AMCP Format v4.1: New Guidance on Evidence Requirements for Unapproved Products and Unapproved Uses

January 23, 2020

AMCP Webinar

AMCP

Optimizing medicine.
Improving lives.

Welcome

Phil Bongiorno

Vice President, Policy & Government Relations

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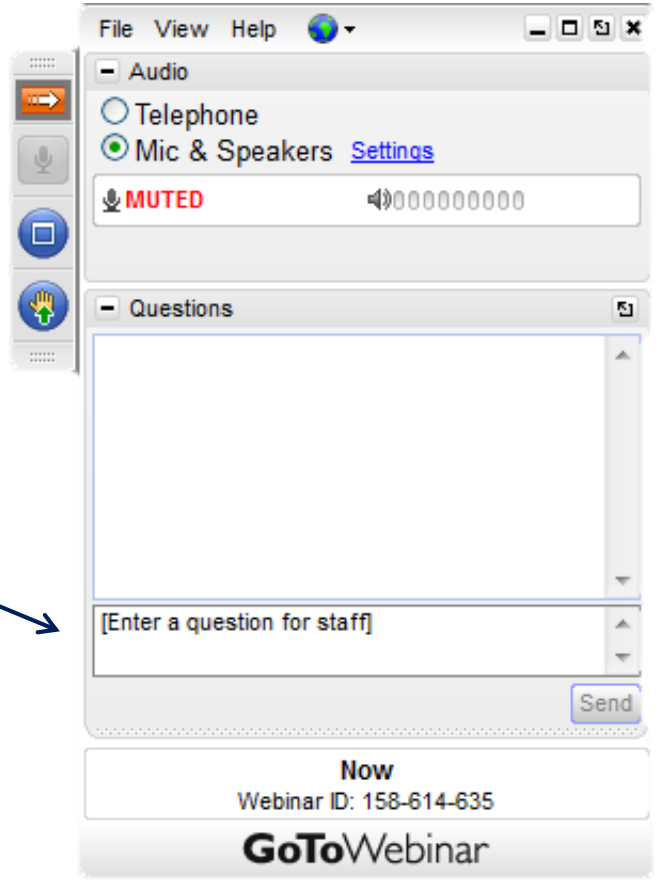

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Faculty

Iris Tam, PharmD, FAMCP
Chair, AMCP Format Executive Committee and
Senior Director, HEOR, Patient Access & Value
Coeus Consulting Group

Jennifer S. Graff, PharmD
Member, AMCP Format Executive Committee and
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Jeff White, PharmD, MS
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Meeting Payers' Needs

Jeff White, PharmD
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Update Process and Recommendations

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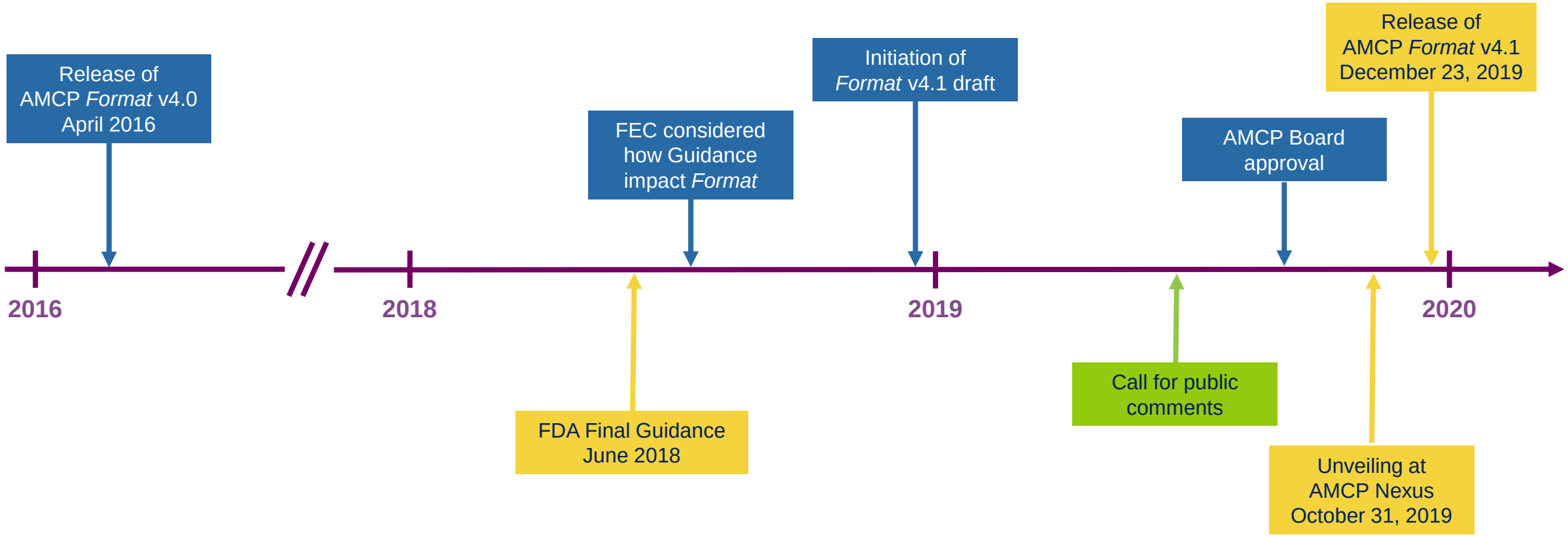
Timeline for Version 4.1



