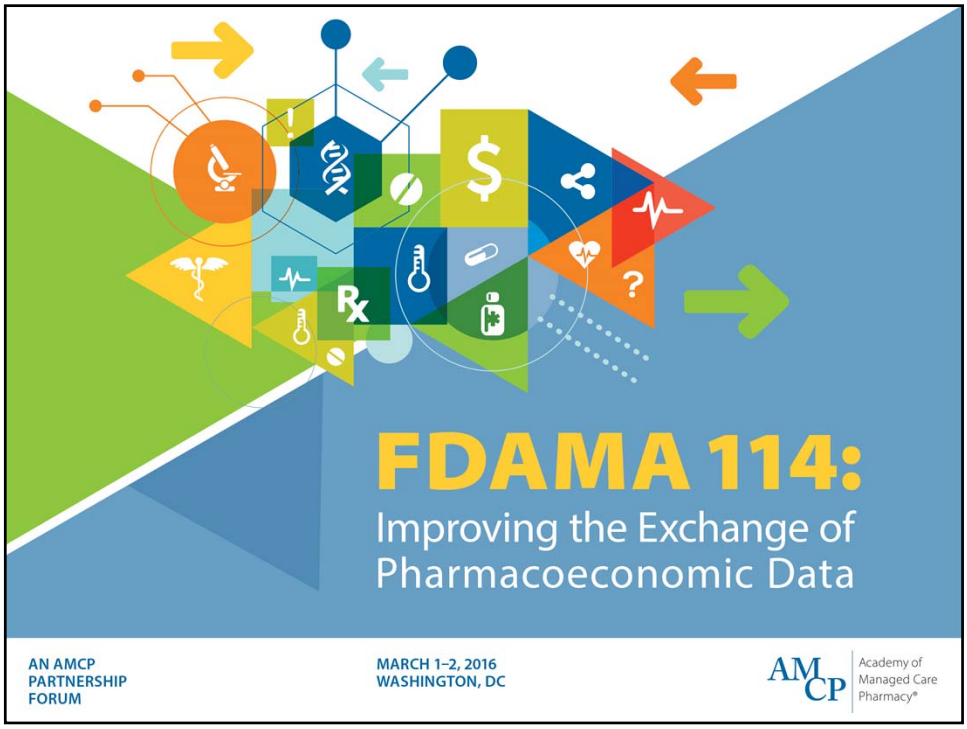
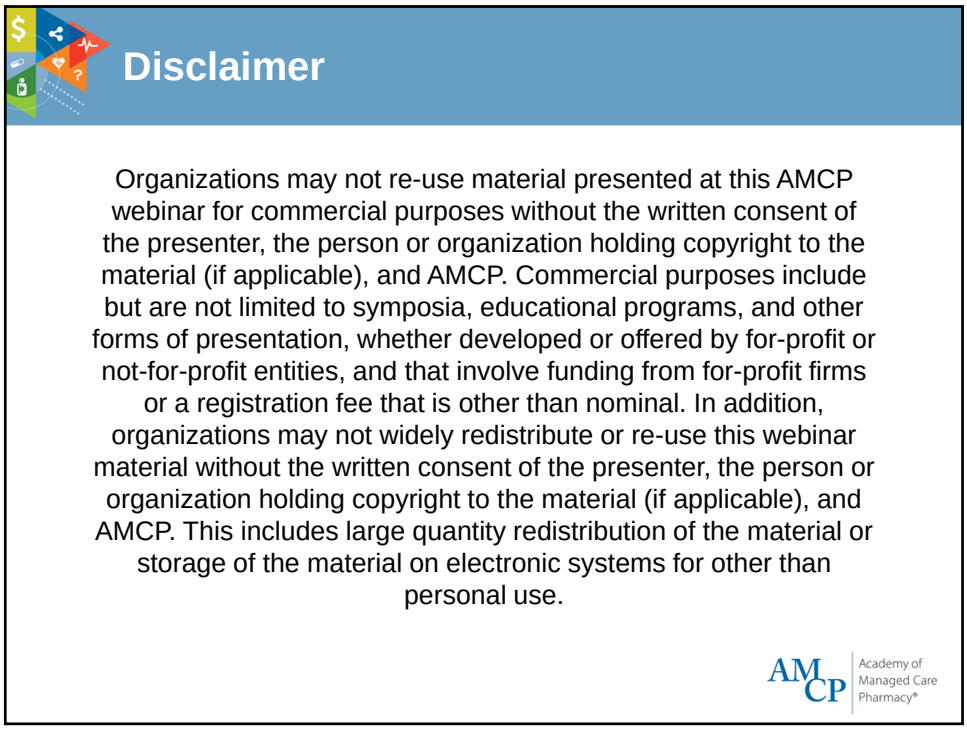

