QIDP) program – were designed to facilitate faster development and approval of NDAs addressing unmet needs. According to the FDA’s annual report, two-thirds of novel drugs approved in 2015 used at least one AP, while a third used two or more.

In order to better understand how major US payers view AP products, PAREXEL and the nonprofit, non-partisan Network for Excellence in Healthcare Innovation (NEHI), conducted a survey of 20 national, regional, public, and private payers whose coverage decisions impact a total of 228 million patients.

BENEFITS, RISKS

Products that have qualified for one or more APs have achieved shorter clinical development and regulatory review times, obtained widespread coverage by payers, commanded premium prices, and achieved rapid uptake, especially in cases where significant patient benefits are supported by strong evidence (e.g., hepatitis C and immunoncology). That’s the good news.

However, APs also come with risks for payers and developers that must be managed. For payers, high initial prices and coverage decisions may be difficult to modify later, even if post-approval real world evidence (RWE) does not support the initial value proposition. For developers, the high prices and prevalence of AP drugs may encourage a trend toward formal value-based assessments that consider broader categories of cost and benefit.

EVERY AP BENEFIT COMES WITH AN AP CHALLENGE

Faster to Market, Less Data When You Get There

The level of evidence needed for FDA approval under APs does not always align with the evidence payers would like to see in order to demonstrate therapeutic value to patients and economic value to the health care system.

Sixty percent of payers surveyed say the “main challenge” with AP drugs is that high initial prices may be difficult to modify if post-approval real world evidence (RWE) is not as robust as the randomized clinical trial (RCT) data used for regulatory approval. Another 30% cite the fact that they may have to revisit initial coverage decisions after full risks and benefits are known (Figure 1). Less than half (47%) of payers think manufacturers should control post-launch data, while 41% say payers...
themselves should maintain the data (Figure 1).

**Rapid Uptake and Premium Pricing, Efforts to Control Utilization**

The FDA’s designation of AP drugs as, by definition, innovative and needed, has been accepted by payers. For example, because the new hepatitis C virus (HCV) treatments (all of which used APs) demonstrated unprecedented clinical benefits, they were reimbursed by payers, despite high prices, high-profile posturing from some stakeholders, and payers’ utilization management tools, such as Prior Authorization.

95% of payers expect AP drugs will be at least “moderately” or “significantly” higher priced than non-AP drugs (Figure 2). Though payers concede that some of these drugs represent “major improvements” over existing therapies, the cumulative cost to the healthcare system concerns them.

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**Figure 1. Payer Responses to Survey Questions About AP Products and Post-Launch Datasets**

<table>
<thead>
<tr>
<th>What is the &quot;Main Challenge&quot; with AP Products?</th>
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<tbody>
<tr>
<td>High Initial Prices Difficult to Modify</td>
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<tr>
<td>Initial Coverage Decisions May Need to be Revisited</td>
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<tr>
<td>Reduced Ability to Plan/Prepare</td>
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<tr>
<td>Product Evaluation</td>
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<tr>
<th>What is your Preferred Type of Dataset Post-Launch?</th>
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<tbody>
<tr>
<td>Open Label Extension of Pivotal Trial(s)</td>
</tr>
<tr>
<td>Claims Database</td>
</tr>
<tr>
<td>Retrospective Studies</td>
</tr>
<tr>
<td>Observational Studies</td>
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<table>
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<tr>
<th>Who Should Be in Charge of Post-Launch Datasets?</th>
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<tbody>
<tr>
<td>Manufacturers</td>
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<tr>
<td>Payers</td>
</tr>
<tr>
<td>Regulators (FDA)</td>
</tr>
<tr>
<td>Academics/Healthcare Providers</td>
</tr>
</tbody>
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*SOURCE: PAREXEL/NEHI 2015 U.S. Payer Survey¹*
According to payers, control measures they are most likely to use in the near-term will be: prior authorization; step edits – escalating from less to more expensive treatments in a step-wise fashion; and increased co-payments (Figure 2).

Valuable Benefits for Patients, Trend Toward Value-Based Assessments

Almost by definition, AP drugs can confer significant patient benefits while commanding high prices, and are thus consistent with the trend toward a more value-based U.S. healthcare system. 71% of payers surveyed expect economic assessments to play an ever-larger role in coverage determinations. That trend will likely impact both AP and non-AP products.

The shift in the U.S. toward a more value-based healthcare system is clear. A majority (52%) of U.S. payers said holistic budget impact modeling (looking at the effects of a drug, or class of drugs, on the overall healthcare budget) will be the most likely form of economic assessment going forward, while 29% said budget impact calculations would look solely at prescription drug budgets (Figure 2).

APS WORK IF YOU WORK THEM

APs confer substantial benefits to companies that can obtain them and that can adapt their commercial strategies to leverage designated assets. Those companies will increasingly need to demonstrate the economic value of AP drugs, in terms of their impact on service costs, costs to reduce adverse outcomes, and costs to achieve clinical success, as well as their overall budget impact.

References

Data and Methodology: The PAREXEL-NEHI Payer Survey
- Dates: Conducted between Sept. 7 and 18, 2015.
- Format: 20-minute multiple choice grouped responses (some free-text input possible).
- Makeup of Responding Payers: 40% are National Private, 15% are National Public/CMS, 29% are Regional Private and 16% are Regional Public/CMS.

About the Authors

Barry Farrimond is Vice President of Commercialization and Global Head of Pricing & Market Access at PAREXEL. His role is to provide strategic and technical oversight to the service line. Mr. Farrimond works closely with delivery teams to ensure that projects are completed to the highest standard, alongside developing PAREXEL’s core service areas. He brings 15 years of Commercialization experience. Mr. Farrimond previously served as a P&MA specialist in the Pricing & Market Access unit within IMS Health.

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Figure 2. Payer Responses to Survey Questions About Prices, Cost Containment and Economic Assessments of AP Drugs

How do you think the pricing of AP products will differ from non-AP products?

- Significantly Higher (+20% or more): 52%
- Moderately Higher (+10-20%): 42%
- Slightly Higher: 6%
- No Different: 0%

What cost containment measures will be most prevalent in the future?

- Prior Authorizations: 65%
- Step Edits (Step Therapy): 30%
- Increased Co-Payment: 6%
- Tier Management: 0%

What is the most likely form of economic assessment in the future?

- Budget Impact (Holistic): 53%
- Budget Impact (Siloed-Prescription Drugs): 29%
- Cost-Effectiveness: 18%

How likely is it that economic reviews of new products might be widened to consider healthcare system and societal burden costs?

- Not Likely: 29%
- Somewhat Likely: 53%
- Likely: 12%
- Very Likely: 6%

SOURCE: PAREXEL/NEHI 2015 U.S. Payer Survey