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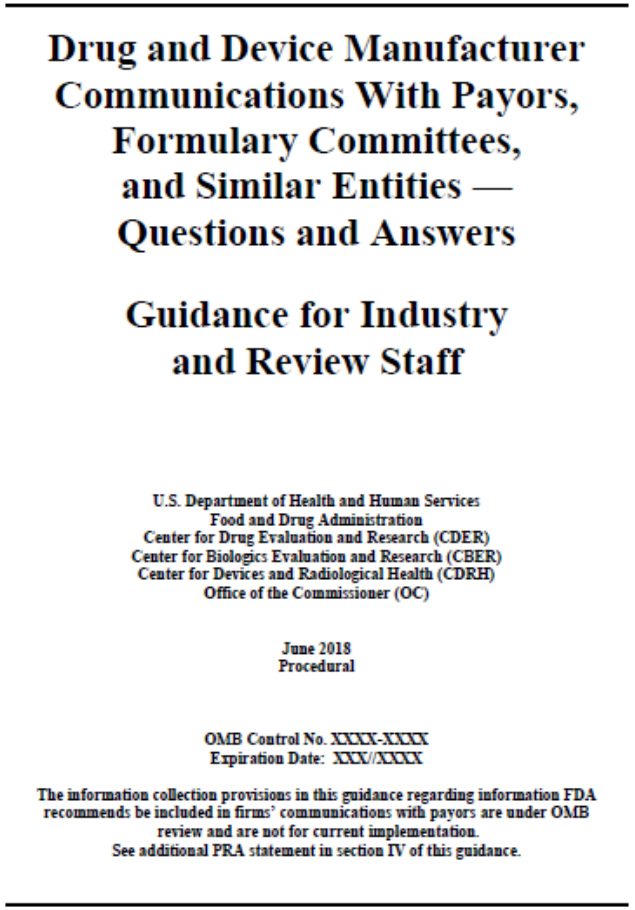
FDA Final Guidance (June 2018)

Sections A and B

- Firms' communication of health care economic information (HCEI) to payors regarding approved drugs and approved/cleared devices.[1] *This pertains to and clarifies the statute found in the Food and Drug Administration Modernization Act [FDAMA] of 1997, Section 114*.[2]

Section C

- Firms' communication to payors, formulary committees, and other similar entities about unapproved products and unapproved uses of approved/cleared products.[1]



Drug and Device Manufacturer Communications With Payors, Formulary Committees, and Similar Entities — Questions and Answers

Guidance for Industry and Review Staff

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)
Center for Biologics Evaluation and Research (CBER)
Center for Devices and Radiological Health (CDRH)
Office of the Commissioner (OC)

June 2018
Procedural

OMB Control No. XXXX-XXXX
Expiration Date: XXX//XXXX

The information collection provisions in this guidance regarding information FDA recommends be included in firms' communications with payors are under OMB review and are not for current implementation.
See additional PRA statement in section IV of this guidance.

1. Food & Drug Administration. Drug and device manufacturer communications with payors, formulary committees, and similar entities – questions and answers: guidance for industry and review staff. June 2018; Available at: https://www.fda.gov/downloads/drugs/guidancecomplianceregulatoryinformation/guidances/ucm537347.pdf. Accessed 09-12-2019.
2. Food and Drug Administration Modernization Act (FDAMA) of 1997, Section 114. Public Law 105-115. November 21, 1997; Available at: https://www.govinfo.gov/app/details/PLAW-105publ115. Accessed 09-12-2019.