FDAMA 114:
Improving the Exchange of Pharmacoeconomic Data
AN AMCP PARTNERSHIP FORUM
MARCH 1–2, 2016
WASHINGTON, DC
AMCP
Academy of Managed Care Pharmacy®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Agenda

FDAMA 114: Past, Present, & Future

Consensus Recommendations & Rationale

Advocacy & Adoption Efforts

Next Steps

Question & Answer Session

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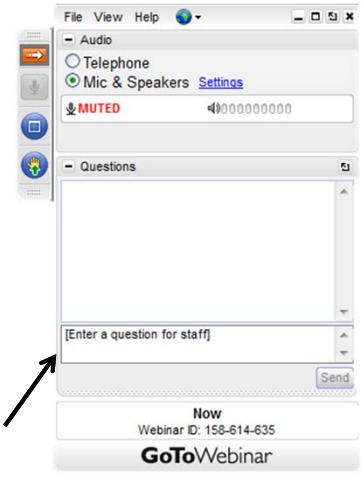
How to Ask a Question



Type your question in the "Questions" area

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Today's Speakers

Jay Jackson
SVP, Scientific Consulting
Xcenda

Soumi Saha
Assistant Director,
Pharmacy & Regulatory Affairs
AMCP

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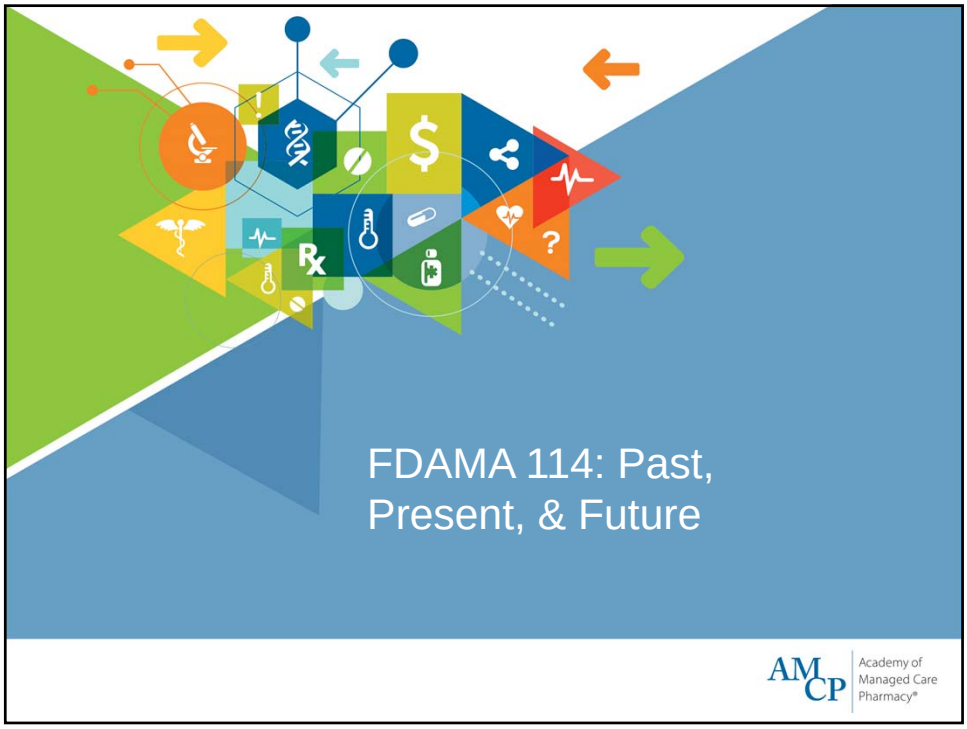

What is FDAMA 114?

