Trends in Manufacturer Perceptions and Practices Regarding Pre-Approval Information

Jonathan Clark, PharmD[1]; Janet Hughes, MSE[1]; Claire Gorey, MA[1]; Amy Duhig, PhD[1]; Jay Jackson, PharmD, MPH[1]
[1]Xcenda, LLC, Palm Harbor, FL, USA

BACKGROUND

- Pre-Approval Information Exchange (PIE) is the communication of information regarding prescription drugs and medical devices to healthcare decision makers (HCDMs) about unapproved products and unapproved uses of cleared drugs.
- The PIE landscape is evolving. The Food and Drug Administration (FDA) provided final guidance on appropriate communication of both healthcare economic information (HCEI) and PIE (excluding HCEI) in June 2018. FDA guidance permits manufacturers to share certain information with HCDMs prior to product approval, as long as specified conditions are met.[1]
- PIE is particularly valuable to payers facing increased challenges to appropriately forecast budgets and set premium rates. Manufacturers benefit from PIE by increasing awareness of impending product launches and informing earlier formulary review.
- Legislation that would have potentially further clarified PIE was previously introduced into Congress (H.R. 2026). Although this bill did not move forward, further action is pending.

OBJECTIVE

- To better understand the evolving needs and considerations for PIE, trends across US biopharmaceutical manufacturer-reported practices, attitudes, and perceptions of PIE from 2018 to 2020 were compared.

METHODS

SURVEY DESIGN

- Trends in biopharmaceutical manufacturer responses were analyzed from online survey data that were collected from April to July 2018 (N=41) and March to April 2020 (N=57).
  - Provided the qualifying criteria were met for the 2020 survey (including current employment and at least moderate familiarity with PIE), a total of 16 questions were included that queried manufacturer representatives on their perspectives regarding PIE. A total of 13 PIE-related questions were included in the 2018 survey.
  - Among these questions, 7 were analyzed between surveys to compare manufacturer perceptions and practices regarding PIE over time and were the focus of this analysis.
- The PIE survey questions included open- and closed-ended questions (eg, 5-point Likert scale items, dichotomously scored items).

DATA SOURCE

- In the current study, at least one representative from over 40 different manufacturers participated in the 2018 or 2020 online survey.

ANALYTIC STRATEGY

- Descriptive and inferential statistics were used to compare and test for statistically significant differences between years and responses.
  - Fisher's tests of statistical significance were used to evaluate differences in trends for categorical variables.

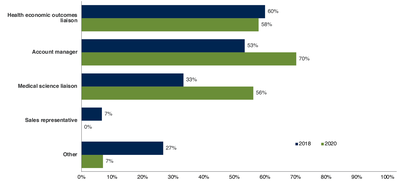
RESULTS

SURVEY RESPONDENT CHARACTERISTICS

- Titles of respondents for both 2018 and 2020 were mostly at the Director level (75% vs 67%, respectively; **Figure 1**). Departments and/or roles of respondents within their organizations consisted largely of Health Economics and Outcomes Research (HEOR), Managed Markets or Market Access, and Non-Field Medical Affairs (**Figure 2**).

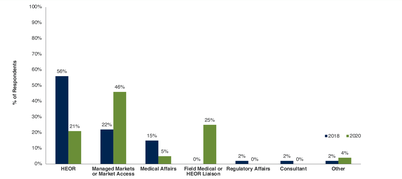
RESULTS (CONT.)

Figure 1. Current Titles for Respondents in 2018 and 2020



Q: Which of the following best describes your current title?
"Other" includes: SR Managed Care Liaison, Individual Contributor, Account Executive, Executive Director, Senior HEOR Liaison, Fellow, and BVS.

Figure 2. Respondent Departments and/or Roles in 2018 and 2020

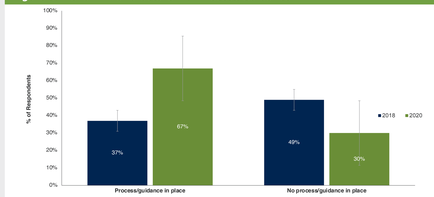


Q: Which of the following best describes your current department and/or role within your organization?
"Other" includes: Value Marketing, Director of Field-Based Medical Value Liaisons, and Policy.

TRENDS IN PIE MANUFACTURER PERCEPTIONS AND PRACTICES FROM 2018 TO 2020

- Compared to 2018, a significantly greater number of respondents in 2020 reported having a specific process/guidance in place within their organization to approve PIE materials (X^2 = 6.08, P<0.05; **Figure 3**).

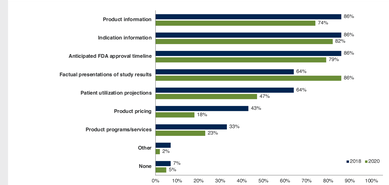
Figure 3. Trend in Manufacturer PIE Process/Guidance



Q: Is there a specific process/guidance (eg, standard operating procedure, formal committee) in place within your organization to approve materials intended for PIE?
Chi-square test for differences between years: X^2 (2) = 6.08; P<0.05.
Error bars represent 95% confidence intervals.
Note: "No process/guidance in place" includes the following responses from 2018 to 2020: "No, my organization does not have a specific process/guidance in place for approval of PIE materials, but a process/guidance is currently under development" (39% vs 23%, respectively) and "No, my organization does not have a specific process/guidance in place, and there are no plans for development of one over the next 12 months" (10% vs 7%, respectively). Excludes respondents that selected "Not applicable. My company does not have any preapproval products for which PIE would apply," "Not sure," and "Other."