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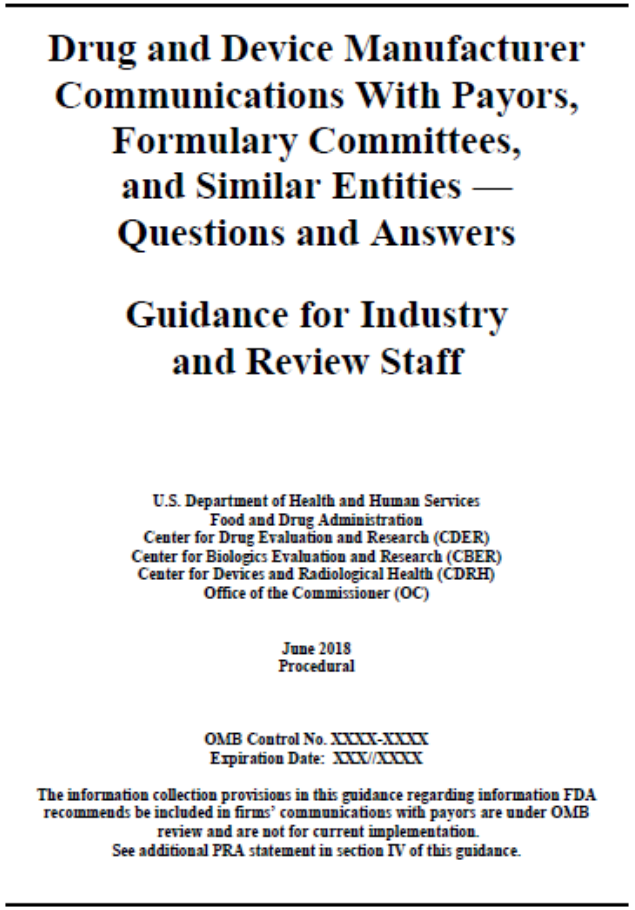

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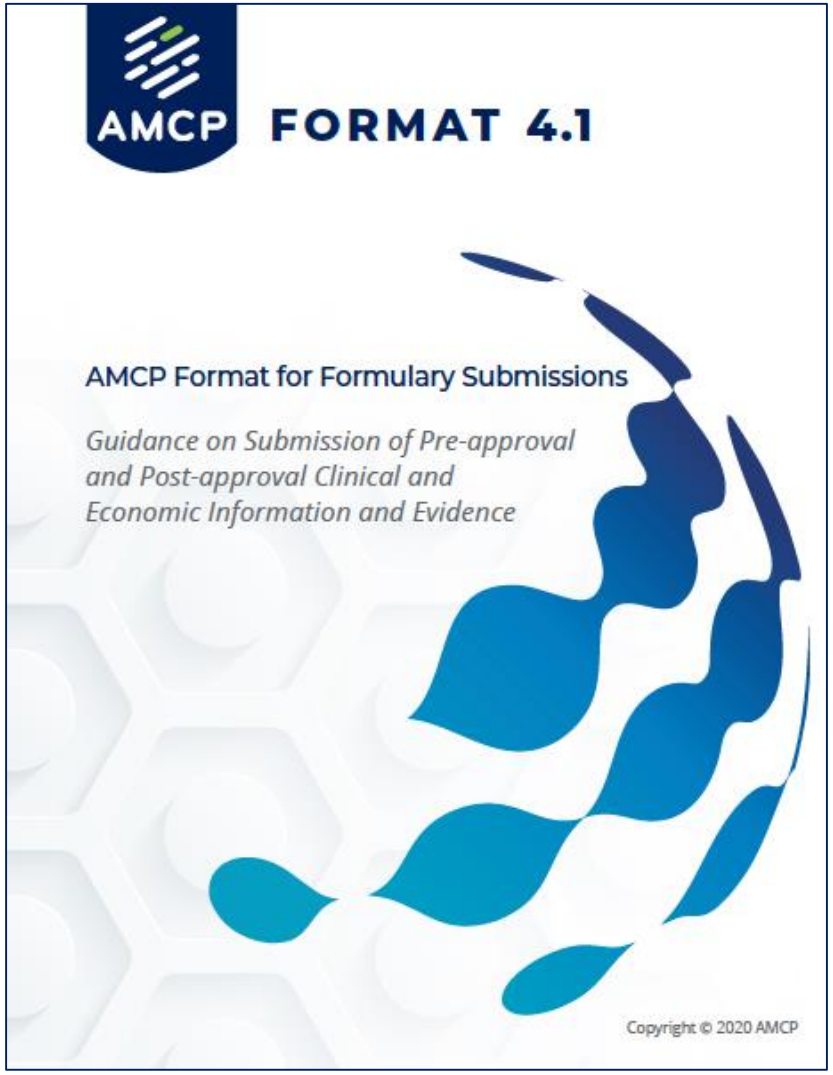
AMCP *Format for Formulary Submissions*, Version 4.1

