The Impact of Legislative Changes and Regulatory Guidance on Proactive Dissemination of Healthcare Economic Information: Payers' Experiences Pre- and Post-Product Approval

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BACKGROUND

- In December 2016, the 21st Century Cures Act was signed into law, along with Section 3037, amending Section 114 of the Food and Drug Administration (FDA) Modernization Act of 1997 (FDAMA 114). Section 3037 aimed to clarify several ambiguous statements in an effort to eliminate barriers to the proactive dissemination of healthcare economic information (HCEI) for approved products by manufacturers.
- In January 2017, the FDA issued a draft guidance titled, "Drug and Device Manufacturer Communications With Payers, Formulary Committees, and Similar Entities – Questions and Answers. Guidance for Industry and Review Staff."[1] In addition to providing further information about post-approval HCEI, the FDA also clarified its stance on proactive communication about investigational products, also known as pre-approval information exchange (PIE). The FDA permits PIE and identified several types of information that manufacturers may proactively provide to healthcare decision makers prior to product approval. However, the FDA and federal law limit proactive sharing of HCEI about investigational products despite the fact that pharmaceuticals are evaluated and policies are considered for therapies prior to FDA approval without HCEI. Thus, an avenue permitting HCEI PIE may allow for more informed decision making.
- Together, the 21st Century Cures Act (Section 3037) and the FDA draft guidance provide greater clarity regarding permitted manufacturer communications of post-approval HCEI and pre-approval information about investigational products. However, the effect of these efforts remains unclear.

OBJECTIVE

- To understand how recent legislation and guidance has affected payers' experiences and perceptions of post-approval HCEI communication and PIE.

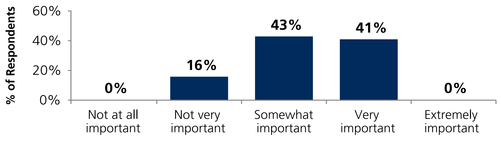
METHODS

- Payers were recruited through Xcenda's market research panel and completed an online survey between 12/22/17 and 1/5/18.
- The 28-item survey inquired about the importance, impact, communication, and changes in frequency and quality of HCEI and PIE received by payers in the past year.

RESULTS

- Payers (N=44) largely represented managed care organizations (68%), pharmacy benefit managers (27%), and/or integrated health delivery systems/integrated delivery networks (16%). Payers oversaw or managed approximately 271.7 million lives at a regional (65%) or national (35%) level. Most payers were either pharmacy directors (64%) or medical directors (25%), and all were directly involved in medical policy, formulary decisions, and/or tracking utilization management within their organization. The majority of payers (70%) served as voting members in their organization's pharmacy and therapeutics committee.
- The majority of respondents (84%) rated post-approval HCEI as "somewhat" to "very" important for formulary or medical policy decision making (**Figure 1**). Respondents reported that post-approval HCEI affected the formulary decision-making process in various ways, such as helping to support the decision to include a product on a formulary (endorsed by 34% of respondents), place competitor products on separate tiers (43%), add marketed products on a formulary (30%), and determine utilization management criteria (48%). Nearly three-quarters (73%) of respondents reported using post-approval HCEI for all 4 decisions. Approximately one-fourth (27%) of respondents indicated that post-approval HCEI had no effect on formulary decision making.

Figure 1. Importance of Post-Approval HCEI in Formulary or Medical Policy Decisions

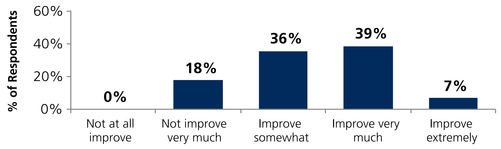


Q: How important is HCEI to the formulary or medical policy decision-making process within your organization?

Key: HCEI – healthcare economic information.

