AMCP Webinar Series

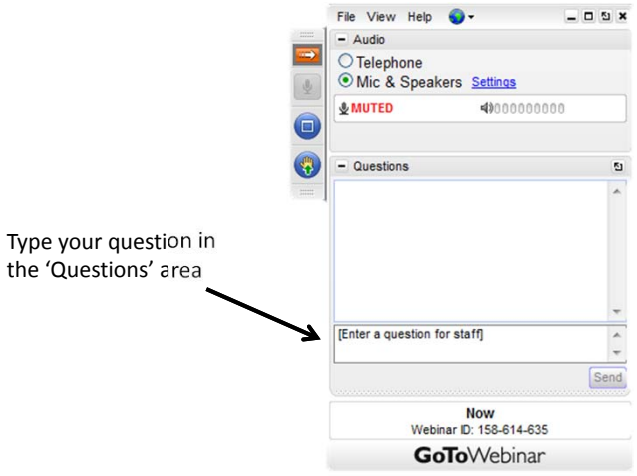
AMCP *Format for Formulary Submissions* Version 4.0

A Guided Tour of Key Changes and Enhancements

May 4, 2016

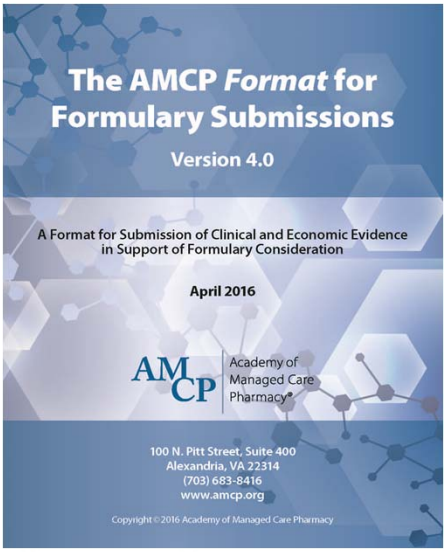
AMCP Academy of Managed Care Pharmacy®

How to Ask A Question



Type your question in the 'Questions' area

Format Version 4.0



Today's Speakers

Jeff Lee
Format Executive Committee Chair

Associate Professor of Pharmacy Practice
Lipscomb University College of Pharmacy

Iris Tam
General Information Work Group Chair

Vice President Patient Access and Quality, Medical Affairs,
Otonomy, Inc.

Pete Penna
Clinical Evidence Work Group Chair

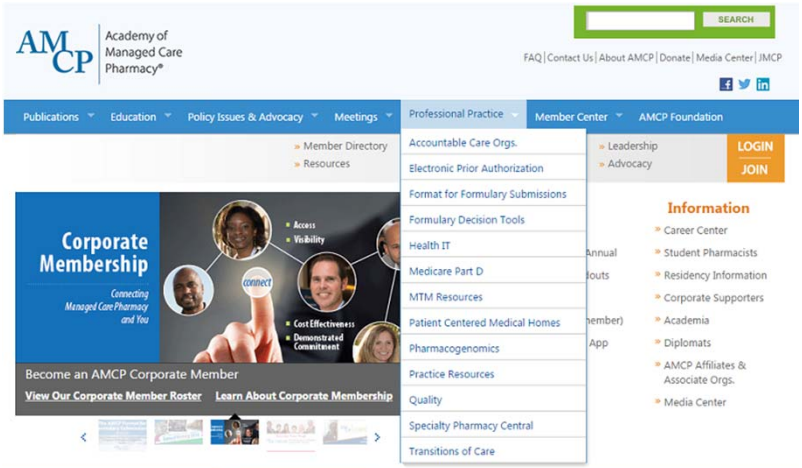
President
Formulary Resources, LLC

Kim Saverno
Economic Evidence Workgroup Chair

Research Lead Comprehensive Health Insights,
Humana, Inc.