December 23, 2019



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Key Terms Used in the AMCP *Format*

Term	Definition
Product	Medical products such as pharmaceuticals, biologics, diagnostics, or medical devices
Manufacturer	Any company that develops, manufacturers, or markets drugs, tests, or medical devices
Health Care Decision Maker (HCDM)	Any health care personnel, committee, or organization that uses an evidence-based process for making health care coverage and reimbursement decisions for patient populations
Dossier	Comprehensive and concise report containing clinical and economic evidence and information about a medical product that is developed and communicated by the manufacturer to HCDMs for the purpose of formulary coverage, policy and reimbursement decision-making
Approval	General term to reflect the appropriate FDA regulatory decision-making process needed before a medical product may be commercialized

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In General…

- The *Format* is a guidance, not a mandate
- Development of dossiers is at the discretion of the manufacturer
- Updates to dossiers should occur when new information becomes available; at the discretion of manufacturer
- Recipients of dossiers include HCDMs, payers, and entities that make or influence formulary, coverage, policy, and reimbursement decisions

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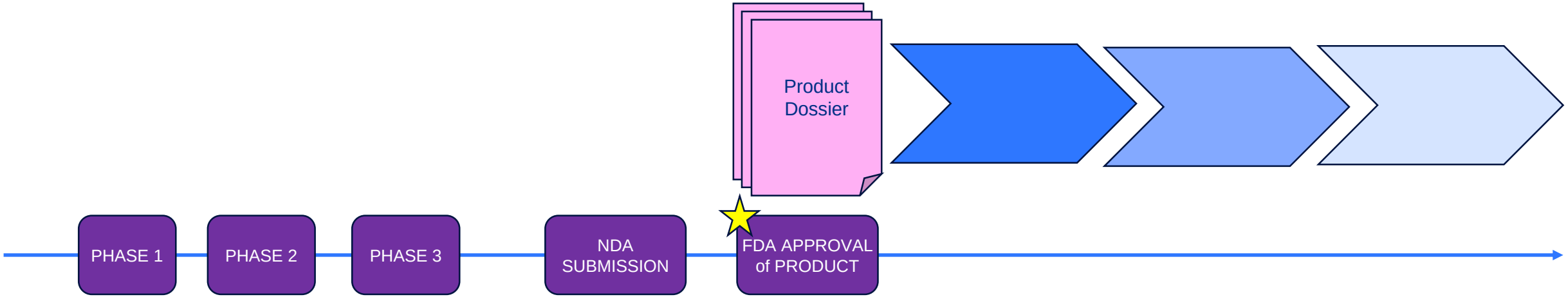
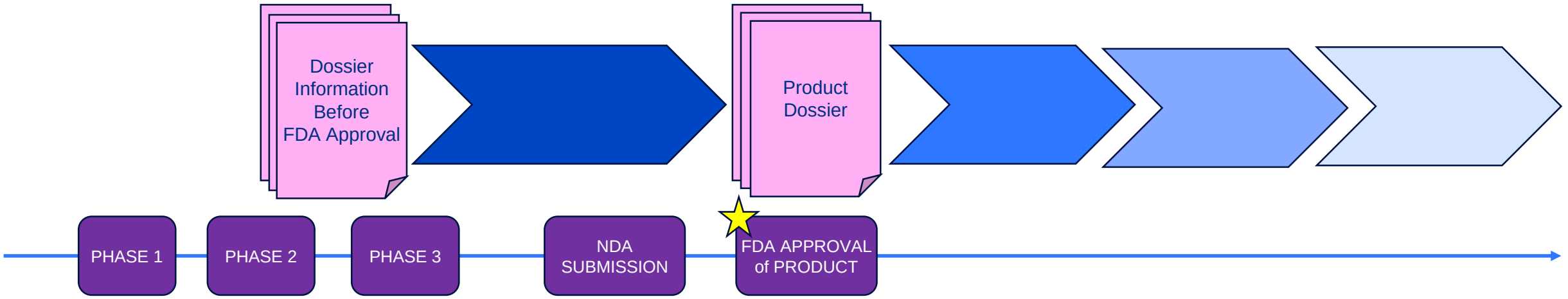
Format Version 1.0 to Version 3.1 (2000 – 2016): Product Dossier at Launch



