PQA CONVENES: QUALITY MEDICATION USE IN RARE DISEASE

A Report on Stakeholder Perspectives on What Constitutes Quality

January 2025

TABLE OF CONTENTS

INTRODUCTION

A rare disease is a condition that affects fewer than 200,000 people in the United States. There are more than 7,000 rare diseases and together they affect more than 30 million Americans.

Only about five percent of rare diseases have FDA-approved treatments, but the development and approval of rare disease medications has grown rapidly over the last 15 years. In 2022, more than half of FDA novel drug approvals were for patients with rare diseases.

The growth of rare disease medications and the cost of rare disease care has prompted a growing focus on quality. Quality medication use in rare disease is about the care processes and outcomes that follow a medication prescription. This includes the degree to which medication use is:

- Accessible
- Affordable
- Timely
- Appropriate
- Safe
- Effective
- Equitable
- Adherent
- Persistent
- Monitored, managed and supported

Health care organizations today approach rare disease quality medication use in a variety of ways, and there are many perspectives on what constitutes quality and how to improve it. A need and opportunity exists to establish a consensus on what constitutes high-quality rare disease medication use.

To advance the national conversation, PQA, the Pharmacy Quality Alliance, hosted a one-day event on November 7, 2024, to discuss stakeholder perspectives on rare disease medication use quality.

The event, **PQA Convenes: Quality Medication Use in Rare Disease**, provided a platform for four key stakeholders in the medication use process to share their perspective on what constitutes quality:

- Patients, caregivers and patient advocates
- Specialty pharmacies and providers
- Biopharmaceutical industry organizations
- Public and private payers

This report summarizes the perspectives shared over a series of four discussion panels featuring 20 speakers and moderators.

The summaries document the many insights that the panelists provided from their professional perspectives. To foster an open dialogue, there are no quotes attributed to any individual panelists or organizations. The perspectives are wide ranging and numerous, reflecting both the panel format and the many issues impacting quality. We hope the dialogue captured in this report is a useful addition to the conversation on rare disease and quality.

Several factors impacting quality were commonly mentioned across all panels:

- Access, including insurance coverage and equitable access to screening, care and medications,
- Education for both patients and providers to better diagnose rare disease and manage rare disease care,
- Support for patients' complex journeys and needs, and
- Data to understand the patient experience over time and the impact of therapies on clinical outcomes and quality of life.

These issues may represent an opportunity for cross-sector collaboration to improve the quality of rare disease medication use. PQA looks forward to engaging the industry as it advances these and similar issues.

PATIENT, CAREGIVER AND PATIENT ADVOCATE PERSPECTIVES

The first panel featured patient, caregiver and patient advocate perspectives. In addition to working in the health care industry, nearly all panel participants are living with a rare disease or caring for an immediate family member with a rare disease. Affordable, equitable access and strong patient support systems were strongly noted as critical to quality outcomes.

Patient, Caregiver and Patient Advocate Perspectives

panelists

AMI GOPALAN | *PharmD, MBA, FAMCP, Senior Vice President, Head of Clinical and Editorial Services Market Access, Precision AQ*

INGRID HE | *JD, MBA, Director of Research, Autoimmune Registry*

AMY MILLER | *PhD, President, PhRMA Foundation*

CARL SCHMID | *MBA, Executive Director, HIV+Hepatitis Policy Institute*

EMILY EASTMAN | *PharmD, MBA, CSP, Associate Director-Centralized Specialty Pharmacy Services, University of Kentucky Specialty Pharmacy and Infusion Services (moderator)*

Access to care and medications

Patients regularly face barriers to medication access, but those issues are compounded for individuals with rare disease. The barriers include limited or no FDA-approved treatment options, delays in authorizations or medication availability, waiting to get into clinical trials, navigating off-label medication use and extensive costs related to rare disease care.

The time between when a physician prescribes a medication to when the patient has the medication in hand can take weeks to months because of factors related to insurance and medication availability. When there are no FDA-approved options for rare disease treatment, the only option left for many patients is long-term steroid use, which can have serious side effects. Although some rare disease patients find relief from medications designed to treat other conditions, off-label use is often not covered by insurance.

