Pre-Approval Communication of Health Care Economic Information to U.S. Payers

Opening Doors for Patient Access

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The communication of health care economic information (HCEI) to payers in the United States (U.S.) **before** regulatory approval is an area of increasing interest and importance. Health care decision makers, specifically payers, formulary committees, etc., need to evaluate their plans and rates a year or more in advance in order to meet submission deadlines that often fall six to nine months before the start of a plan year.[1] Allowing manufacturers to share HCEI with payers prior to U.S. Food and Drug Administration (FDA) approval can lead to more accurate forecasting of budgets, more precise rates, and the possibility of more affordable patient access.[1] Historically, there have been a number of regulations regarding what can be discussed prior to FDA approval, but over the past 20 years there has been an effort to increase the amount of information that can be shared about a drug before its approval, most notably the Food and Drug Administration Modernization Act (FDAMA) Section 114 in 1997[2] and the FDA draft guidance on the communication of HCEI between drug and device manufacturers and formulary decision makers in 2017.[3]

FDA Draft Guidance on the Communication of Health Care Economic Information

This draft guidance on communication of HCEI, released in January 2017, aimed to clarify FDAMA Section 114, which was passed nearly 20 years earlier to facilitate HCEI exchange.[2] Because of the ambiguity surrounding FDAMA 114, few manufacturers took advantage of the act for fear of penalties associated with off-label promotion.[4] Given the need of formulary decision makers to review this information, the FDA draft guidance defines what constitutes HCEI, as well as who is considered an appropriate audience for such information *(Figure 1)*.[3] Examples of HCEI that manufacturers can communicate include budget impact models, health care utilization, and information on product pricing.[3, 4]

Format for Communication

One question that has been posed is whether or not a standardized format, such as one similar to the AMCP Format, should be used to communicate HCEI prior to FDA

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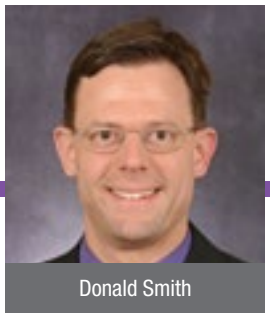

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approval.[1] While this practice would probably benefit those familiar with the AMCP Format, it may not aid others who are unfamiliar with this framework.[1] However, starting with the AMCP Format should facilitate the development of a complete AMCP dossier when one is needed for post-approval decision making. In addition, the AMCP Format is updated on a regular basis, which allows it to adapt to reflect changing payer evidence needs.

The AMCP eDossier system

Currently, manufacturers may also provide HCEI to formulary decision makers through the AMCP eDossier system.[5] Using this system, manufacturers are notified when a formulary decision maker has requested information, and after directly authorizing the request, manufacturers may grant access to the dossier. However, manufacturers may not proactively distribute information to formulary decision makers or directly inform them that a dossier is available without first receiving an unsolicited request. Therefore, the AMCP eDossier System does not proactively inform payers that a particular dossier is available on the system.[6]

In accordance with version 4.0 of the AMCP Format, manufacturers may include dossier information on the eDossier system prior to FDA approval,[5] however, as noted above, payers must make an unsolicited request to receive the pre-approval dossier information.[6] In a November 2016 survey of payers currently using the eDossier system (N=172), more than 85% had been involved in requesting pre-approval information within the last year.[6] Payers in the same survey responded that the manufacturer response rate to information requests was better (40%) or the same (26%) for those using the eDossier system compared with manufacturers who did not.[6]