NDA = New Drug Application

For illustrative purpose only. Timeline is not to scale.
Milestone events shown may vary and may not be all inclusive of product's life cycle
Manufacturer has discretion on the development of dossiers at all stages of life cycle.

Format Version 4.0 (2016 – 2019):
New – Information Before FDA Approval



NDA = New Drug Application

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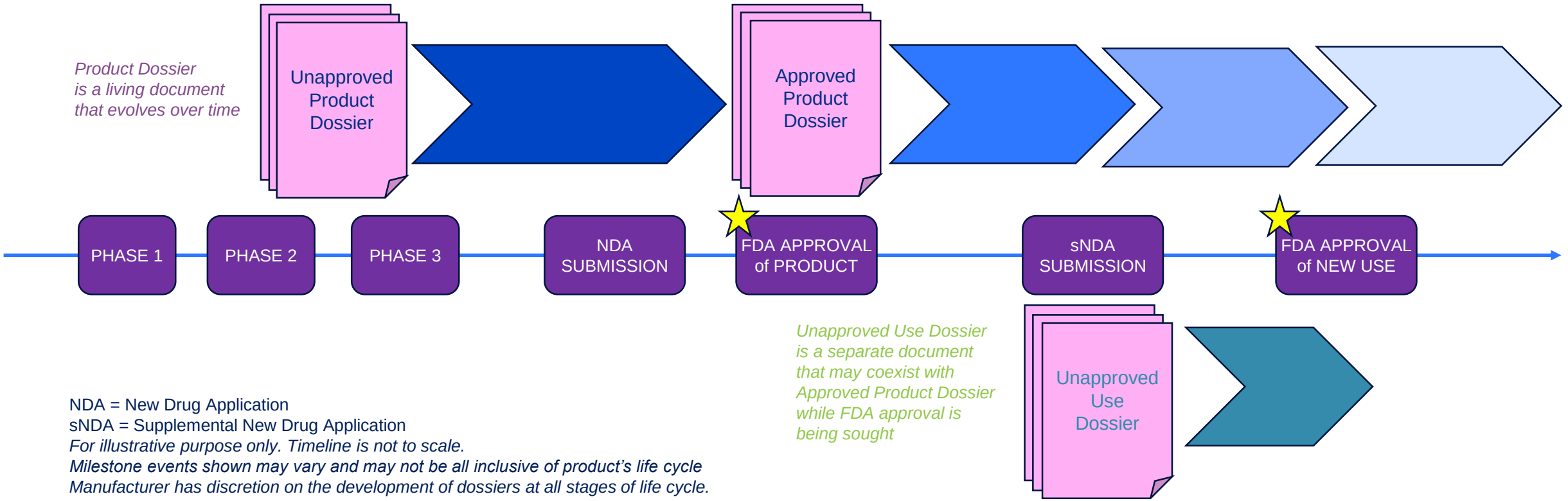
Manufacturer has discretion on the development of dossiers at all stages of life cycle.

Format Version 4.1 (2019 – present):
New – Unapproved Product & Unapproved Use Dossiers

Consider Dossiers Relative to Product Life Cycle



NDA = New Drug Application
sNDA = Supplemental New Drug Application
For illustrative purpose only. Timeline is not to scale.
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Dossier Table of Contents

Approved Product Dossier

1. Executive Summary
2. Product Information & Disease Description
3. Clinical Evidence
4. Economic Value & Modeling Report
5. Additional Supporting Evidence
6. Dossier Appendices

Unapproved Product Dossier & Unapproved Use Dossier

1. Highlights & Overview
2. Product Information & Disease Description
3. Clinical Evidence
4. Economic Information

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1: Highlights & Overview

- 1.1 Table Highlights for Unapproved Product (or Unapproved Use of an Approved Product)
- At-a-glance overview of key information
- No executive summary
- Some information may not be available
- Manufacturer should update accordingly