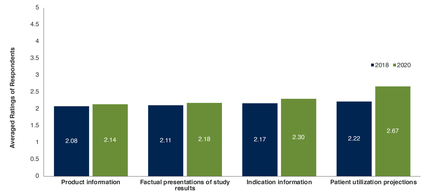
- Overall, no significant differences were observed in the types of approved PIE between 2018 and 2020 (see **Figure 4**).
  - Product pricing information was rated as "very difficult" or "extremely difficult" at higher rates in 2020 compared to 2018 (50% vs 0%, respectively).
  - Product-related programs or services (eg, patient support programs) were also rated as "not at all difficult" or "not very difficult" at a lower rate in 2020 compared to 2018 (38% vs 80%, respectively).
  - Similar mean levels of approval difficulty were endorsed across 2018 and 2020 respondents for product information, information about product indications, patient utilization projections, and factual presentations of clinical findings (**Figure 5**).

Figure 4. Trends in Approved Types of Information for PIE



Q: Which, if any, of the following types of information about investigational products are approved in your organization for PIE discussions with eligible entities?
"Other" includes: Distribution strategy / REMS plans, unknown.
Note: Sample size for 2018 was restricted based on the previous survey question response regarding having a PIE process/guidance in place (n=15).

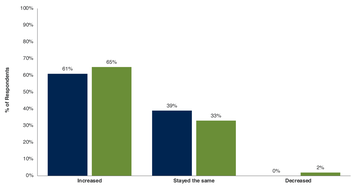
Figure 5. Trend in Difficultly of Approval for Select Types of PIE by Rating (1=Not at all difficult, 5=Extremely difficult)



Q: For each type of PIE listed please rate the level of difficulty experienced in gaining approval.
Note. Respondents were asked to rate difficulty on a scale of 1 to 5 (1 = not at all difficult, 5 = extremely difficult). Higher values suggest greater levels of difficulty for approval.

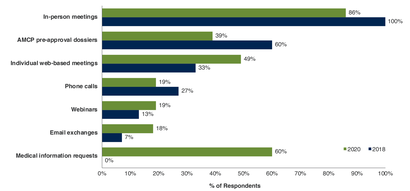
- Rates of proactive communication with payers regarding PIE were found to marginally increase from 2018 to 2020 (**Figure 6**), though these increases were not statistically significant (P>0.05).
- Manufacturers also reported using a variety of methods to communicate PIE to payers (**Figure 7**).

Figure 6. Trend in Frequency of Proactive Communication About PIE With Payers



Q: Within the past year, how would you characterize the change in frequency in your organization's proactive communication of information on unapproved products or unapproved uses of approved products with payers?

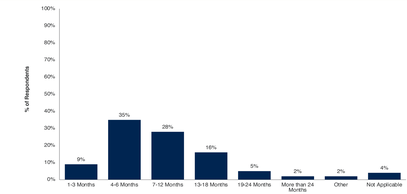
Figure 7. Trend in Methods Used by Manufactures to Communicate PIE



Q: How has your organization communicated or how is your organization currently communicating pre-approval information to eligible entities?

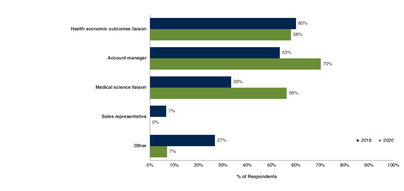
- In 2020, most respondents (63%) reported disseminating PIE materials 4 to 12 months prior to the anticipated approval date (**Figure 8**).
- Types of positions conveying information about PIE to eligible entities was comparable in 2018 and 2020 for health economic outcomes liaisons, though numbers appeared to increase for account manager and medical science liaisons (**Figure 9**).
- 70% of manufacturers in 2020 were required to verify the audience for PIE and reported several key barriers to the provision of PIE, including:
  - Confidentiality concerns (39%)
  - Lack of resources to develop materials (25%)
  - Lack of resources to disseminate materials (5%)
  - Internal education needs (35%)
  - Lack of functional alignment on the need for PIE (30%)
  - Lack of internal approval and/or dissemination process (25%)
  - Other (14%; eg, uncertainty around product, compliance concerns, strategy development)

Figure 8. Reported Timing for Disseminating PIE Materials Prior to Anticipated Approval in 2020



Q: How early is your organization disseminating or planning to disseminate pre-approval information?

Figure 9. Trends in Organizational Positions Communicating Information About PIE



Q: Who in your organization is conveying and/or is planning to convey pre-approval information to eligible entities?
"Other" includes: Trained National Accounts, Payer Account Executives, Medical Director, Medical Affairs, Executive Team, Medical Value Liaison, Managed Markets, and Market Access.
Error bars represent 95% confidence intervals.

CONCLUSIONS

- From 2018 to 2020, the number of manufacturers with a process or guidance in place to approve PIE materials has nearly doubled (37% vs 67%; P<0.05). Many manufacturers also are in the process of developing these processes within the next year.
- In addition, the change in frequency of proactive PIE has increased from 2018 to 2020 (61% vs 65%, respectively), reflecting growing interest from biopharmaceutical manufacturers to provide such communications to payers to better inform their formulary decisions. In 2020, nearly two-thirds of respondents reported disseminating PIE materials 4 to 12 months prior to the anticipated approval date.
- These recent trends show that manufacturers have continued to adapt to payer needs for PIE. However, there is a continued need for legislative clarity to provide appropriate guardrails. Until this occurs, manufacturers will continue to remain heterogeneous in their approach and will need a customized strategy and tactical plan for effective pre-approval communications.

Reference

1. US Food and Drug Administration. Drug and device manufacturer communications with payors, formulary committees, and similar entities – questions and answers. Guidance for industry and review staff. www.fda.gov/media/102683/download. June 2018. Accessed September 28, 2020.

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Contact

- For any inquiries, please contact Amy Duhig (amy.duhig@xcenda.com).

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