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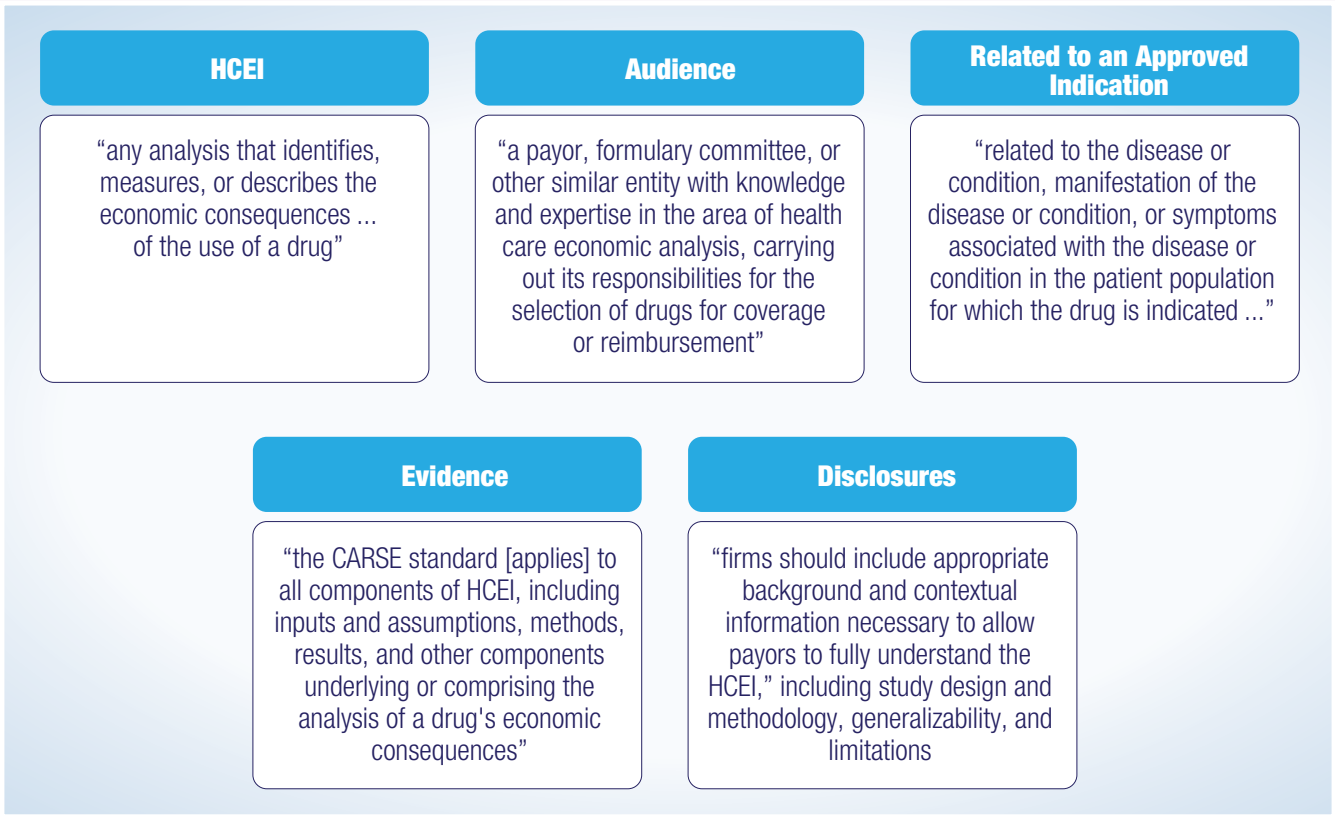
In addition to providing another pathway through which manufacturers can share HCEI with payers, the AMCP eDossier system also:

- Allows manufacturers to verify that their dossier is available[6]
- Informs payers when new approvals occur and when updated labels are available[5]
- Provides Prescription Drug User Fee Act (PDUFA) dates to help keep payers aware of when different drugs might be obtaining approval[6]

Figure 1. FDA Draft Guidance on Manufacturer Communication with Payers, Formulary Committees, and Similar Entities



CARSE=Competent and Reliable Scientific Evidence; **HCEI**=Health Care Economic Information

SOURCE: FDA 2017[3]

A manufacturer using the AMCP eDossier system also knows which payers have requested the dossier because the manufacturer is the one who grants access to the payer's unsolicited request.[6] In addition, payers can designate a timeframe during which they will need the information (e.g., during the next three months).[6] Designating a timeframe allows payers to request pre-approval and post-approval dossier information even if an AMCP dossier is not available (e.g., sometimes an AMCP dossier is not available when a drug receives approval).[6]

The Pharmaceutical Information Exchange (PIE) Act

In April 2017, the Pharmaceutical Information Exchange (PIE) Act (HR 2026) was introduced to the House of Representatives by Representative Brett Guthrie (R-KY). The PIE Act would provide for earlier exchange of HCEI, theoretically leading to quicker and improved patient access following FDA approval. Unlike the current system of HCEI, in which an unsolicited request from a formulary decision maker is required to initiate the exchange, HR 2026 provides for the proactive exchange of HCEI.[7] Formulary decision makers have consistently called for access to pipeline information 12 to 18 months prior to approval to accurately forecast the following year's budget and premiums.[6] HR 2026 is currently under review by the House Energy and Commerce Committee, but if it does eventually become a law, it has the potential to empower formulary decision makers to conduct quicker and more accurate assessments of drugs for their members.

Conclusion

Currently, the U.S. health care system is still evaluating the optimal way in which to use HCEI to accurately manage health care costs, but recent regulation and guidance have been instrumental in making improvements to the system. There is increased communication between the key stakeholders – drug manufacturers, the FDA, and payers – and continuing efforts to improve the system in a safe and structured manner. With the FDA draft guidance allowing some communication of HCEI to payers prior to regulatory approval, and the potential for even greater expansion of this communication through HR 2026, the U.S. could see improved formulary decisions, budget forecasting, and precision in rates, all translating to better patient access to medical treatments.

It is imperative that biopharmaceutical companies continue the dialogue with the FDA, payers, and other stakeholders (such as AMCP) to keep the momentum moving forward on this issue. With the changing landscape of health care and the shifting emphasis to value-based pricing, early approval of treatments to meet unmet need, and personalized medicine, the pre-approval communication of HCEI will only become more important to payers to adequately plan for patient access to the treatments they need. ■

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