Shown here is Table 1.1 for Unapproved Product

Type of Information	*Description of Information*
Revision dates	List the dates of revisions to this table in reverse chronological order
Manufacturer name	List the names of companies involved in developing and marketing the unapproved product
Unapproved product name	List the names of the unapproved product (brand, generic, chemical, molecular, company-assigned name, research compound number)
Drug class	Describe the drug class in which the product belongs
Disease or anticipated indication	List the diseases, indications, and target populations for which the unapproved product is being studied and FDA approval is being sought
Special FDA designations	List special designations per FDA (e.g., fast track, orphan, breakthrough) and the date of designation; provide links to source information (e.g., FDA, press release)
NDA/BLA submission date	List the date of NDA/BLA submission to the FDA
FDA Advisory Committee meeting	List the date of the planned or anticipated FDA Advisory Committee meeting
PDUFA or FDA approval date	List the date or time frame (e.g., 2023, Q1'22) of anticipated FDA approval
Product launch data	List the date of anticipated product launch in the market
Approval dates in other countries (outside of the United States)	List other countries and (anticipated) approval dates
Phase 3 trials completed	List the name or citation of trials and dates completed, key endpoints, and number of patients; provide links to Clinicaltrials.gov or PubMed
Phase 3 trials in progress	List the name or citation of trials and dates completed, key endpoints, and number of patients; provide links to Clinicaltrials.gov or PubMed
Phase 2 trials completed	List the name or citation of trials and dates completed, key endpoints, and number of patients; provide links to Clinicaltrials.gov or PubMed
Phase 2 trials in progress	List the name or citation of trials and dates completed, key endpoints, and number of patients; provide links to Clinicaltrials.gov or PubMed
Anticipated routes and dosing information	Describe the routes of administration for the unapproved product that were used in clinical trials and anticipated to be approved by the FDA
Anticipated location/settings for product administration	Describe the location or health care setting where the product was administered in clinical trials and anticipated to be given when approved by the FDA
Prevalence of condition associated with anticipated indication in the United States	Express results per 100,000 (e.g., 1 per 100,000 women, 5 per 100,000 live births, 10 per 100,000 per year)
Annual incidence of condition associated with anticipated indication in the United States	Express results per 100,000 (e.g., 1 per 100,000 women, 5 per 100,000 live births, 10 per 100,000 per year)
Product pricing information	Indicate the anticipated annual cost per patient of the product in terms of price ranges or corridors, rather than an absolute dollar figure. For example, indicate one of the following: [ ] ≥$1,000,000 [ ] $500,000 to $999,999 [ ] $300,000 to $499,999 [ ] $100,000 to $299,999 [ ] $50,000 to $99,999 [ ] $10,000 to $49,999 [ ] ≤$9,999 Alternatively, or in addition, indicate any other information that might help HCDMs consider the anticipated cost impact of unapproved product
Anticipated patient support programs	Describe potential plans for patient support programs
Anticipated distribution strategy	Describe anticipated distribution plans for product (e.g., limited distribution)

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2: Product Information & Disease Description

- 2.1 Product Information
  - Clear statement that product (or use) is not FDA-approved
  - Phase and status of product development
  - Product information (generic/brand name, drug class, MOA, PK, dosing, etc)
  - Indications being sought
  - Timeline for commercialization
  - Product pricing information (may be provided in Section 4)
  - Patient utilization projections
  - Product-related programs or services
  - Factual information from studies (may be provided in Section 3)
  - Other factual information per manufacturer's discretion

- 2.2 Disease Description Some information may not be available
  - Exact indication of unapproved product/use is not fully known until final FDA approval
  - Epidemiology, risk factors, pathology, clinical presentation, burden of disease

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3: Clinical Evidence

- 3.1 Study Summaries
- 3.2 Evidence Tables
- Factual presentation of studies (phase 1, 2, 3 studies; peer-reviewed publications; congress proceedings; ClinicalTrials.gov; data on file per manufacturer's discretion
- No characterizations or conclusions should be made regarding safety or effectiveness
- Provide study summaries or evidence tables or both

4: Economic Information

- Strongly recommended that manufacturers provide as much product pricing information as possible (for unapproved use, price of product is known but describe any potential changes)
- Budget impact and cost-effective models may not be feasible
- Possible ways in providing pricing information
  - Price ranges or corridors, rather than absolute dollar figure
  - Directional estimates relative to other treatment options
  - Rationale for pricing strategy

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Key Challenges and Considerations

Jennifer Graff, PharmD
Vice President, Comparative Effectiveness Research
National Pharmaceutical Council

What is an Unapproved Product Dossier?
What is an Unapproved Use Dossier?

