Updating the AMCP *Format for Formulary Submissions*

Summary of Revisions to Version 3.1

December 11, 2012

Background

Since the release of Version 1.0 of the AMCP *Format*, FMCP, AMCP and the *Format* Executive Committee (FEC) have continued to collect constructive comments on the use of the *Format* from numerous colleagues in the clinical pharmacy and health economics and outcomes research fields. Since the release of Version 3.0 in 2009, numerous inquiries have been received regarding the development and use of AMCP *Format*-based dossiers for specialty pharmaceuticals and companion diagnostic tests and the place of comparative effectiveness research within the context of the *Format*.

In order to maintain the *Format* as a "living document" that continues to track with the current trends in technology assessment for coverage and reimbursement, the AMCP *Format* Executive Committee made the decision to provide further guidance in the aforementioned three areas, which would be provided as addenda to the *Format*, rather than do a full revision.. A call for comments was issued in April 2012. The notice went out to AMCP members and other creators, users and potential users of AMCP *Format*-based dossiers in health care systems, hospitals and other health care entities, and the pharmaceutical industry.

The FEC also decided to designate this iteration as Version 3.1 to draw attention to the new additions. Precedent was set in 2005 with the release of Version 2.1 which was not a wholesale rewrite. Like this version (V3.1), Version 2.1 was a response to numerous questions and comments, which required that only certain sections be streamlined, a section added to describe evidence requirements for biologics, and additional language inserted that encouraged clarity and transparency in the presentation of economic models and differentiated requirements for budget impact vs. cost-effectiveness models.

Addenda Development Process: The FEC established 3 subcommittees:

- Specialty: Jeff Lee, Allergan (Chair)
- Companion Diagnostics: Dave Veenstra, U Washington (Chair)
- Comparative Effectiveness Research: Bryan Luce, United BioSource (Chair)
- Staff support: Marissa Schlaifer, Liz Sampsel, and Welton O'Neal
- Final editing/consolidation: Richard Fry

Each subcommittee was subdivided into a writing team and a review team with expert volunteer team members added from academia, managed care, and pharmaceutical industry. (See Acknowledgements in Version 3.1). The subcommittees vetted all comments, and consequently developed the attached addenda following a second round of input on the draft documents. The FEC approved the addenda, which were then incorporated into the body of the current version of the *Format*. Notices and References to the addenda were added in the Preface and each section of the Evidentiary Requirements. Definitions were added to the Terms and Definitions section, writing and review team members were added to the Acknowledgements page, and references to the December 2011 FDA draft guidance on unsolicited requests were added to the Preface and Appendix C.

Specialty Pharmaceuticals (SP): Addresses application of the *Format* to SP; where and how special considerations are needed.

SP Addendum Areas of focus:

- Definition of SP ("delivery" process & management)
- Specialty pharmacies
- Application of the *Format* to SP
- Guidance on evidence to be provided (and evaluated)
    - Emphasis on specific sections of the *Format*

SP Evidentiary Requirements

- NDC as well as HCPCS and CPT codes
- Special dosing, administration, delivery devices (not in PI)
- Access issues
- Comparator issues for unique drugs and rare conditions
- Ancillary disease/care management concerns
- Modeling issues

Companion Diagnostic Tests (CDT): Provides guidance to drug and CDT manufacturers, P&T committees, and Medical Technology Review committees about clinical and economic evidence for CDTs under consideration for coverage and reimbursement.

CDT Addendum Areas of Focus

- Definition of terms and description of types of CDTs including approval procedures
- CDTs are tests that provide information to improve the safety and efficacy of a drug:
  - ID patients most likely to benefit from the drug
  - ID patients likely to be at increased risk from the drug
  - Monitor patient response
- Regulatory approval is very different from drugs
  - Clear evidence of clinical utility may be lacking
- Evidence gathering frameworks for evaluation of CDTs
- A CDT dossier should contain supporting clinical & economic evidence for tests detailed in *Format* Section 2.3, Evidence for Pharmacogenomic Tests and Drugs, and most of the data and evidence listed for a drug dossier.

Comparative Effectiveness Research (CER): Provides an overview of the types of CER evidence and the role it plays in the *Format.*

- Not intended to be a comprehensive review of the complexities of CER or the methodologies of CER
- Reinforces the *Format* as safe harbor for manufacturers to submit all available evidence (including CER evidence) in support of formulary evaluation.

CER Addendum Areas of Focus

- Adopts Institute of Medicine definition of CER
- Brief review of CER references in *Format* Version 3.0 (sections 3 and 4, and Appendix F)
- Overview of major types of CER study design
  - Bayesian and adaptive trials
  - Pragmatic clinical trials
  - Prospective observational studies
  - Retrospective observational studies
  - Systematic evidence review
  - Modeling studies
- Comparison of CER designs with traditional RCTs
- Compares and contrasts CER to health technology assessment and EBM