The utilization of pre-approval information and impact on formulary decision-making timelines

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Background and Objective

- Pre-approval information exchange (PIE) involves manufacturers supplying clinical and economic information to healthcare decision makers (HCDMs) before product approval by the Food and Drug Administration (FDA).[1]
- Previous research has highlighted an increase in the quality and quantity of PIE among manufacturers and HCDMs to support product review.
- However, there is limited evidence on how PIE influences the formulary decision-making process to facilitate the preparation of budget impact analyses and policy development.
- The objective of the research was to assess trends in utilization of PIE among HCDMs and identify the content that may support organizations to make formulary decisions closer to the product approval date.

Methods

- Double-blinded, web-based surveys were fielded to FormularyDecisions® HCDM users from May 16, 2022, to May 23, 2022.
  - FormularyDecisions is an online platform that houses credible and transparent information for those involved in the formulary decision-making process.
  - Participation in this survey was voluntary, and a modest honorarium was paid by Xcenda to participants who completed the survey.
- Respondents were HCDMs from health plans, integrated delivery networks (IDNs), and pharmacy benefit managers (PBMs) who reported having a role in the formulary decision-making process.
- The survey gathered insights on HCDMs' experience with PIE in the last year, content of interest, and value perceptions across pre-approval content.
  - Free-text responses were coded into themed categories.
  - Descriptive statistics were used to summarize results for multiple-choice and free-text answers.

Results

- A total of 17 respondents completed the survey. As shown in **Table 1**, health plans represented the largest percentage of respondents' organizations, followed by PBMs.

Table 1. Respondent demographics (N=17)

Organization type,[a] n (%)	Health plan	PBM	IDN
	7 (41)	4 (24)	2 (12)
Geographic representation, n (%)	**National**	**Regional**	
	8 (47)	9 (53)	
Primary role of advisors, n (%)	**Pharmacy director**	**Pharmacist[b]**	**Consultant**
	9 (53)	7 (41)	1 (6)

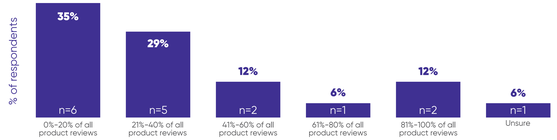
[a]Other respondent organization types included consulting group, 2 (12%); health system or hospital, 1 (6%); and academic institution, 1 (6%). The organizations represent lives across commercial, Medicare, and Medicaid lines of business.
[b]Clinical, drug information.
Key: IDN – integrated delivery network; PBM – pharmacy benefit manager.

- Of the 17 respondents, 94% indicated that their organizations were at least somewhat experienced with using pre-approval information. This is an 11% increase compared to a similar survey conducted from August 30 to September 31, 2021, which showed that 83% were experienced with using pre-approval information.[2]

Results (cont.)

- Varying levels of use of pre-approval information were reported in product reviews for formulary decision making, with 30% of respondents reporting using pre-approval information for more than 40% of their product reviews (**Figure 1**).
  - For those who reported using pre-approval information in less than 40% of reviews, limited availability from manufacturers and relevance to the process based on the product or timing of the review were noted as challenges with PIE (**Figure 1**).

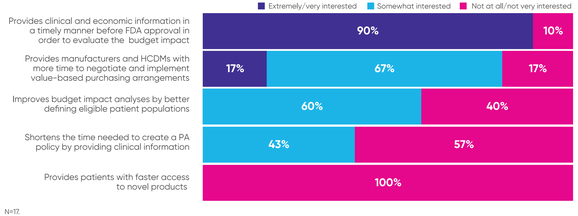
Figure 1. Frequency of pre-approval information utilization for formulary decision making



Free-text responses included: "Approximately 21%-40% of the new drugs to market consisted of novel mechanisms of action and/or first in class. All others were either me-too drugs or spin-offs of existing therapies." "Not every new product that comes out has pre-approval information available and is relevant to us. We dig into the drugs, as soon as possible, that are most relevant and review the rest after launch." "Only some manufacturers provide this information in advance of their FDA approval." "Pre-approval information may not have been available or the timing of the review of the drug/drug class may not have made pre-approval information necessary. There are also times when the information is not critical to an efficient or effective review."
N=17.
Q. For newly FDA-approved products in 2021 and 2022, for how many product reviews did your organization use pre-approval information in your formulary decision-making process?

- Almost all (90%) respondents ranked the availability of clinical and economic information in a timely manner to evaluate budget impact as the top benefit of PIE (**Figure 2**). Place in therapy and value-based contracting were also identified as unmet needs that PIE may help address.

Figure 2. Benefits associated with availability of pre-approval information

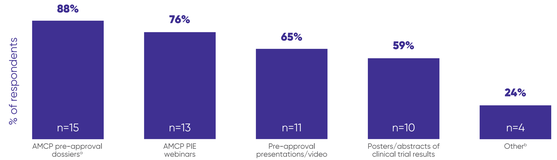


N=17.
Q. In order of importance, please rank the top 3 benefits associated with availability of pre-approval information for your organization, with #1 being the most important.
Key: HCDM – healthcare decision maker; PA – prior authorization.