Link to *Format* 4.0

www.amcp.org/FormatV4



Format Executive Committee

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- Diana Brixner
- Jeff Lee
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- Alan Pannier
- Pete Penna
- Elizabeth Sampsel
- Kim Saverno
- Helen Sherman
- Iris Tam
- John Watkins
- Jeff White
- Lynn Nishida (Board Liaison)
- Susan Oh (Staff Liaison)

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Rationale for V4.0
& Process Overview

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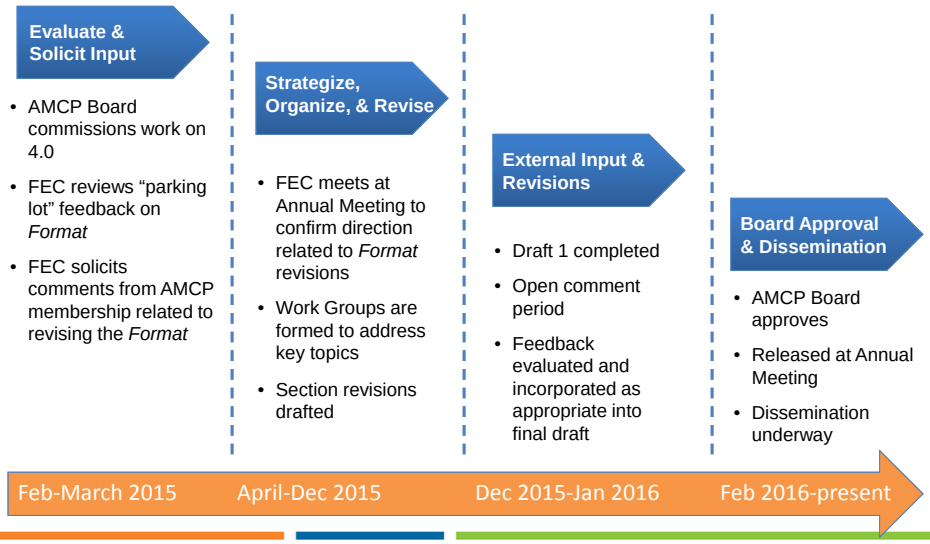
Why Version 4.0?

- Address contemporary issues in health care related to formulary management and evidence assessment
- Address feedback from users related to the *Format*
- Refresh *Format* alignment with external best practices in clinical and economic evidence development and communication
- Provide updated guidance to enhance the clarity, transparency, and usability of the *Format*

Process Overview



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Format Version 4.0

Key Changes
and Enhancements

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Table of Contents – Background Information

Version 4.0

- Preface
- General Logistical Considerations
  - Decision Makers & Manufacturers
  - Communications Between HCDMs and Mfrs
  - Confidentiality
  - Updating dossiers
  - Page Limits
  - Dossier Information Before FDA Approval
  - Media for Dossier and Model Submissions
  - Implementation of Version 4.0
- Special Content Considerations
  - Comparative Effectiveness Research (CER)
  - Dossier for Drugs, Tests, and Devices
  - Companion Diagnostic Tests (CDT)
  - Biosimilars
  - Heterogeneity of Treatment Effect

Version 3.1

- Preface
  - The Role of the AMCP *Format*
  - Confidentiality
  - Unsolicited Requests
  - Requests for and Submission of Updates to Existing Dossiers
  - Multiple Dossiers Generally Are Not Needed
  - Implementation Date for Version 3.1

General Logistical Considerations

- Decision Makers and Manufacturers
  - Health Care Decision Maker (HCDM) = any personnel, committee, or organization that make coverage decisions
  - Manufacturer = any drug, test, or device company
- Communications Between HCDMs and Manufacturers
  - FDA Guidance on unsolicited requests process
  - Ongoing communications throughout evaluation process
  - HCDMs should provide constructive feedback
- Confidentiality
  - Signed confidentiality agreements
  - HCDMs should maintain confidentiality of dossiers

General Logistical Considerations

- Updating Dossiers
  - When there are significant changes; evidence-based
    - New indication
    - FDA issued advisory statements
    - Significant new clinical or economic evidence
  - Re-write vs amendment
  - Multiple indications
  - Provision of updated dossiers
  - Reasons for not updating dossier
- Page Limits
  - Recommended lengths – be relevant, concise, clear
  - Do NOT be overly verbose just to meet page limits

General Logistical Considerations

- Dossier Information Before FDA Approval
  - Manufacturers should decide own policies; may include
    - Clinical trial information; pre-clinical studies
    - Clinicaltrials.gov
    - Data on file per manufacturer's discretion
    - Disease state information
    - Pipeline product information
    - Other per manufacturer's discretion
- Media for Dossier and Model Submissions
  - Electronic formats preferred
- Implementation of Version 4.0
  - For new creation or updates, implement V4.0
  - In midst of creation or update, manufacturer's discretion

