AMCP

PARTNERSHIP FORUM

No. 2 — 2020

Preparing for and Managing Rare Diseases

SEPTEMBER 8-10, 2020

Moderator Welcome

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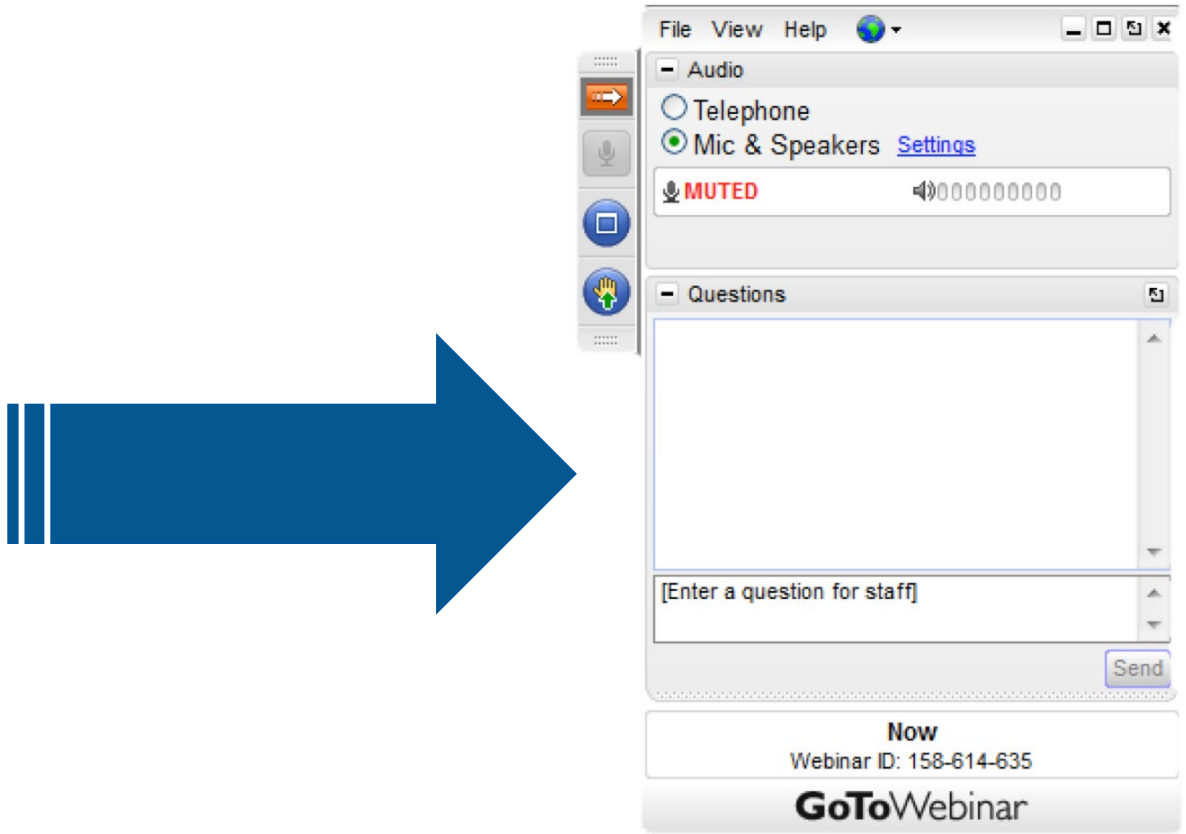
AMCP

How to Ask Questions



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.