Unapproved Product Dossier	Approved Product Dossier	Unapproved Use Dossier
Document containing factual presentation of evidence supporting the development of an unapproved product No characterizations/ conclusions should be made regarding the safety or effectiveness of the unapproved product	Comprehensive document containing clinical and economic evidence and information about an FDA-approved product, including off-label information supported by evidence Used to convey the overall value proposition of the product	Document containing factual presentation of evidence supporting the development of an unapproved product No characterizations/ conclusions should be made regarding the safety or effectiveness of the unapproved use

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Why are new dossiers needed?

Unapproved Product Dossier	Approved Product Dossier	Unapproved Use Dossier
HCDMs need to plan and budget for future coverage and reimbursement decisions about unapproved products before FDA approval	To evaluate an approved product for formulary, coverage, policy, or reimbursement decisions	HCDMs need to plan and budget for future coverage and reimbursement decisions about unapproved uses before FDA approval

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Can product value proposition be communicated in a dossier?

Unapproved Product Dossier	Approved Product Dossier	Unapproved Use Dossier
Factual evidence grounded in clinical and economic evidence and information may be provided. No characterizations or conclusions should be made regarding the safety or effectiveness of the unapproved product	Yes, value that is grounded in clinical and economic evidence and information may be described	Factual evidence grounded in clinical and economic evidence and information may be provided. No characterizations or conclusions should be made regarding the safety or effectiveness of the unapproved use

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When should the dossier be available?

Unapproved Product Dossier	Approved Product Dossier	Unapproved Use Dossier
Anytime before FDA approval May be 6 to12 months or up to 2+ years before FDA approval	Anytime after FDA approval	Anytime before FDA approval May be 6 to12 months or up to 2+ years before FDA approval

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What clinical content can be in the dossier?

Unapproved Product Dossier	Approved Product Dossier	Unapproved Use Dossier
Factual presentation of clinical evidence for the unapproved product that is available at the time of communication No characterizations/ conclusions should be made regarding the safety or effectiveness of the unapproved product	Clinical evidence and information regarding an approved product, including any off-label uses supported by evidence	Factual presentation of clinical evidence for the unapproved product that is available at the time of communication No characterizations/ conclusions should be made regarding the safety or effectiveness of the unapproved product

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What economic content, i.e., product pricing information can be in the dossier?

Unapproved Product Dossier	Approved Product Dossier	Unapproved Use Dossier
Anticipated product price or reflected as a range The manufacturer has discretion on whether and how to provide economic information	Product price; health economics and outcomes research; economic models on budget impact and cost-effectiveness	Anticipated product price or reflected as a range The manufacturer has discretion on whether and how to provide economic information

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How are dossiers communicated?

Unapproved Product Dossier	Approved Product Dossier	Unapproved Use Dossier
Used per manufacturer's discretion to communicate information to HCDMs about an unapproved product The manufacturer may provide the dossier based on their discretion and internal policies and procedures	Used by the manufacturer to respond to unsolicited requests from HCDMs after FDA approval of the product (dossier contains on-label and any/all off-label information) Provided upon an unsolicited request only	Used per manufacturer's discretion to communicate information to HCDMs about an unapproved use The manufacturer may provide the dossier based on their discretion and internal policies and procedures

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Who from the manufacturer can communicate or provide the dossier?

Unapproved Product Dossier	Approved Product Dossier	Unapproved Use Dossier
The FEC strongly recommends personnel with appropriate medical/clinical/scientific credentials, expertise, and responsibilities	Personnel with appropriate medical/clinical/scientific credentials, expertise, and responsibilities	The FEC strongly recommends personnel with appropriate medical/clinical/scientific credentials, expertise, and responsibilities