- FDAMA 114 is an acronym that stands for the Food and Drug Administration Modernization Act of 1997 Section 114
- FDAMA 114 established an evidentiary standard and safe harbor for the promotional dissemination of health care economic information (HCEI) by biopharmaceutical companies to formulary decision makers
- Proactive distribution of HCEI has been underutilized by biopharmaceutical companies partly because of:
  - Vague wording in the statute
  - Absence of FDA-implementing regulations

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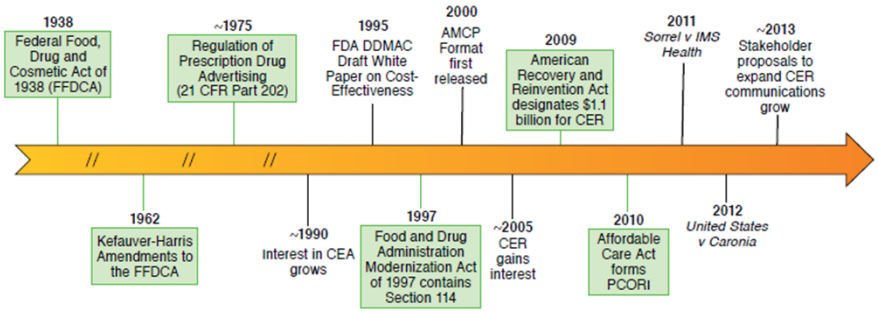
Evolution of Drug Effectiveness Evidence and Communication Standards



Boxed events denote governmental action.
Legend: AMCP=Academy of Managed Care Pharmacy; CEA=cost-effectiveness analysis; CER=comparative effectiveness research ; CFR= Code of Federal Regulations ; DDMAC=Division of Drug Marketing, Advertising, and Communications; FDA= U.S. Food and Drug Administration; FFDCA=Federal Food, Drug, and Cosmetic Act; PCORI=Patient-Centered Outcomes Research Institute .

Perfetto et al. FDAMA Section 114: Why the Renewed Interest? *JMCP.* May 2015; Vol 21, No. 5.

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What Does Section 114 Say and Not Say?

Term	Definition	Caveats
Health care economic information	"Any analysis that identifies, measures, or compares the economic consequences, including the costs of the represented health outcomes, of the use of a drug to the use of another drug, to another health care intervention, or to no intervention"	Appears to exclude comparisons based solely on effectiveness or efficacy
Formulary committee or other similar entity	"Committee or the entity carrying out its responsibilities for the selection of drugs for managed care or other similar organizations"	Appears to exclude communications with organizations or individuals making population health decisions
Competent and reliable scientific evidence	"Tests, analysis, research, studies or other evidence based on the expertise of professionals in the relevant area…conducted and evaluated in an objective manner by persons qualified to do so, using procedures generally accepted by others in the profession to yield accurate and reliable results"	Appears to require substantiation but does not necessarily require clinical trial data
Directly relates to an approved indication	Refers to an FDA-approved use for the product	Appears to forbid any extension beyond an FDA-approved, labeled use

Perfetto et al. FDAMA Section 114: Why the Renewed Interest? *JMCP.* May 2015; Vol 21, No. 5.

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Why the Limited Use in Promotion?

- The language is unclear and lacks detail
- The FDA has not updated the 1997 language or provided guidance
- The FDA has **never** applied the "competent and reliable scientific evidence" standard in a regulatory letter
- Promotional materials pursuant to Section 114, by definition, are not in the public domain
- Viewed as overly restrictive
- Extensive use of AMCP dossiers

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Why the Renewed Interest?

- Comparative effectiveness research
- More "big data," so more observational studies
- Commercial-free speech
  - United States vs. Caronia
  - Sorrell vs. IMS Health
  - Amarin Pharma, Inc. vs. FDA

Perfetto et al. FDAMA Section 114: Why the Renewed Interest? *JMCP.* May 2015; Vol 21, No. 5.