Special Content Considerations

- Comparative Effectiveness Research (CER)
  - V3.1 CER Addendum incorporated
  - *Format* recommends CER but not specific research designs
  - *Format* suggests HCDMs use CER Collaborative Tool or ICER
  - Additional resources on page 14-15
- Dossier for Drugs, Tests, and Devices
  - *Format* aims to provide guidance to non-drug products
  - Companion Diagnostics Tests – new Section 2.3
  - Medical devices – related to the use of a drug

Special Content Considerations

- Companion Diagnostic Tests (CDT)
  - V3.1 CDT Addendum incorporated
  - vs diagnostic or prognostic tests which are about disease
  - Drug manufacture and CDT manufacture relationship
    - CDT is co-developed with drug (drug mfr)
    - CDT is developed independently of drug
      - CDT is required in the drug label (drug mfr)
      - CDT is not required in the drug label (CDT mfr)
    - CDT developed independently of drug for a class of drugs (CDT mfr)
  - Additional resources on page 16

Special Content Considerations

- Biosimilars
  - Biosimilar manufacturers should provide same evidentiary requirement as innovator product
  - Biosimilars are similar but not identical, thus, clearly state whether evidence from innovator or biosimilar (direct evidence or extrapolated evidence)
  - Additional resources on Page 18
- Heterogeneity of Treatment Effect
  - Patient variability in treatment response should be supported by evidence
  - Describe in Section 2.2; detail evidence in Section 3.0 or 5.0
  - Additional resources on page 19

Section 1.0 Executive Summary

- Clinical and economic value of the product
- Increase page limit to 5 pages (8 max)
- Comparative effectiveness relative to available alternative therapies added
- Include economic impact of special handling, deliver, route and site of administration, REMS

Section 2.0 Product Information & Disease Description

- 2.1 Product Description
  - Additional codes CPT and ICD-10 (ICD-9)
  - Contract price removed
  - Links to off-label sections & clinicaltrials.gov
  - Special populations added
  - Specialty: preparation, administration, devices
  - Access: specialty distribution, prescribing restriction, handling, ordering, patient assistance
  - Rationale and benefit of co-prescribed
  - How product may impact quality measures
  - Product comparison (2.1.1) emphasized, including biosimilars

Section 2.0 Product Information & Disease Description

- 2.2 Place of the Product in Therapy
  - 2.2.1 Disease description
    - Greater detail for rare diseases
  - 2.2.2 Approaches to treatment
    - Briefly include clinical practice guidelines (details in Sec 5)
    - Appropriate care setting - new
    - Heterogeneity of treatment effect - new
    - Ancillary disease or care management - expanded
    - Post-marketing obligation - expanded
    - Post-approval monitoring of safety - new

Section 2.0 Product Information & Disease Description

- 2.3 Evidence for CDT - new
  - 2.3.1 Product Information for CDT
    - Analytical validity, clinical validity, clinical utility, and economic value
  - 2.3.2 Place of CDT in Clinical Practice
    - For stand-alone CDT dossiers
  - 2.3.3 Supporting Clinical Data for CDT
    - For evidence that do not fit in Section 3.0
    - For stand-alone CDT dossiers, include clinical trials here

Section 3.0 Clinical Evidence

pp1

- Overall increased and improved guidance
- Focus on drugs, biosimliars, CDTs, CER, and devices
- Clinical evidence requirements for biosimilars, specialty pharmaceuticals, and CDTs defined/expanded
- Improved definition of and guidance for submitting "Supporting Clinical Evidence" and "All relevant clinical studies"
- Restructured Section 3 (Clinical Evidence) and Section 5 (Additional Supporting Evidence) to provide additional clarity and guidance regarding recommended evidence components for each section

Slide 22

pp1 Are these bullets appropriate and are there any addition or deletions to the list?
pete penna, 4/27/2016

Section 3.0 Clinical Evidence

Refined guidance on relevant studies for inclusion.

Prospective trials investigating any aspect of the drug regardless of study design:

- Randomized clinical trials, prospective observational trials, registries, & any others measuring clinical endpoint, measure patient outcomes, or collect data directly from patients
- Retrospective studies supporting use and value
- Studies that synthesize studies listed above, e.g. meta-analyses of the drug and/or primary comparators
- Studies of comparative effectiveness

Section 3.0 (studies to include - continued)

Other criteria for inclusion

- Studies supporting FDA approved uses and unapproved uses
- Published and unpublished studies and data (including abstracts, posters, presentations & submitted reports accepted for publication)
- Any study design
- Studies with positive, negative or null findings
- US and ex-US studies
- Studies and findings from the FDA and other federal agencies
- Ongoing trials