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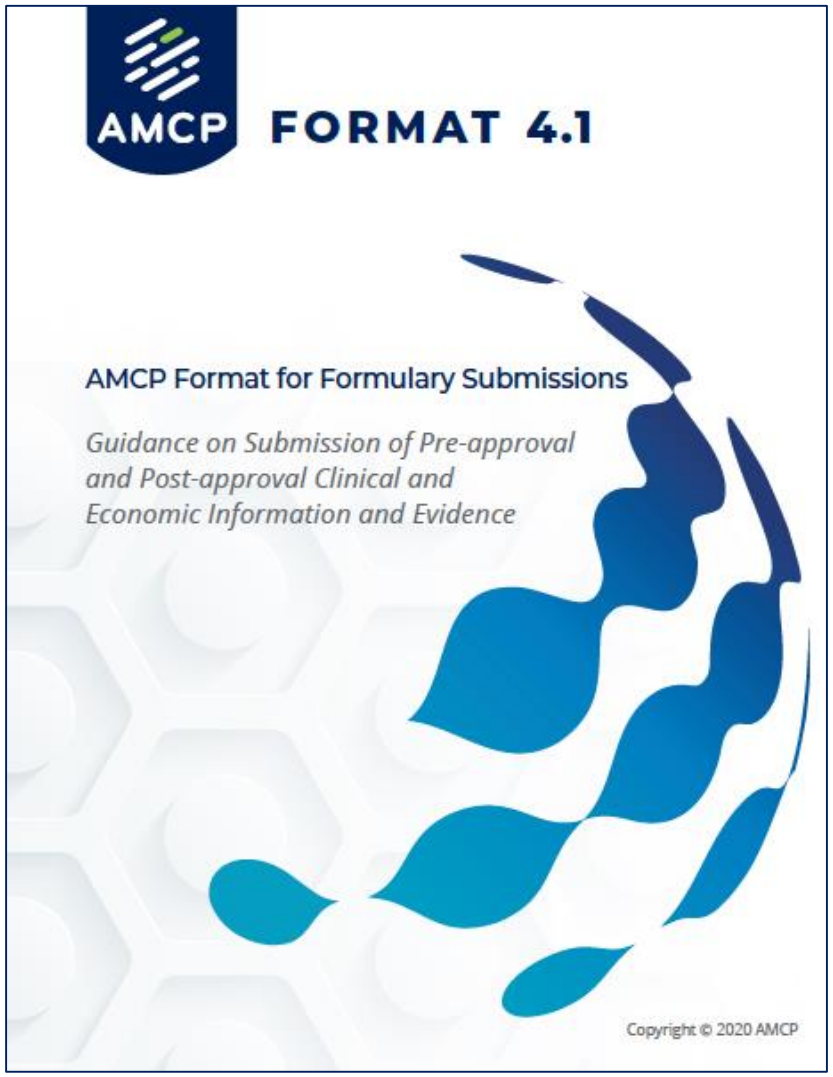

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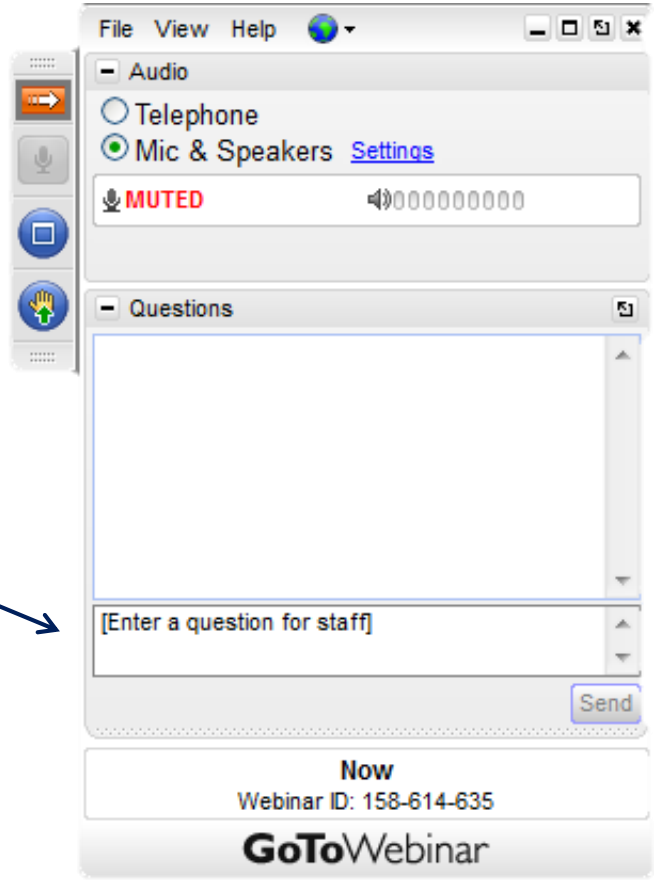

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