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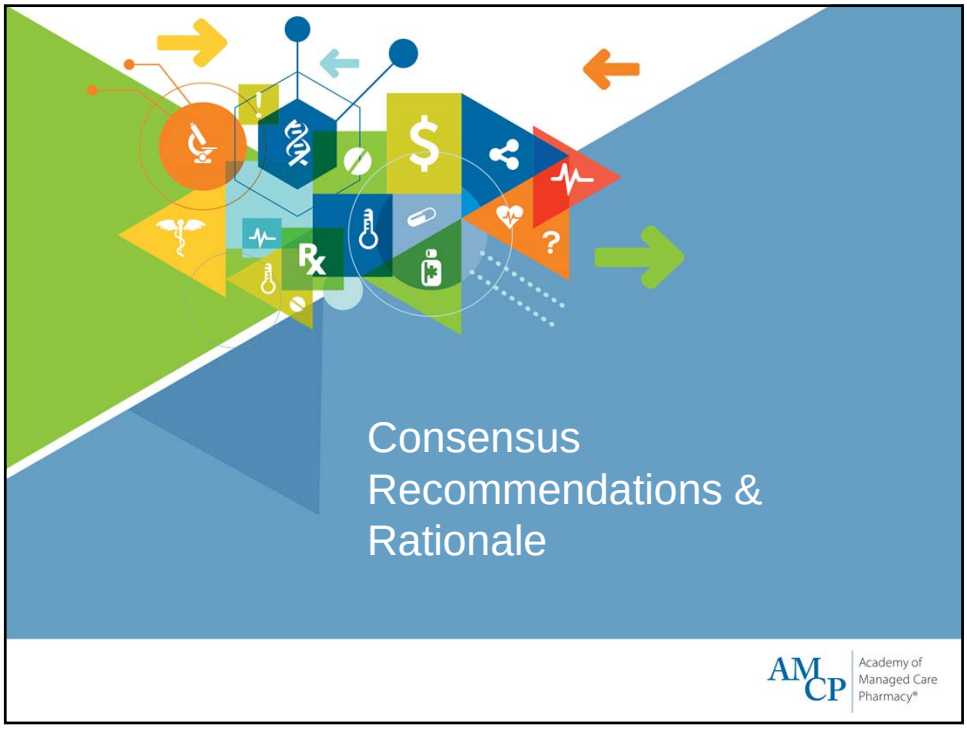
Consensus
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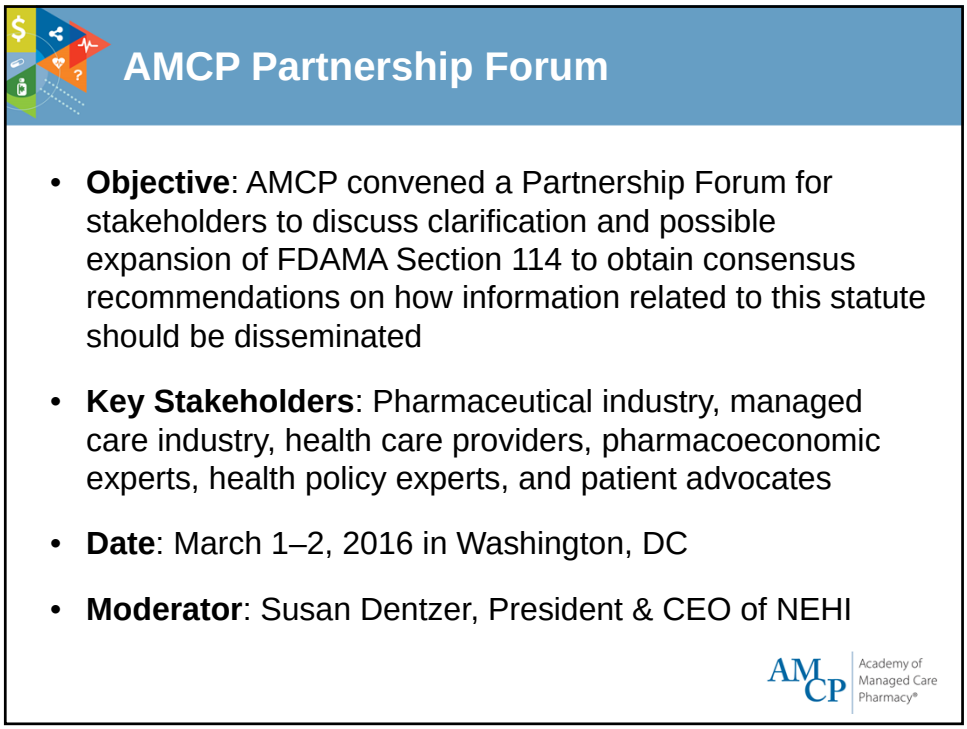
AMCP Partnership Forum
• Objective: AMCP convened a Partnership Forum for stakeholders to discuss clarification and possible expansion of FDAMA Section 114 to obtain consensus recommendations on how information related to this statute should be disseminated
• Key Stakeholders: Pharmaceutical industry, managed care industry, health care providers, pharmacoeconomic experts, health policy experts, and patient advocates
• Date: March 1–2, 2016 in Washington, DC
• Moderator: Susan Dentzer, President & CEO of NEHI
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Purpose of the Forum