Section 3.0 Transparency

Include all studies – but if there are "too many" then segregate into 3 categories:

- Large key studies critical to the knowledge base of the drug – describe in text summaries and evidence tables
- Smaller (but informative) studies which add evidence but are not as rigorous – describe only in evidence tables
- All others should be listed only in a bibliography (define criteria for study placement)

Other: If results have been published/presented more than once, report all together in one summary

Section 3.0 Other

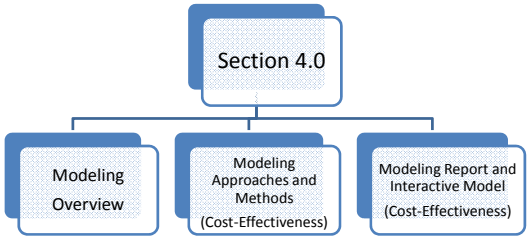
- If drug has more than one approved indication, manufacturer should decide if all are in one dossier, or in separate dossiers; if separate, each requires an unsolicited request.
- Other data elements: NNT, risk ratio, odds ratio, risk difference
- Text summaries should be 2 pages/study (max 5 pages)
- Evidence tables should be 1 page per study (max 2 pages)
- Evidentiary requirements for breakthrough drugs, biosimilars, & specialty drugs are the same as for "traditional" drugs

Section 5.0 Additional Supporting Evidence

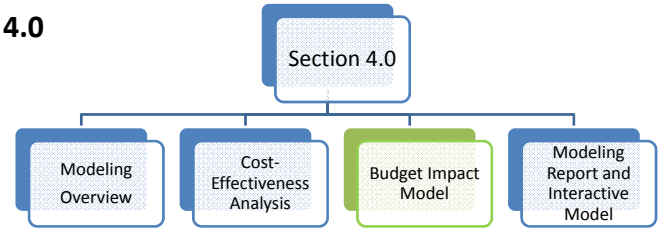
- Treatment guidelines moved from Section 2 to Section 5 with more detail to be provided
- Health Technology assessments and systematic reviews added
- Compendia (per HHS)
- Other economic & outcomes evidence not supplied elsewhere
- Impact on quality that does not fit elsewhere
- Other evidence that does not fit elsewhere, e.g., pharmacokinetic studies for biosimilars, data of effect on patient's families
- 2 pages per study/report (max 5 pages)

Section 4.0 Economic Value and Modeling Report

Version 3.1



Version 4.0



Section 4.1 Modeling Overview

- Types of Models
  - Verbiage added to specify model purpose
    - Cost-effectiveness models - Is the technology good value for the money?
    - Budget impact models - Is the technology affordable?
  - Financial models are not required, but may be included

Section 4.1 Modeling Overview

- Other considerations (new additions)
  - A clear, written statement of the decision problem, modeling objective, study perspective, and scope of the model should be developed. This should include: the spectrum of disease considered, target population, alternative interventions, health and other outcomes, and time horizon.
  - International Society for Pharmacoeconomics and Outcomes Research (ISPOR) and Society for Medical Decision Making (SMDM) have produced comprehensive guidance related to various aspects of modeling.[1] ISPOR-SMDM best practices should be followed when applicable.

1. Caro JJ, et al. *ViH*. 2012;15(6):796-803.

Section 4.1 Modeling Overview

- 4.3.1 Other considerations (new additions cont.)
  - Specialty pharmaceuticals should generally be addressed similarly to traditional pharmaceutical products. Additional considerations may be required for site of care (e.g. inpatient, home infusion, outpatient infusion center).
  - Due to similarity to their reference product, biosimilars generally do not require the development of specific cost-effectiveness models. Budget impact models or cost-minimization analyses may be more relevant.

Section 4.2 Cost-Effectiveness Analysis

- Numerous revisions made throughout section
- In general, the cost-effectiveness framework should consider recommendations published by ISPOR and SMDM Modeling Good Research Practices Task Force
- Time horizon
  - Long enough to reflect all important differences in costs and outcomes between technologies being compared
- Drug effectiveness
  - *Format* recommends that when available, RCTs should be considered basis of all efficacy/effectiveness estimates. When available, real world evidence should be assessed for relevance and validity and incorporated into the model when appropriate.

Section 4.2 Cost-Effectiveness Analysis

- Economic data
  - Because the costs of infused and injected drugs may also depend on site of care, models should take these attributes into consideration.
- Utilities
  - Preference estimates should be derived from studies surveying either patients or the general population, using a direct elicitation method or an instrument such as the time-trade off, standard gamble, EQ-5D, HUI, SF-6D, or QWB. Because cost-effectiveness analysis is conducted at the population level, the ideal source of utility values is the general population. This may be impractical in some situations and trial-derived utilities may be used.