Partnership Forums

2020

1. Helping Patients Anticipate and Manage Drug Costs
2. Preparing for and Managing Rare Diseases
3. Biosimilars: Policy, Practice, and Post Marketing Surveillance to Support Treatment and Coverage Decisions

2021

1. Racial Health Disparities: A Closer Look at Benefit Design
2. Digital Therapeutics
3. Addressing Evidence Gaps in the Expedited Approval Process: Payer Perspectives

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Chief Quality Integration Officer
Physicians of Southwest
Washington/Multicare Connected Care

Agenda

- Background & pre-forum survey
- Forum findings and recommendations
- Q&A
- Next steps and action items

Forum Goals

- Identify key barriers by stakeholder to plan for and manage rare disease therapies
- Propose strategies to address these barriers
- Determine relevant differences in methods to manage one-time versus long-term therapies in managed care settings
- Recommend best practices that key stakeholders may apply to optimize rare disease therapies
- Suggest next steps for consideration as the category of rare disease therapies continues to evolve

Rare Diseases Create Unique Challenges

- A rare disease, as defined in the United States, is one that affects fewer than 200,000 patients
- Estimated 7,000 rare diseases affecting 30 million individuals
- Many rare diseases are serious or life-threatening with limited treatment options available
- This need led to the passage of the Orphan Drug Act (1983) to incentivize the commercial development of rare disease therapies
- The result was a substantial increase in the number of treatments
  - 58% of drugs approved by FDA in 2020 were indicated for rare or orphan drugs

EvaluatePharma, Orphan Drug Report 2019, 6th ed – April 2019; U.S. FDA, FDA News Room, Rare Disease Day 2020: FDA Continues Important Work on Treatments for Rare Diseases ; https://www.fda.gov/drugs/new-drugs-fda-cders-new-molecular-entities-and-new-therapeutic-biological-products/new-drug-therapy-approvals-2020#:~:text=Novel%20drugs%20approved%20in%202020,%2C%20Tazverik%2C%20Tepezza%2C%20Tukysa%2C

Orphan Drugs Offer Treatments Options for Rare Diseases

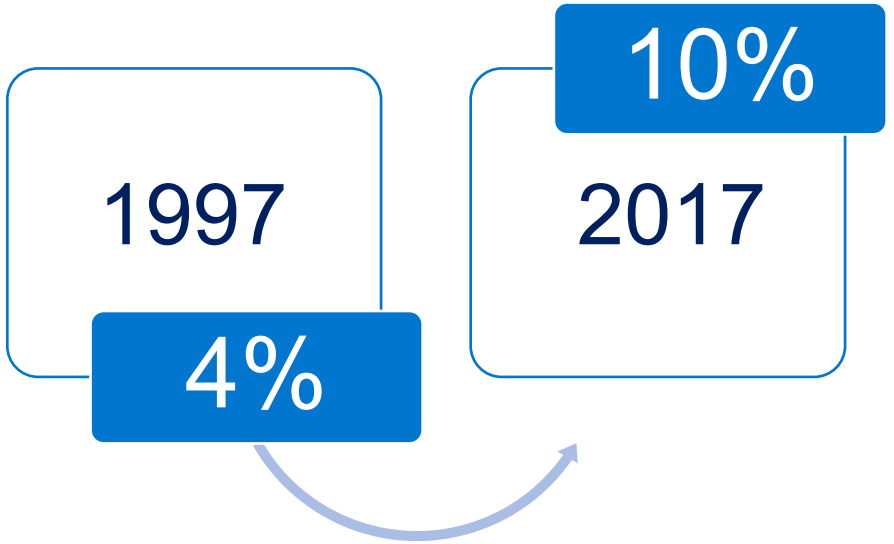
- Orphan drugs encompass both specialty drugs used chronically to manage a condition and one-time treatments such as gene therapies that modify a patient's genes with potential to cure disease
- Opportunity for important health gains in complicated populations with limited treatment options
- Represent a growing portion of the overall pharmacy and medical drug market
  - Orphan drugs account for ~one-third of the drug pipeline through 2024
  - Sales projections during this period are $242 billion

EvaluatePharma, Orphan Drug Report 2019, 6th ed – April 2019; U.S. FDA, FDA News Room, Rare Disease Day 2020: FDA Continues Important Work on Treatments for Rare Diseases; U.S. FDA, Office of Orphan Product Development; U.S. FDA, What is Gene Therapy?; Chambers JD et al, Orphan Drugs Offer Larger Health Gains but Less Favorable Cost-effectiveness than Non-orphan Drugs, J Gen Intern Med, 2020 Sep;35(9):2629-2636