- AMCP pre-approval dossiers (88%) and PIE webinars (76%) were the most commonly utilized resources in formulary decision making (**Figure 3**).
- Autoimmune diseases (82%), followed by oncology (59%) and rare diseases (47%), were ranked as top therapeutic areas of interest for obtaining pre-approval information (**Figure 4**).
- Based on respondent feedback, there are 53 products across 13 therapeutic areas that are of interest for PIE, with highest interest in gene therapy, biosimilars, and tirzepatide (**Figure 5**). This is consistent with the 2021 survey that showed high interest in gene therapy and biosimilars for pre-approval information.[2]

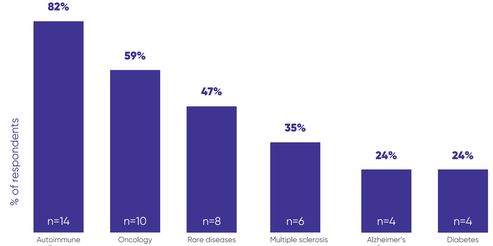
Results (cont.)

Figure 3. Pre-approval information resources utilized for formulary decision making



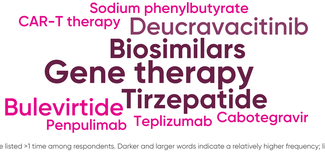
[a]AMCP pre-approval dossiers include unapproved product and unapproved use of approved product dossiers.
[b]Other included: "ICER," "pharma-led internal meetings," "legitimate medical publications," and "other information available from other PBMs' approvals and reviews."
N=17.
Q. Which of the following pre-approval information resources have you utilized in formulary decision making? Select all that apply.

Figure 4. Top 5 reported disease states of interest for PIE



N=17.
Q. In order of importance, please rank the top 5 therapeutic areas that would interest you the most for obtaining pre-approval information, with #1 being the most important.

Figure 5. Top 5 reported products and product categories of interest for PIE[a]



[a]The figure includes products that were listed >1 time among respondents. Darker and larger words indicate a relatively higher frequency; lighter and smaller words indicate a relatively lower frequency.
Q. In order of importance, please list the top 5 products in development that would interest you the most for obtaining pre-approval information, with #1 being the most important.
Key: CAR T – chimeric antigen receptor T-cell.

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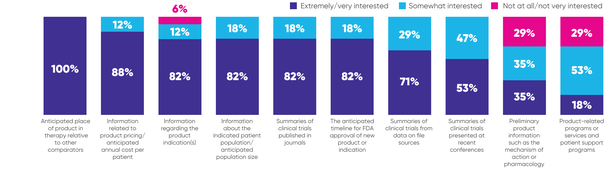
Results (cont.)

- Almost half of the respondents (47%) noted that the availability of pre-approval information has shortened their time to make a formulary decision.
- The following were rated as extremely/very impactful on shortening the formulary decision-making timeline: the anticipated place of product in therapy relative to other comparators (100%), information related to product pricing/budget impact (88%), and product indications (82%) (**Figure 6**).
  - Respondents indicated that they leveraged this information to compare products to standard of care, create prior authorization policy criteria, and finalize formulary decisions.

Figure 6. Type of pre-approval information impact on shortening the formulary decision-making timeline



"Other" for *extremely/very impactful* included "how supplied, product size," "contracting strategy," and "CEA analysis, ICER analysis, NNT, or QALYs; value-based contracts."
N=17.
Q. In order of importance, please rank the top 5 therapeutic areas that would interest you the most for obtaining pre-approval information, with #1 being the most important.

Limitations

- This research reflects the perspectives of HCDMs identified from users of FormularyDecisions; other user types (eg, healthcare providers, patients, manufacturers) were not represented in this survey.
- The respondent sample had greater representation from pharmacy directors from PBMs compared with health plans, IDNs, and other organization types, which could affect generalizability of the results across all types of HCDMs.
- Survey results were descriptive in nature and based on a small number of respondents and thus may not be generalizable to all HCDM organizations or payer types.
- Because all respondents voluntarily completed the survey, voluntary response bias may exist, and survey results may over-represent respondents with stronger interest in payer-manufacturer partnerships.

Conclusions

- The results suggest that pre-approval information is beneficial in various aspects of the formulary review process.
- HCDMs prioritize pre-approval information for high-impact products (eg, large population size, novel or high-cost therapies) and most often utilize AMCP pre-approval dossiers and PIE webinars to obtain this content.
- The availability of pre-approval information has an impact on shortening formulary decision-making timelines, particularly content regarding anticipated place in therapy and product pricing.
- Manufacturers should consider sharing this information prior to product approval, including rationale for pricing and/or pricing estimates relative to other treatment options, to help address the needs of HCDMs.
- Future research will assess how pre-approval information affects formulary coverage policies and patient access.

References

1. Brixner D, Woodward TC, Seifter N, et al. Preapproval information exchange: perspectives of U.S. population health decision makers on preferences for early engagement with investigational therapies. *J Manag Care Spec Pharm.* 2019 Feb;25(2):164–173.
2. Hydery T, Pearson A. Best practices in designing pre-approval information (PIE) for healthcare decision makers (HCDMs). Presented at: AMCP Annual Meeting; March 29–April 1, 2022; Chicago, IL.

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