AMCP Partnership Forum

Building the Foundation for Patient-Reported Outcomes: Infrastructure and Methodologies

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Welcome

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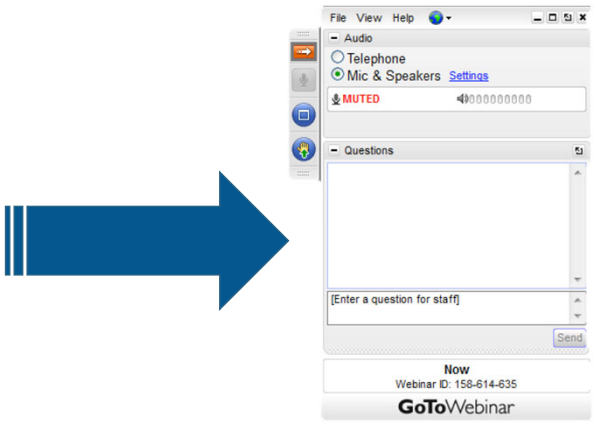
How to Ask Questions



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AMCP Partnership Forums
Collaboration for Optimization

The live, hands-on AMCP Partnership Forums bring key decision-makers in managed care, integrated care, the pharmaceutical industry, and others together to discuss and collaborate on tactics and strategies to drive efficiencies and outcomes in integrated care and managed care.

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Partnership Forums…

- **Proactive, collaborative approach** to solving important issues and challenges
- **Provide a voice** to all stakeholders
- **Gain consensus** on tactics to address a key challenge or opportunity, as well as how to **remove barriers** to improve patient care and optimize expenses
- Represent opportunities for payers, manufacturers, and other stakeholders to **work together on common goals and interests**
- **Have high visibility** among industry stakeholders and policy-makers
- Bring individuals and organizations with different priorities together to **find common ground and actionable results**

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LEADING CHANGE

On health care issues that are important to you

Live and hands-on, AMCP Partnership Forums, bring together key decision-makers in managed care, integrated care, the pharmaceutical industry, and others to drive efficiencies and outcomes in managed care.

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2019 Partnership Forums

2019 Forum Topics:

1. Pharmacy and Therapeutics (P&T) Practices: What's Next?
2. Optimizing Prior Authorization for Appropriate Medication Selection.
3. Digital Therapeutics: What are they and Where do they Fit in Pharmacy and Medical Benefits?
4. What's Next for Specialty Medication Benefit Design and Reimbursement?

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Our Faculty

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Agenda

- Patient-Reported Outcomes
- Forum Findings and Recommendations
- Q&A
- Next Steps and Action Items

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Patient-Reported Outcomes (PROs)

- PROs are clinical outcome assessments based on information collected directly from the patient.
- Include a wide variety of self-reported measures:
  - Individual health status, ability to conduct activities of daily living, pain intensity, perception of health/treatment, etc.
- Using PROs is an important component of the shift toward patient-centered health care.
- PROs are increasingly being used as:
  - Assessments of value and cost effectiveness of clinical interventions.
  - Endpoints that are included in product labeling.

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2018 Forum Goals

- Describing the current state of using PROs for FDA regulatory and value-based coverage decisions.
- Define a process for collecting and sharing PROs with the FDA and managed care organizations for value-based decisions.
- Identifying the necessary infrastructure needed to support the ideal use of PROs.

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Federal Legal and Regulatory Developments

2009

In 2009, the U.S. Food and Drug Administration (FDA) guidance on inclusion of the patient experience in the drug development process.

In 2016, Section 3002 of the 21st Century Cures Act Directed the FDA to issue guidance regarding methods and approaches to be used in capturing and measuring patients' experiences and perspectives.

2016

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PRO Use in Drug Development

- Current PROs in labels generally are for outcomes that can only be assessed by the patient (e.g., pain).
- Opportunities exist to expand the use of PROs.
- Manufacturers are:
  - Focusing more on the patient experience throughout the drug development cycle
  - Engaging with patients and viewing them as the experts in the experience of their disease
  - Utilizing advisory boards to ensure that the patient perspective is addressed during clinical trial design

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Challenges and Initiatives for Creating Useful Measures

- Need a consistent and defined methodology for developing and implementing PROs
- Measures must be meaningful to patients and reflect what they believe about their health
- Initiatives are underway to develop core outcome sets for drug development and routine practice use
  - Databanks for PROs
    - E.g., Patient-Reported Outcomes Measurement Information System (PROMIS)

https://grants.nih.gov/grants/guide/notice-files/NOT-FD-18-014.html.
https://commonfund.nih.gov/promis/index.

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Recommendations for Collecting PROs for Drug Development

Interview	Formalize	Develop
Interview relevant stakeholders (e.g., patient advocacy groups) to identify which outcomes are most important and meaningful to patients	Formalize PROs in the drug development process and ensure that these measures are systematically incorporated and addressed	Develop a central repository of available tools for assessing PROs as a resource for manufacturers

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PRO Use in Value-Based Contracts

- Need to assess the validity and appropriateness of a PRO for a VBC
- Real-world populations differ from clinical trial populations and therefore, PRO measures used in labeling may not be ideally-suited for VBCs
- Need to determine the predictive validity of a PRO as a measure of a meaningful outcome
  - Important for determining how performance on PRO metrics will impact payments

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Challenges Using PROs for Value-Based Contracts

Standardized, generally-accepted metrics are lacking for many PROs.

Difficult to evaluate performance on PROs across contracts.

Diversity of measures complicates operations for health care providers.