Orphan Drug Innovations Come at a Cost

- Average cost of an orphan drug is **4.5-times higher** than the average cost of a non-orphan drug

- Orphan drugs contribute to a growing proportion of total drug spend



EvaluatePharma. Orphan drug report 2019. 6th ed. April 2019; IQVIA Institute for Human Data Science. Orphan drugs in the United States: growth trends in rare disease treatments. October 2018

Meeting the Affordability Challenges Posed by Orphan Drugs:
AMCP & PRECISION value

A Pre-Forum Survey of Payers, Providers and Employers

Objectives and Methodology

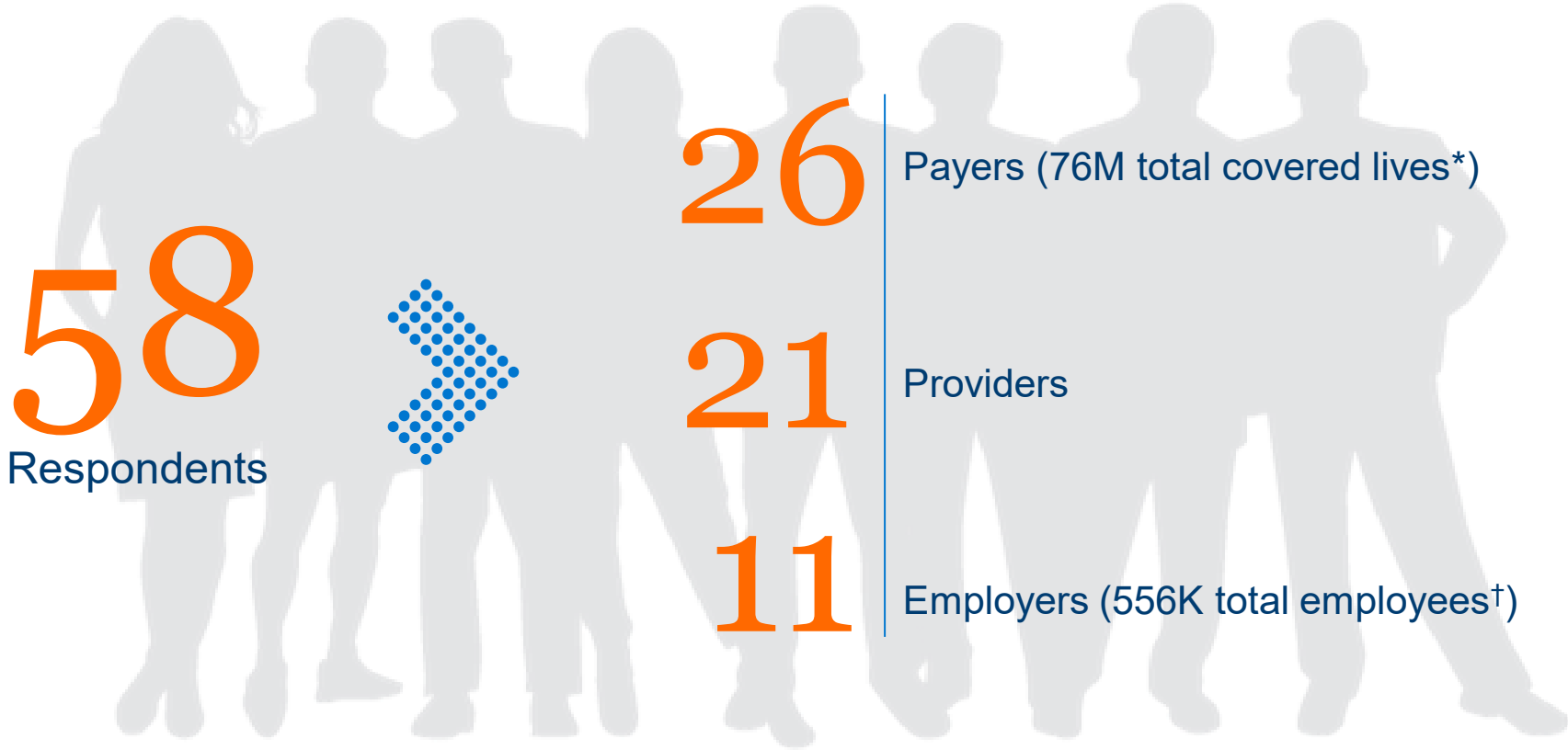
Objectives

- Characterize the challenges associated with addressing patients with rare/orphan diseases and understand how rare/orphan disease treatments are evaluated and managed
- Learn how stakeholders are meeting the financial challenges posed by rare/orphan diseases and associated treatments
- Understand how benefit design is impacting the coverage and utilization of rare/orphan disease drugs
- Identify strategies used to improve the care experience for individuals with rare/orphan diseases

Methodology

- PRECISION Value surveyed payers, providers, and employers from our proprietary network of market access decision makers (as well as AMCP attendees) to learn about the management of rare/orphan diseases
- Fielded between July 24 and August 4, 2020
- For the purposes of this research, a rare/orphan disease was defined as a condition that affects fewer than 200,000 people in the United States

Respondent Demographics



*Covered lives data for payers only available for plans in the PRECISION Value network (n=13); does not include respondents from payer component of health system or from AMCP attendee respondents (n=6). PBM respondents not reported due to small subsample (n=2) and disparate covered-life values.
†Represents employers only (n=5); does not include data from employee benefit consultants or coalitions or from AMCP attendee respondent (n=1).

Affordability Concerns are Compelling Stakeholders to Evolve Their Approach to Orphan Drugs

- Payers and providers increasingly considering healthcare economic information (HCEI), caregiver burden data, and real-world evidence as part of rare/orphan drug evaluations
- Payers and employers value cost offsets in direct healthcare costs