- Provide recommendations to the FDA for the promulgation of regulations or guidance to provide clarification and consistency of FDAMA 114 requirements
  - Create definitions for the following terms referenced in FDAMA 114 to clarify what is considered relevant HCEI:
    - Competent and reliable scientific evidence (CRSE)
    - Formulary committee or other similar entity
    - HCEI
    - Directly relates to an approved indication
  - Articulate the type of information, format, and process by which health care decision makers would like to receive HCEI from biopharmaceutical companies

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Purpose of the Forum

- Consider whether FDAMA 114, or other areas of existing laws and regulations, should be expanded to provide HCEI to additional entities and articulate the value that would be gained. Audiences for consideration include:
  - Payers
  - Health care providers
  - ACOs
  - IDNs
  - Patient advocacy groups
  - Organizations that develop value frameworks
  - Research societies

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Approach

- To accomplish the goals of the Forum, the discussion was divided into two sections:
  - Recommendations to the FDA for clarification of FDAMA 114 under the existent statutory language
  - Recommendations to Congress to amend, provide clarification, and/or incorporate possible expansion of FDAMA 114 or other areas of existing laws

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Recommendations to the FDA

"Competent and Reliable Scientific Evidence"

"truthful and non-misleading tests, analyses, research, studies, models, or other evidence. Such evidence would be based on the expertise of professionals in the relevant area and be derived using methods that are transparent, disclosed, reproducible, accurate, and valid."

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Recommendations to the FDA

"Formulary or Other Similar Entity"

"health care decision makers beyond health plan formulary committees, including organizations, or individuals in their role in an organization, who make health care decisions for patient populations and organizations that evaluate HCEI or develop value frameworks and compendia, including individuals in such organizations."

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Recommendations to the FDA

"Health Care Economic Information"

"any analysis that identifies, measures, or compares the economic, clinical, or quality of life consequences for any treatment. This includes the costs and resource utilization of a drug or health technology relative to another drug, health technology, or no intervention."

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Recommendations to the FDA

"Directly Relates to an Approved Indication"

"information about a product that may vary from the parameters utilized in a randomized control trial, such as dosage forms, settings, or populations studied, as long as it is still used within the approved disease indication."

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Recommendations to the FDA

- Format
  - Fully disclosed
  - Leave-behind models
  - Adaptation of AMCP dossiers one possible option
- Process
  - Objective independent body to develop "good research practice" guidelines for CRSE
  - Central repository
  - Alert system

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Recommendations to Congress

- Inclusion of all definitions, format, and process previously discussed
- Change “directly relates” to “relates”
- Disclosures of transparency

Recommendations for Future Discussions

- Two additional areas, considered outside the scope and original intent of FDAMA 114, were identified as warranting further discussion:
  - Dissemination of HCEI to payers and other entities 12–18 months prior to FDA approval during the forecasting process
  - Dissemination of HCEI to patients to be an advocate for their own health care decisions

Advocacy & Adoption Efforts

- Significant interest on Capitol Hill and among stakeholders, driven in part by attention on drug pricing
  - Congressional committees with purview over the FDA
  - Associations representing Forum participants

Advocacy & Adoption Efforts

- Congress is already considering changes in this area
  - House-passed 21st Century Cures bill includes provisions that would:
    - Clarify provisions on dissemination of pharmacoeconomic data
    - Direct the FDA to issue guidance on off-label communication
  - Senate yet to act on similar legislation

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Advocacy & Adoption Efforts

- Potential vehicles for change
  - FDA guidance
  - Legislation
    - 2016 – 21st Century Cures
    - 2016 – Innovations
    - 2017 – PDUFA reauthorization

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Advocacy & Adoption Efforts

- Upcoming advocacy opportunities for AMCP
  - Brief FDA leadership on Forum proceedings
  - Continue discussions with Capitol Hill and stakeholder organizations