Broad, more widely-used measures of quality-of-life (e.g., the SF-36) many not be sensitive enough to detect meaningful changes .

Social determinants of health can dramatically impact measures of health and well-being.

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Recommendations for Using PROs for Value-Based Contracts

Support Services to Improve Patient Experiences	Account for Social Determinants
Identifying barriers to care and implementing appropriate supports or connecting patients with community resources	Structure Contracts to account for the influence of social determinants of health

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Collecting and Reporting PRO Data

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Drug Development Collection Strategies

Development	Identify stakeholders—patients, relatives, caregivers, clinicians, payers, and researchers.
	Select/develop measures for outcomes of relevance.
	Qualitative interviews with patients regarding signs and symptoms, quality of life, function, and preferences.
Infrastructure	Determine where to collect the data (provider office, EHR survey, personal devices, community pharmacies) and aim to cause the least disruption to patients.
	Determine who will record the data (patient, physician, caregiver, pharmacist, nurse, care manager) and consider reporter bias.
	Determine how often to collect data (consider disease state, patient needs, length of survey).
Implementation and Use	Implement PROs in trials in a patient-friendly manner.
	Address user interface (dashboard reports, gamification, adaptive designs for patients with disabilities).
	Educate patients on the use of PRO data at the beginning of a trial so they understand the importance of reporting.
	Determine who is responsible for collection of data (are there incentives aligned with data collection?).
	Provide feedback loops between patients and clinicians that present data in an actionable format.
	Provide incentives for sustained patient engagement.

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Drug Development Reporting Strategies

Development	Create consensus-based standardized reporting standards that are measurable, reproducible, and meaningful.
	Validate the financial value of PROs.
	Consider how to validate data.
Infrastructure	Consider the use of registries.
	Develop partnerships with industry and the FDA to address data sharing across systems
	Develop infrastructure for monitoring and interpreting data
Implementation and Use	Consider who will own the data.
	Collaborations to build models to define and validate the value of PROs for contracts
	Promote clear communication and transparency for reporting strategies for PROs.
	Protect patient health information through security, aggregation and deidentification of data.
	Consider opt out and opt in strategies for data sharing.

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Strategies to Support PRO Use by Managed Care Stakeholders

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Strategies to Support Patients and Caregivers

Engage	Engage patients and caregivers in discussions on how to collect data
Allow	Allow flexibility for data collection methods based on patient needs and preferences (e.g., some can be assessed by providers, others can input data in smartphone apps)
Use	Use adaptive designs that address certain disabilities, functional limitations, language barriers, and health literacy
Tailor	Tailor frequency of data collection based on actual needs and collection method (e.g., length of the survey)
Provide	Provide incentives to support sustained adherence to data collection
Address	Address privacy concerns and be transparent regarding how data will be used and shared

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Strategies to Support Health Care Providers

- Structure workflows to allow data collection and clinically relevant time points
- Adapt EHR systems to facilitate PRO data collection and reporting in real-time to inform clinical decision making
- Inform clinicians how to use PRO data in a manner that improves patient care
- Implement incentives that support clinician use of PRO data
- Explore roles for PRO collection and analysis during medication therapy management visits conducted by pharmacists

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Strategies to Support Managed Care Organizations

Engage	Design and implement	Align
Engage patients and providers through education on the importance of PRO data.	Design and implement systems and processes that facilitate collection of PRO data.	Align incentives to support PRO collection.

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Strategies to Support Life Sciences Companies and Researchers

Implement	Collaborate	Work
Implement PROs throughout the drug development process	Collaborate with FDA's Patient Engagement Collaborative	Work with third party entities to develop value assessments that include PROs

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Summary—Drug Development

- PROs are increasingly being valued during drug development processes
  - Manufacturers are interested in having PROs incorporated in clinical trial design to help support drug approval and meaningful comparisons across drugs and as a basis for FDA approval
- PRO measure development must balance the need for simplicity and standardization with the need to have sensitive and clinically relevant measures
- PROs should be designed to detect changes that provide value that is meaningful to patients

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Summary—Post-Approval Environment

- PRO standardization can help create a framework to inform best practices around care delivery and coverage determinations
- Improved HIT infrastructure, including interoperability of EHR systems, will be necessary to support effective utilization of PROs across the health care system
- Cross-industry partnerships and public-private partnerships to are needed to facilitate PRO development and implementation

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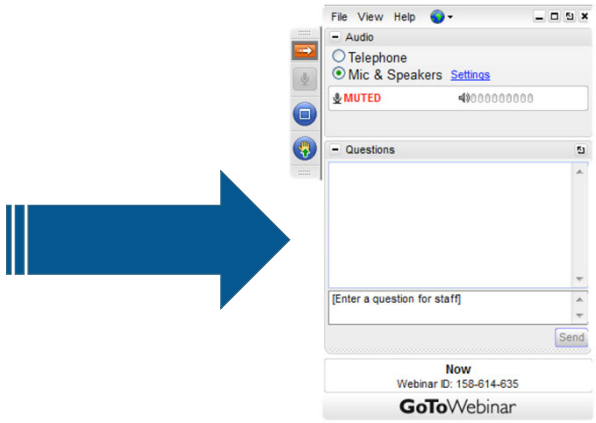
Reminder: How to Ask Questions During the Webinar



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Additional Information Next Steps

Forum Proceedings are Available: *Journal of Managed Care & Specialty Pharmacy* 2019;25(4):501-506

Upcoming Partnership Forum Webinars

- (July 10, 2019) Integrated Delivery Networks' Role in Value-Based Agreements for Pharmacy and Medical Benefits

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