- Both payers and providers are challenged by limited long-term/outcomes data
- Payers are considering exclusions and carve-outs to maintain affordability of rare/orphan products
- Reinsurance, risk pools, amortization/installment models, and gene therapy carve outs are growing areas of employer management of rare/orphan drugs
- Some employers and payers are pursuing VBCs for high-cost rare/orphan drugs
- Both payers and employers are closely monitoring the pipeline

Payers Generally Apply Specialty Drug Utilization Management and Benefit Design Strategies to Orphan Drugs

- Most payers find that limited distribution networks reduce their ability to manage rare/orphan products
- Payer coverage of HCP-administered rare/orphan drugs can occur on either the pharmacy or medical benefit, based on financial considerations, site-of-care policies, and utilization management opportunities
- Payers generally cover rare/orphan drugs on the specialty tier or the tier with the highest patient cost-share
- Providers often obtain rare/orphan drugs through the buy-and-bill process, but may utilize an SPP based on payer requirements or reduction in administrative burden

Growing Interest Across Stakeholders in Rare Disease PROs and Patient Support Programs

- While providers are tracking PROs for patients with rare/orphan disease, most payers are not
- Payers most commonly support patients with rare/orphan disease through care management, disease state/medication education and support, and linkage to behavioral health resources
- Providers are growing their rare/orphan disease resources related to financial assistance, medication adherence, caregiver support, and behavioral health resources
- Employers are looking to expand offerings related to caregiver support, community resources, and financial resources for patients with rare/orphan disease
- Providers and employers are open to partnerships with pharmaceutical manufacturers, particularly related to financial resources, adherence, and disease state education

Preparing for and Managing Rare Diseases

Challenges Identified by Stakeholder

Issue	Patients/ Caregivers	Payers	Employers	Developers
Access	●	●	●	●
Benefit design	●	●	●	●
Clinical expertise		●	●	
Durability of effect		●	●	●
Education	●			●
Evidence gaps	●	●	●	●
Financial impact	●	●	●	●
Government regulations	●			●
Identifying patients		●	●	●
Product distribution		●		