Section 4.2 Cost-Effectiveness Analysis

- Sensitivity Analysis
  - Both univariate and probabilistic sensitivity analyses should be conducted.
  - Analysts should justify the distribution used for each parameter that is included in a probabilistic sensitivity analysis.
  - Comprehensive one-way sensitivity analysis of all parameters in the model is also strongly recommended.
  - Use of arbitrary lower and upper values is strongly discouraged.
  - Generation of cost-effectiveness scatter plots and acceptability curves are recommended to display the results of the analysis.

Section 4.3 Budget Impact Model

- Entirely new section
- The modeling approach and analytic framework of the budget impact model should generally follow the guidance provided by ISPOR.[1]
- The base case model (as presented in the written dossier) should be representative of the US population or a general commercial/Medicare population. However, the model should be sufficiently flexible to allow users to input data specific to their setting.
- Any expected off-label use of the new health technology should not be included in the main budget impact analysis, but may be considered in sensitivity analyses.

1. Sullivan SD, et al. *ViH.* 2014;17(1):5-14.

Section 4.4 Modeling Report & Interactive Model

- Offers more specific guidance for presenting the results of economic analyses, including both cost effectiveness and budget impact models, reflecting updated reporting standards for economic evaluations (Consolidated Health Economic Evaluation Reporting Standards (CHEERS) Statement).[1]
- Statement added encouraging manufacturers to publish economic models in the peer-reviewed literature and to update the model and publications with real-world evidence as available.

1. Husereau D, et al. *ViH.* 2013;16(2):231-250.

Section 6.0 Dossier Appendices

- References Contained in Dossiers
- Economic Model(s)
- Product Prescribing Information - new
- Patient Information - new
- Material Safety Data Sheet (MSDS) - new

Miscellaneous

- Three 3.1 Addenda incorporated into 4.0
- Terms and Definitions were updated
- Appendices that remain include
  - Sample Unsolicited Request Letter
  - Formulary Monograph Template
- Citations and resources throughout *Format* were updated with links where available
- Removed draft recommendation for manufacturers to rate quality of studies

Resources & Next Steps

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Link to *Format* 4.0

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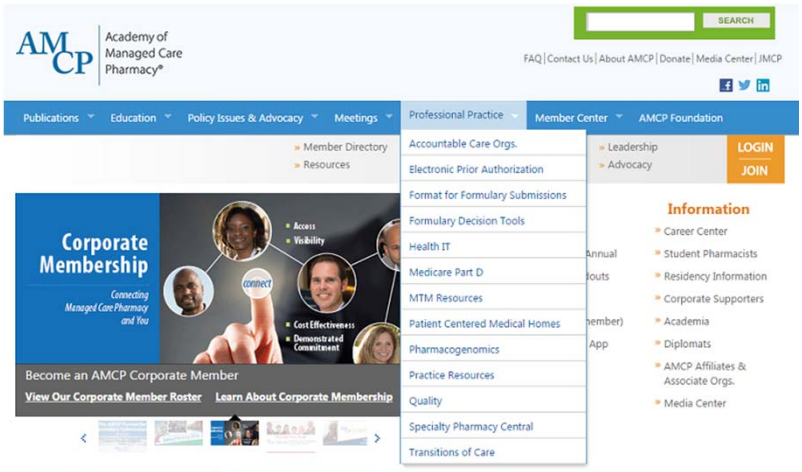

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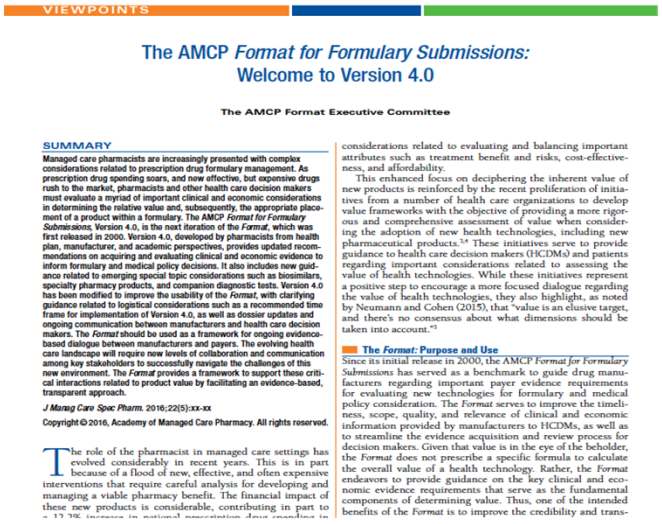