- Nearly two-thirds (64%) of respondents perceived a gap between needed and available post-approval HCEI. Among those respondents, approximately half (46%) noted that addressing this gap would "very much" or "extremely" improve formulary decision making (**Figure 2**). Half (50%) of respondents also indicated that their organization's formulary decision making was "somewhat" to "extremely" limited by the amount and type of HCEI shared by manufacturers (data not shown).

Figure 2. Impact of Addressing the Gap Between Needed and Available Post-Approval HCEI

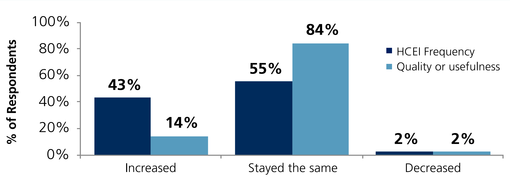


Q: If the gap between the HCEI your organization needs to support formulary decision making and what HCEI is available were closed, how much would it improve your formulary decision-making ability?

Key: HCEI – healthcare economic information.

- A large majority (80%) of respondents "sometimes" or "rarely" received post-approval HCEI from manufacturers (data not shown). Further, while 55% reported no change in the frequency of proactive communication of post-approval HCEI by manufacturers over the past year, 43% reported an increase. The quality or usefulness of HCEI communication over the past year remained the same, according to 84% of respondents (**Figure 3**).

Figure 3. Change in Frequency and Quality or Usefulness of Post-Approval HCEI Since December 2016



Q: Over the past year (since December 2016), how would you characterize the change in frequency for proactive communication of HCEI by manufacturers with marketed products?

Q: Over the past year (since December 2016), how would you characterize the change in quality or usefulness of the type of information for proactive communication of HCEI by manufacturers with marketed products?

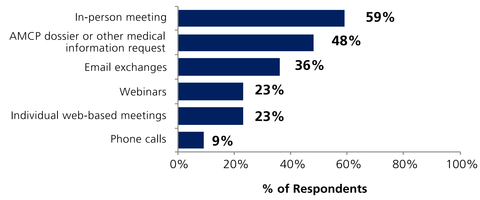
Key: HCEI – healthcare economic information.

- Over half (59%) of survey respondents ranked[a] in-person meetings as the most preferred way to communicate with manufacturer representatives about post-approval HCEI (**Figure 4**). Approximately half (52%) of respondents would consider post-approval HCEI "very" or "extremely" credible if delivered by a health economic outcomes liaison, followed by a medical science liaison (45%), account manager (25%), and sales representative (5%).

[a] Ranked as first or second.

RESULTS (cont.)

Figure 4. Preferred Way to Communicate With Manufacturers About Post-Approval HCEI (Ranked First or Second)

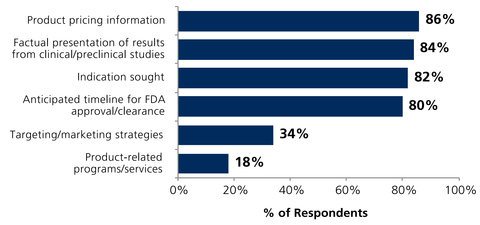


Q: Please rank order your preferred way to communicate with manufacturer representatives regarding HCEI.

Key: AMCP – Academy of Managed Care Pharmacy; HCEI – healthcare economic information.

- Over three-quarters of respondents reported that it is "very" or "extremely" important to proactively receive product pricing information (86%), factual presentation of results from clinical or preclinical studies (84%), information about the indication sought (82%), and anticipated timeline for possible FDA approval/clearance prior to the product's approval (80%) (**Figure 5**).

Figure 5. Importance of PIE by Type of Information



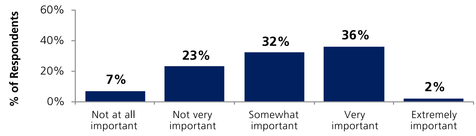
*Q: According to the draft guidance, the following information about **investigational products** may be communicated by manufacturers to a payer or similar organization. For each of the following, please rate the level of importance to you or your organization in receiving this information proactively and prior to approval from a manufacturer.*

Note: Percentages reflect the proportion of respondents who rated each type of information as "very" or "extremely" important.