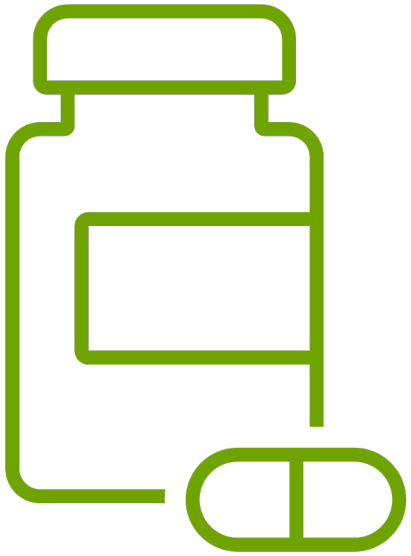
General Principles

- Planning often reactive and not cohesive or collaborative across various therapies, stakeholders, or groups of stakeholders
- Differences in ability to plan based on organizational size
  - Larger populations may offer greater predictability
  - National presence provides broader access to providers and treatment centers, often leading to fewer network challenges
- Differences related to segmentation
  - Self-insured groups require stop-loss coverage

Clinical Data

- Complicated clinical trials design/enrollment
  - Inherently small and potentially widely dispersed patient populations
  - Limited availability of disease-specific clinical sites
- Difficult to identify meaningful outcomes measured in reasonable timeframe to demonstrate efficacy appropriately
  - Heterogeneity in clinical course
  - Lack of well-established clinical endpoints
- Clinical trials may be conducted without a control
  - Some conditions have no alternative treatment
  - Ethical considerations with utilizing placebo
- Variation in pre-approval information exchange

Coverage



- Employers and payers may take a cautious approach to coverage
  - Uncertainties with available clinical evidence, appropriate treatment population, durability of effect
  - Potential lack of predictability of financial impact, patient portability
- Variability once coverage is in place (e.g., states with multiple Medicaid coverage providers)
  - Cost-sharing requirements
  - Medical vs pharmacy benefit
  - Type and restrictiveness of utilization management strategies
  - Genetic testing requirements
  - Process for re-approval

Financial Considerations

- Managing risk and anticipating budget impact
  - Difficult to predicting potential patient populations
  - Uncertainty in determining value of a given therapy
- One-time therapies often have considerable investment up front despite variable data on long-term durability of effect
  - Also, unique challenges if patient leaves initial treating plan
- Quality payment scores may represent financial risk if they are negatively impacted by delays in therapy
- Concerns around overall affordability/continued aggregate impact as additional therapies get approved
- Implications of buy and bill mark-up on overall costs

Value-based Contracting

- Payers often lack appropriate technology to support implementation
- Long-term tracking of patient outcomes must be operationalized
  - May require patient participation/coordination with other entities if the patient's health plan or provider changes
- Few opportunities to share lessons learned as there is little standardization and many details are proprietary
- Regulatory limitations (e.g., Medicaid Best Price, Avg Manufacturer Price, 340B Price Reconciliation, Antikickback Statute)
- Difficulty identifying a mutually agreed upon outcome

Access

- Identifying the right patients for treatment at the right time to prevent missed opportunities for beneficial interventions
- Supporting complex care coordination including ensuring receipt of ancillary services, availability of treatment centers
- Prevent and manage waste via appropriate vial sizes, appropriate dosing, etc.
- One-time therapies may require unique administration, nuanced management
- Supply chain management if limited distribution
- Dispensing preferences of providers who choose to buy and bill

Opportunities in Preparing for and Providing Timely Access to Rare Disease Therapies

Clinical Data

- Take FDA-approval requirements, payer evidence needs into accounts as soon as possible in the drug development process
- Develop core outcome sets
- Allow opportunities for transparent, timely, consistent pre-approval communication
- Increase communication between FDA and other stakeholders with respect to benefit vs risk assessment in approval process
- Use all available evidence throughout evidence review/coverage development
- Enhance collection of real-world evidence/long-term data through patient incentive programs, leveraging registry data