VIEWPOINTS

The AMCP *Format for Formulary Submissions:* Welcome to Version 4.0

The AMCP Format Executive Committee

SUMMARY

Managed care pharmacists are increasingly presented with complex considerations related to prescription drug formulary management. As prescription drug spending soars, and new effective, but expensive drugs rush to the market, pharmacists and other health care decision makers must evaluate a myriad of important clinical and economic considerations in determining the relative value and, subsequently, the appropriate placement of a product within a formulary. The AMCP *Format for Formulary Submissions*, Version 4.0, is the next iteration of the *Format*, which was first released in 2000. Version 4.0, developed by pharmacists from health plan, manufacturer, and academic perspectives, provides updated recommendations on acquiring and evaluating clinical and economic evidence to inform formulary and medical policy decisions. It also includes new guidance related to emerging special topic considerations such as biosimilars, specialty pharmacy products, and companion diagnostic tests. Version 4.0 has been modified to improve the usability of the *Format*, with clarifying guidance related to logistical considerations such as a recommended time frame for implementation of Version 4.0, as well as dossier updates and ongoing communication between manufacturers and health care decision makers. The *Format* should be used as a framework for ongoing evidence-based dialogue between manufacturers and payers. The evolving health care landscape will require new levels of collaboration and communication among key stakeholders to successfully navigate the challenges of this new environment. The *Format* provides a framework to support these critical interactions related to product value by facilitating an evidence-based, transparent approach.

J Manag Care Spec Pharm. 2016;22(5):xx-xx

The role of the pharmacist in managed care settings has evolved considerably in recent years. This is in part because of a flood of new, effective, and often expensive interventions that require careful analysis for developing and managing a viable pharmacy benefit. The financial impact of these new products is considerable, contributing in part to

considerations related to evaluating and balancing important attributes such as treatment benefit and risks, cost-effectiveness, and affordability.

This enhanced focus on deciphering the inherent value of new products is reinforced by the recent proliferation of initiatives from a number of health care organizations to develop value frameworks with the objective of providing a more rigorous and comprehensive assessment of value when considering the adoption of new health technologies, including new pharmaceutical products.[3,4] These initiatives serve to provide guidance to health care decision makers (HCDMs) and patients regarding important considerations related to assessing the value of health technologies. While these initiatives represent a positive step to encourage a more focused dialogue regarding the value of health technologies, they also highlight, as noted by Neumann and Cohen (2015), that "value is an elusive target, and there's no consensus about what dimensions should be taken into account."[5]

The *Format*: Purpose and Use

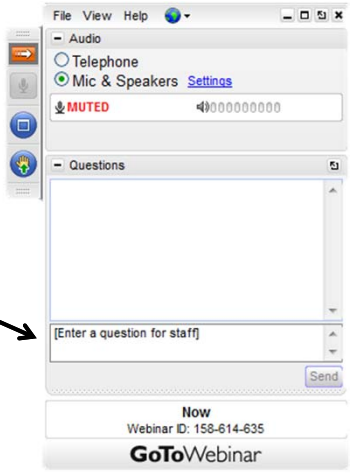
Since its initial release in 2000, the AMCP *Format for Formulary Submissions* has served as a benchmark to guide drug manufacturers regarding important payer evidence requirements for evaluating new technologies for formulary and medical policy consideration. The *Format* serves to improve the timeliness, scope, quality, and relevance of clinical and economic information provided by manufacturers to HCDMs, as well as to streamline the evidence acquisition and review process for decision makers. Given that value is in the eye of the beholder, the *Format* does not prescribe a specific formula to calculate the overall value of a health technology. Rather, the *Format* endeavors to provide guidance on the key clinical and economic evidence requirements that serve as the fundamental components of determining value. Thus, one of the intended benefits of the *Format* is to improve the credibility and trans-

Next Steps/Resources

- FEC is currently evaluating various dissemination initiatives as it relates to Version 4.0
- Additional training resources are in development
- Feedback on 4.0 is welcomed
  - Jeff Lee: jeff.lee@lipscomb.edu
  - Susan Oh: soh@amcp.org

How to Ask A Question

Type your question in the 'Questions' area



THANK YOU!

upcoming webinars

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