Key: FDA – Food and Drug Administration; PIE – pre-approval information exchange.

- While manufacturers are not currently permitted to share HCEI PIE, payers' perceptions about the importance of proactively receiving HCEI regarding investigational products was also assessed; 39% of respondents rated the importance of HCEI PIE as "very" or "extremely" important, while 30% rated it "not very" or "not at all" important (**Figure 6**).

Figure 6. Importance of HCEI PIE

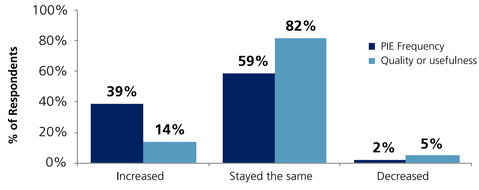


Q: The FDA draft guidance does not include PIE related to HCEI. How important is it for you to receive proactive HCEI communications about investigational products?

Key: FDA – Food and Drug Administration; HCEI – healthcare economic information; PIE – pre-approval information exchange.

- Prior to January 2017, the majority of respondents (64%) "rarely" or "never" received PIE from manufactures (data not shown); 39% reported an increase in PIE over the past year, while 59% reported no change in frequency. The quality or usefulness of PIE over the past year remained the same, according to 82% of respondents (**Figure 7**).

Figure 7. Change in Frequency and Quality or Usefulness of PIE Since January 2017



Q: Over the past year (since January 2017), how would you characterize the change in frequency for proactive communication by manufacturers for investigational products?

Q: Over the past year (since January 2017), how would you characterize the change in quality or usefulness of the type of information shared proactively about investigational products?

Key: PIE – pre-approval information exchange.

- Close to half of respondents (45%) perceived a gap between the needed and available PIE. Among these respondents, nearly one-third (30%) indicated that addressing this gap would "very much" or "extremely" improve their formulary decision-making ability, while 65% noted that it would "somewhat" improve decision-making (data not shown).
- Almost all respondents (95%) indicated that they would like to receive information from a manufacturer within 1 year of the product's anticipated approval. Among those respondents who want to receive PIE from manufacturers, 91% ranked[b] a medical science liaison as the most preferred manufacturer representative to share such information, followed by health economic outcome liaison (56%), an account manager (51%), and sales representative (0%).

[b] Ranked as first or second.

CONCLUSIONS

- New legislation and regulations pertaining to the proactive communication of HCEI and PIE may have prompted an increase in such communication by manufacturers in the past year.
- Payers view post-approval HCEI and PIE about investigational products as important, yet a gap currently exists between the information received and the information needed. The gap pertaining to PIE may, in part, stem from the lack of PIE related to HCEI—information that payers identify as important. These unmet needs may affect the utilization of HCEI and PIE for decision-making purposes.
- More frequent, relevant, and timely communications between manufacturers and population health decision makers may be helpful for improving formulary decision making and ultimately, treatment decisions for patients in need of therapy.
- HCEI PIE may be particularly helpful for decision making, and therefore, legislative efforts should support the communication of this information.

LIMITATIONS

- Survey respondents were from Xcenda's Managed Care Network® and may not be generalizable to all decision makers receiving HCEI.
- This cross-sectional study was purely descriptive and associations must be interpreted with the study design in mind.
- Response and recall bias may have affected the results obtained.

1. FDA. Drug and device manufacturer communications with payors, formulary committees, and similar entities – questions and answers. Guidance for industry and review staff. https://www.fda.gov/downloads/drugs/guidancecomplianceregulatoryinformation/guidances/ucm537347.pdf. Published January 2017.

Presented at International Society for Pharmacoeconomics and Outcomes Research 2018 | Poster Presentations, Session 3: Healthcare Use & Policy Studies
Tuesday, May 22, 2018 | This research was conducted by Xcenda, LLC, in partnership with the Academy of Managed Care Pharmacy.

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