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Next Steps

- **Education Session** at AMCP Nexus Meeting
  - Tuesday, October 4th 9:35 am to 11:05 am
  - Speakers:
    - Jeffrey K. Francer, JD, MPP
      Vice President and Senior Counsel
      Pharmaceutical Research and Manufacturers of America
    - Daniel C. Malone, RPh, PhD, FAMCP
      Professor, University of Arizona
      Immediate Past President, ISPOR
  - Registration is available at: http://www.amcpmeetings.org/

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Next Steps

September Partnership Forum

- **Title**: "Enabling the Exchange of Clinical and Economic Data Pre-FDA Approval"
- **Dates**: September 13 – 14, 2016
- **Location**: Tysons Corner, VA
- **Goals:**
  - Provide recommendations to Congress and the FDA as to how current laws or regulations should be amended to allow biopharmaceutical manufacturers to provide clinical and economic information related to drugs and health care technologies to payers and other entities at least 12–18 months prior to FDA approval
  - Consider necessary public health protections to prevent dissemination of pre-FDA approval clinical and economic information to unintended entities

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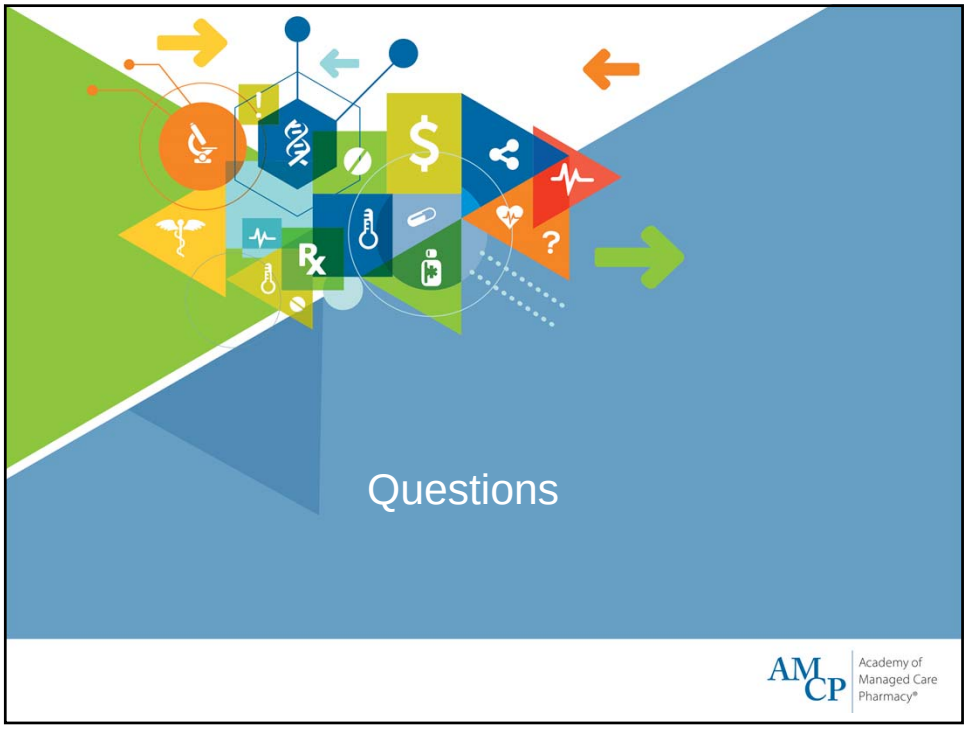

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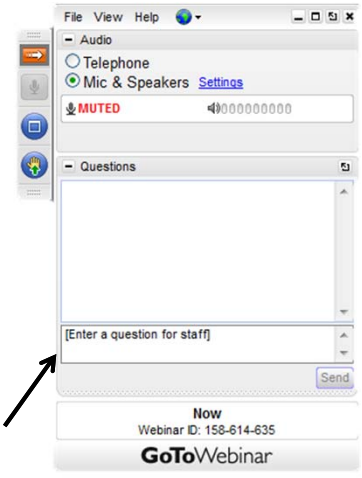

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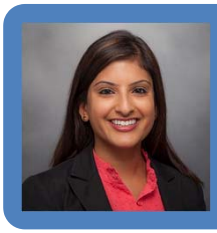

Soumi Saha, PharmD, JD
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Thank you for attending!

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