Financial Considerations

Value-based contracts

- Attempt to standardize some components (e.g., value determination)
- Sharing of best practices
- Mechanisms to spread risk

Value-based pricing

- Based on the strength of available evidence

Continue efforts to modernize price reporting under Medicaid Drug Rebate Program

Close loopholes in Orphan Drug Act

- e.g., direct contracting with developers could lead to positive price impacts as fewer parties would be involved in each transaction

Support and Education

- Videos of patients successfully reaching outcomes to demonstrate a medication's true benefit
- Ensure timely access and seamless care coordination
  - Bring treatment centers into the planning process early
  - Centers of excellence
  - Utilize support/hub services offered by many biopharmaceutical companies
- Assisting stakeholders via toolkits by disease and ongoing horizon planning and education especially for smaller entities by national organizations/associations

Participant Identified Best Practices

Topic	Summary
Priority outcomes	Engage patients/providers to advise on meaningful, priority outcomes
Treatment populations/ data collection	• Identify patient populations/associated risk (e.g., registries) • Patient incentive programs to support adherence/long-term data collection
Coverage development	• Open and transparent process for coverage criteria development • Include patients and clinical experts in a consultative capacity
Timely access	Through proactive contracting/having complex care models in place
Contracting arrangements	• Share lessons learned from VBCs, develop core outcome sets to support them • Consider rebate over time rather than payment over time arrangements
Regulatory support	• Modernize price reporting under Medicaid Drug Rebate Program, close loopholes in ODA • Legislation that enhances provision for proactive/timely PIE
Medication use evaluation (MUE)	Incorporate MUE principles to fill in knowledge gaps for value determination
Supply chain	Evaluate to address barriers, non-essential steps, and/or waste
Ongoing work groups	Utilize multidisciplinary, multi-stakeholder workgroups to provide ongoing input

ODA – Orphan Drug Act; PIE – pharmaceutical information exchange; VBC – value-based contract

Next Steps

AMCP PARTNERSHIP FORUM
No. 2 ⟹ 2020

EXECUTIVE **SUMMARY**

Preparing for and Managing Rare Diseases

To discuss how to ensure timely access to orphan drugs and gene therapies for rare diseases, AMCP held a virtual multidisciplinary stakeholder forum from Sep. 8–10, 2020. Forum participants represented diverse sectors, including integrated delivery systems, health plans, pharmacy benefit managers (PBMs), employer groups, biopharmaceutical companies, patient advocacy organizations, health policy researchers, and consulting firms, and were tasked with:

- Identifying key barriers by stakeholders around planning for and managing rare disease therapies and proposing strategies to address these barriers;
- Determining relevant differences in how to manage one-time versus long-term therapies in managed care settings; and
- Recommending best practices that key stakeholders may apply to optimize rare disease for rare disease therapies.

Participants acknowledged complex challenges associated with rare disease therapies:

- **Financial impact and limited data:** Most stakeholders are concerned about the financial impact of rare disease therapies. Payers and providers are additionally challenged by limited data on their durability of effect and the potential disconnect between clinical trial data and Food and Drug Administration (FDA)-approved labeling.
- **Uncertainty around key factors:** Some employers and payers take a cautious approach to coverage of rare disease therapies due to uncertainty around the available evidence, durability of effect, predictability of the financial impact, and patient mobility. These factors also impact their interest in implementing value- or outcomes-based arrangements.

continued on next page

WATCH FOR FOLLOW-UP

The Partnership Forum was just the beginning of AMCP's efforts to find innovative and collaborative solutions to support timely access to orphan drugs and gene therapies for rare diseases. From here, we will:

- **Publish a proceedings document** on all findings and recommendations from the Partnership Forum in an upcoming issue of AMCP's *Journal of Managed Care & Specialty Pharmacy* and disseminate it widely to decision makers around the country.
- **Host a forthcoming webinar** to report these findings and recommendations.
- **Provide more education** around establishing coverage policies for rare disease therapies.
- **Support efforts to modernize the Medicaid Drug Rebate Program** and the Orphan Drug Act to reflect changing insurance and payment models as well as pharmaceutical innovations.
- **Continue discussions with Members of Congress** to support legislation that enhances and provides proactive and timely pharmaceutical information exchange for rare disease therapies.

