Prepublication Overview

AMCP Format for Formulary Submissions – Guidance on Submission of Pre-approval and Post-approval Clinical and Economic Information and Evidence, Version 4.1

Background

AMCP will release Version 4.1 of the AMCP Format for Formulary Submissions in December 2019. It includes new sections describing the evidence needs of health care decision makers (HCDMs) for unapproved products nearing the end of the product development pipeline, as well as unapproved uses of existing products for which FDA approval is being sought. By defining those information needs, AMCP Format 4.1 provides recommendations that can be helpful for biopharmaceutical companies and other stakeholders involved in the preparation of product dossiers.

Since its initial release in 2000, the Format has become the gold standard by which manufacturers submit the objective, credible, and relevant information required by HCDMs in their consideration of products for formulary placement. The new version continues AMCP's legacy of providing tools that help ensure patients get the medications they need at a cost they can afford.

Changes in AMCP Format 4.1 reflect the growing pace of pharmaceutical innovation, particularly the advent of more complex, high-cost therapies coming to market. Today, HCDMs are calling for mechanisms to remove barriers to patient access by obtaining information sooner to consider and plan for these new products.

To address this need, the AMCP Format Executive Committee began work in late 2018 to expand Format 4.0 to define information needs to support pre-approval assessments and budgeting. In the summer of 2019, AMCP collected public comments on a draft revision of the publication from manufacturers, payers, consultants, academia, and associations. The committee then worked to address those comments with the result being AMCP Format 4.1.

What's in Format 4.1

AMCP Format 4.1 builds on existing guidance on dossiers for approved products by providing expanded recommendations for unapproved products as well as unapproved uses of approved products for which FDA approval is being sought. These additions are in accordance with HCDMs' evidence and information needs and complement the long-standing guidance for the traditional dossier for approved products. Specifically, Version 4.1 includes guidance on three types of dossiers that reflect the evolving nature of a product's lifecycle:

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I. Unapproved Product Dossier

- A document containing factual presentations of evidence supporting the development of an unapproved product
- No characterizations/conclusions should be made regarding the safety or effectiveness of the unapproved product
- Inclusion of anticipated product price or price range is recommended.

This dossier aligns with early phases of a product's lifecycle—when a product is not yet approved by the FDA. The unapproved product dossier evolves to an approved product dossier once the product is approved.

II. Approved Product Dossier

- A comprehensive document containing clinical and economic evidence and information about an FDA-approved product, including off-label information supported by evidence
- Inclusion of information on product price, economic and outcomes research, economic models, budget impact, and cost-effectiveness is recommended
- Used to convey the overall value proposition of the product based on clinical and economic evidence and information

This dossier aligns with an FDA-approved product.

III. Unapproved Use Dossier

- A document containing factual presentations of evidence supporting the development of an unapproved use of an approved product for which FDA approval is being sought
- No characterizations/conclusions should be made regarding the safety or effectiveness of the unapproved use
- Inclusion of the price that is already known for the approved product and a description of any potential or anticipated changes in cost based on the unapproved use is recommended

This dossier aligns with the post-FDA approval phase, but before FDA new-use approval. This dossier may co-exist with an approved product dossier that contains information on the unapproved use.

As with previous versions of the AMCP Format, updates to dossiers are recommended whenever new information becomes available. However, the Format is not a mandate and instead serves as guidance to describe the information needs of HCDMs. Development and use of dossiers is at the discretion of the manufacturer and is subject to their individual legal and regulatory compliance policies.

Format 4.1 Relationship to Preapproval Information Exchange Legislation

Pre-approval Information Exchange (PIE) is a concept designed to improve patient access to emerging pharmaceuticals and devices. PIE allows manufacturers and population health decision makers to share information about certain health care economic and scientific information on products ahead of FDA approval. The need for PIE communications is especially important as the health care system evolves from a fee-for-service payment system to a system rewarding quality, improved patient outcomes, and value.

AMCP recognizes that FDA, under circumstances outlined in a June 2018 FDA Final Guidance, Drug and Device Manufacturer Communications with Payors, Formulary Committees, and Similar Entities ("FDA Final Guidance"), permits drug and device manufacturers to communicate with payers and other health care decision-makers (HCDMs) regarding health care economic information prior to drug approval. These communications are supported by a broad array of stakeholders, all of whom recognize the various ways in which these communications can be beneficial, such as in developing and implementing value-based contracts, or in expediting coverage decisions for emerging therapies that FDA approves as breakthroughs.

For the last three years, AMCP has been working diligently on bipartisan legislation that would codify current regulatory safe harbors that allow for PIE between manufacturers and HCDMs. Such a safe harbor would allow for the sharing of truthful and non-misleading clinical and economic information about medications and devices in the pipeline, as well as new uses of approved products, prior to FDA approval during the forecasting and rate setting process.

A legislative safe harbor for PIE will confirm that the dissemination of certain information prior to FDA approval does not violate the prohibitions against promotional communications and does not run afoul of the labeling, misbranding, and intended use provisions of the Federal Food, Drug, and Cosmetic Act and its implementing regulations.

In the absence of this legislation, AMCP Format 4.1 is intended to align with the FDA Final Guidance, which acknowledges the needs of HCDMs to receive information about products in development.