AMCP PARTNERSHIP FORUM
No. 3 — 2021

EXECUTIVE **SUMMARY**

Addressing Evidence Gaps in Accelerated Approval: Payer Perspectives

The U.S. Food and Drug Administration's (FDA) expedited pathway programs provide important mechanisms to make treatments for serious conditions with high unmet needs available to patients sooner. However, despite the rigorous steps in the drug development process, limitations exist due to the difference between FDA requirements for an expedited pathway and payer evidence needs for these often high-investment or high-risk safety profile medications. This is particularly true in the accelerated approval pathway, which allows the use of surrogate endpoints to predict clinical benefit, posing challenges to payers' need to pay for value.

> *"Forums like this one give us the opportunity to learn from people we might not have the opportunity to hear from and talk with in our day-to-day work."*
>
> **Kim McCleary**
> Founder and CEO, Kith Collective

To support payer needs and thus maintain patient access to medications for serious conditions, AMCP held a Partnership Forum in Alexandria, Va., Nov. 18–19, 2021. This group of 37 experts represented payers, pharmacy benefit managers, integrated delivery systems, patient advocates, academicians, and other key stakeholders in the managed care setting.

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WATCH FOR FOLLOW-UP

The Partnership Forum is a critical part of AMCP's efforts to address evidence gaps and reduce financial uncertainty for products approved through FDA expedited review or accelerated approval pathways. Our next steps will be to:

- Publish a proceedings document on findings and recommendations from the Partnership Forum in an upcoming issue of AMCP's *Journal of Managed Care + Specialty Pharmacy (JMCP)* and disseminate it widely to decision makers around the country.
- Host a webinar to report these findings and recommendations.
- Provide educational opportunities around expedited programs and payer evidence needs.
- Monitor and support policy and evidence ecosystems to improve processes and communication within the accelerated approval pathway.

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These participants were asked to: 1) identify gaps between FDA accelerated approval requirements and treatment outcomes valued by payers; 2) identify opportunities for an evidence ecosystem to address gaps between FDA accelerated approval requirements and payer coverage requirements; and 3) develop policy recommendations to facilitate communication of payer needs, reduce financial uncertainty, and potentially decrease the time between FDA approval/clearance and coverage decisions.

After deliberation, several action areas were identified by participants as significant:

- **Support innovation** in the development of drugs and other therapies, and consider meaningful outcomes during the approval process to aid timely coverage determinations.
- **Clarify terminology** for the definition that serves as the basis for accelerated approval. Refining, codifying, and sharing the definition of surrogate endpoints that are "reasonably likely to provide clinical benefit" will serve to increase stakeholder trust.

> *"Having different key stakeholders as part of this partnership has been very insightful for me as a payer."*
>
> **Lilian Ndehi-Rice**
> Associate VP,
> Pharmacy Clinical and Specialty Strategies,
> Humana Inc.

- **Correlate surrogate endpoints** with meaningful outcomes to increase confidence in longer-term treatment effects and provide greater certainty for payment and reimbursement.
- **Develop an evidence ecosystem** with both enhanced data and analytic capabilities to more readily deliver information on the long-term safety, effectiveness, and durability of treatment benefit projected at approval. This will also connect stakeholders to various sources of data such as registries, as these data sources evolve to assist in monitoring products post-approval.
- **Advance potential policy solutions** and practices to reduce the financial uncertainty associated with drugs approved via the accelerated approval pathway. This may include, for instance, a voluntary body to gather payer input on meaningful outcomes or mechanisms to incentivize timely completion of required confirmatory trials.

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