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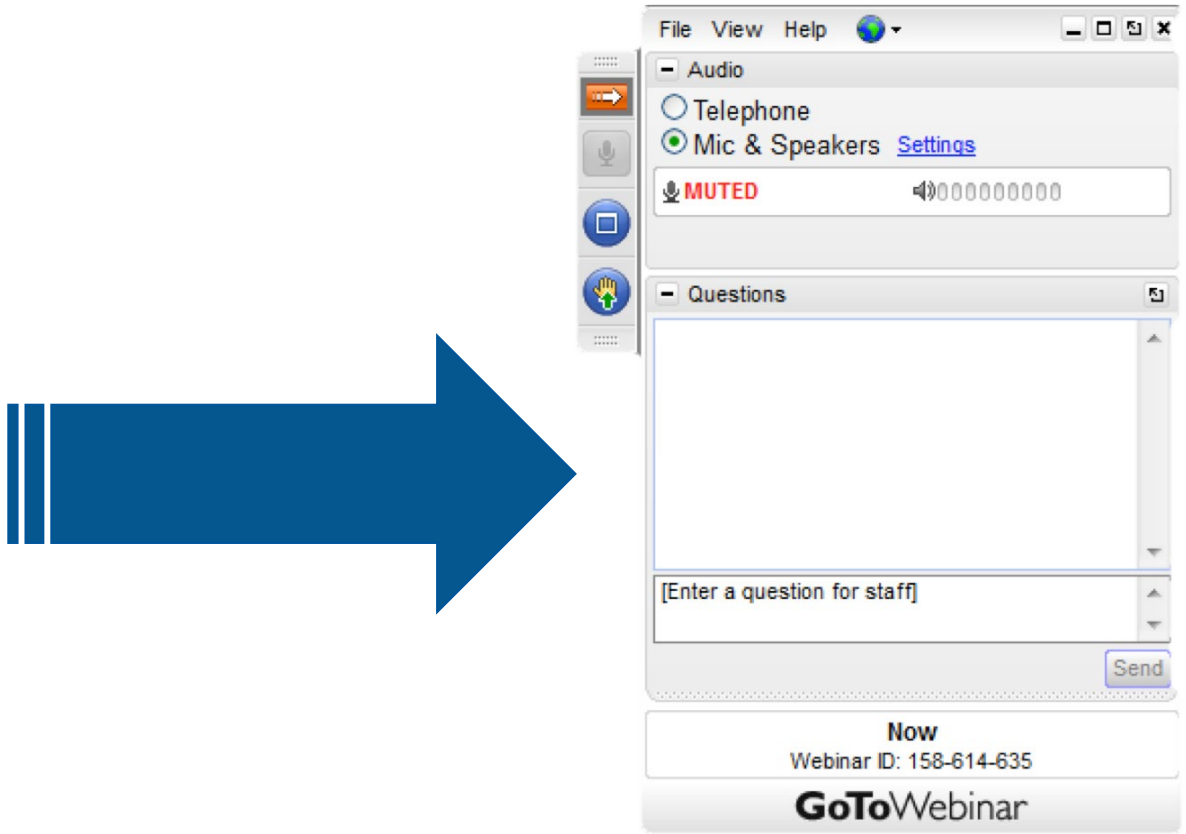
AMCP2021

Defining managed care pharmacy's future

Poster presentation: U24
Understanding Payer, Provider, and Employer Resources and Opportunities to Support Patients and Caregivers Impacted by Orphan Diseases.

Poster presentation: U3
Understanding the Challenges and Drivers of Orphan Drug Evaluation from the Payer, Provider, and Employer Perspectives.

How to Ask Questions



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To improve patient health by ensuring access to high-quality, cost-effective medications and other therapies.