RTI (h)(s)
Health Solutions

FDA and AMCP Guidance on Pre-Approval Submissions and the Continuous Process of Dossier Development

FDA Guidance:

Payors prefer to receive information from manufacturers well in advance of a product's approval by the FDA so that they can make timely coverage and reimbursement decisions. FDA guidance recommends providing the following unbiased, factual, accurate, and non-misleading information:

- Information about the indications sought
- The anticipated timeline for possible FDA approval, clearance, or licensure of the product or new use
- Product pricing information
- Patient utilization projections (e.g., epidemiological data projection on incidence and prevalence)
- Product-related programs or services
- Factual presentations of results from studies, including clinical studies (i.e., no characterizations or conclusions regarding the safety or effectiveness of the unapproved product or the unapproved use).

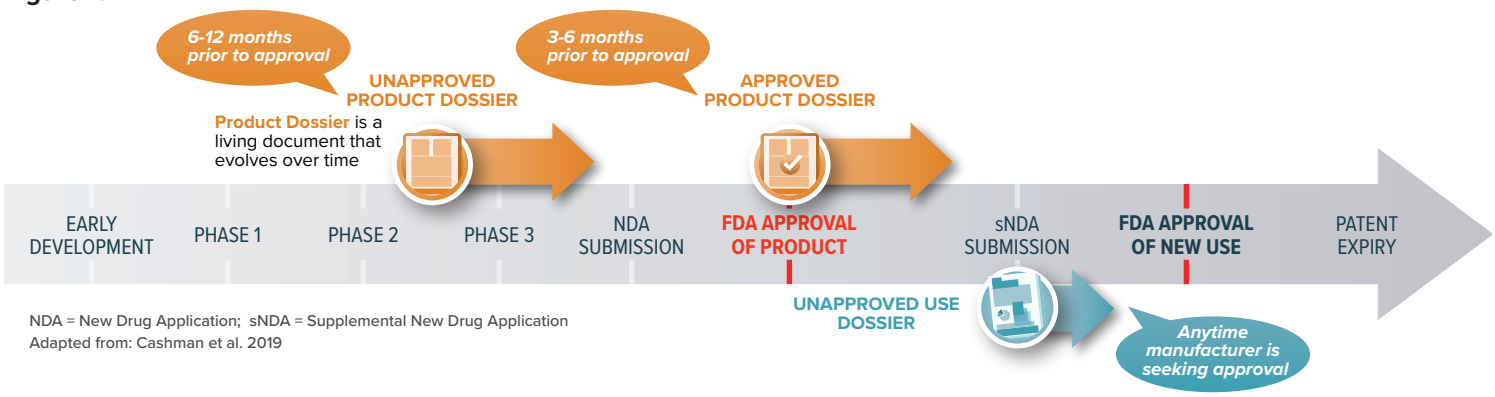
AMCP Guidance:

The AMCP Format, version 4.0 (April 2016) contains pre-approval submissions guidance that can be used as a template for information in response to a payor's request for a pre-approval dossier. In October 2019, the AMCP Format Executive Committee (FEC) provided an overview of upcoming changes to the AMCP guidelines to more closely align with FDA guidance. The FEC outlined three distinct dossier types, as shown in figure 1 and summarized in the table below.

AMCP FEC Recommendations for Content of Each Dossier (Key Sections)

	Unapproved product dossier	Approved product dossier	Unapproved use dossier
Section 1	Highlights and Overview in a single table (no executive summary/value proposition)	Executive Summary highlighting clinical and economic value	Highlights and Overview in a single table (no executive summary/value proposition)
Section 2	**Product Information and Disease Description**		
Section 3	Clinical Evidence - studies supporting unapproved product; may be text and evidence tables	Clinical Evidence - approved indications as well as off-label uses; text and evidence tables	Clinical Evidence - studies supporting unapproved use; may be text and evidence tables
Section 4	Economic Information - provide as much pricing information as possible; CEA and BIM likely not possible	Economic Value and Modeling Report - cost-effectiveness model and BIM	Economic Information - pricing should be available since product approved; economic models likely not possible

Figure 1.



NDA = New Drug Application; sNDA = Supplemental New Drug Application
Adapted from: Cashman et al. 2019

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