Many patients with health insurance coverage are enrolled in plans with high "out-of-pocket caps," making it difficult for patients to afford medications or pay for other needs. There are financial assistance and co-pay assistance programs to help, but these often do not count towards patients' annual cap.

Given the low volume of available rare disease medications, clinical trials are an important opportunity to access care and medications. Clinical trials provide access to costly therapies at no cost to the participant. Unfortunately, many trials are not accessible or practical for people who live in more rural areas. Decentralized trials could make these more accessible. Registries can also help patients access trials. There is an existing autoimmune registry that aims to collect demographics, prevalence, comorbidities, and long-term outcomes.

Effectiveness of the care coordination

Care coordination is critical for organizing, managing and supporting a patient's complex needs across multiple care providers and specialties. In today's system, care coordination largely falls to rare disease patients and their caregivers. Patients endure a trial-and-error process with all new providers. Panelists shared having to "fire" physicians who didn't talk with the other specialty providers they see for care. There are information gaps when patients switch insurance providers, and more information gaps when new residents and fellows rotate through their specialists' offices. All panelists agreed that without a full-time care coordinator, the onus of care coordination ultimately falls to the patient.

Key Quality Insight

Care coordination is critical for organizing, managing and supporting a patient's complex needs across multiple care providers and specialties.

Patients with rare disease see multiple providers, and the burden is on them to track who they've seen and when, which tests have been conducted, which medications they currently take, and which medications or treatments they have tried in the past. Patients must be their own advocates – ensuring providers have all the relevant history, asking questions, probing for more information and searching for providers who see them as whole people and help them maintain as much normalcy as possible. "Whole person" care is a patient-centered approach that accounts for an individual's physical, behavioral, psychological and social needs.

High-quality experience and positive outcomes

Having affordable, high-quality health insurance is the most beneficial tool in achieving positive outcomes and a positive patient experience. It's also paramount to find the right doctor. Patients need doctors who believe them without dismissing symptoms. They need providers who are willing to look for the right diagnosis and willing to work to find which treatments are most effective.

It's also important for patients and their caregivers to receive education on their condition so they can be better advocates. Patients who have the best experience are often a "squeaky wheel" who doesn't take no for an answer. Creating ambassador programs and awareness campaigns can provide a sense of community to patients and a way to connect with others who have the same condition.

Patients, especially the underinsured, would benefit from improved and expanded access to genetic tests, which could speed up diagnosis and help avoid unnecessary tests or unsuccessful treatments. There also are significant non-monetary costs to patients, when they are not accurately diagnosed, as ineffective medications can lower or delay improvements to quality of life and daily functioning.

Even when patients have health care training or knowledge, it can be difficult to navigate care and benefits. This underscores the challenges of our health care system and patients' need for partners and a supportive system to achieve the best outcomes.

Health equity

Health literacy and limited time and resources are major barriers to high-quality care for many rare disease patients. Simply being able to take time off from work to travel for appointments is not feasible for many patients. Patient advocates and knowledgeable case workers are needed to support these individuals.

For populations that speak little to no English, who need translators or who may be undocumented, there are additional barriers to care. Building trust with these communities is the first step in breaking down barriers. Patients need to feel safe and believe you are working in their best interests.

Patients need to be met where they are, by people who look at the journey from the patient's perspective rather than the clinical perspective. The "what" and "why" need to be explained. If the care plan doesn't make sense to the patient, the individual is much less likely to participate in follow-up care. Additionally, patients who have lower levels of education often have their concerns dismissed by physicians and are less equipped to "fight" for a different answer.

Opportunities for progress

- An increased focus on whole person care is important for rare disease patients, given the many physical, mental, social and economic issues that affect their health and wellbeing.
- Many rare diseases are chronic and long-term conditions and shifts from fee-for-service to fee-for-value care models could provide patients with more consistent care management and access to services, which could benefit long-term health outcomes and costs.
- As changes to insurance policy are developed and implemented, such as those of the Inflation Reduction Act of 2022, it will be important to monitor for unintended consequences, for example, on co-pay assistance program eligibility, especially for off-label use.
- To address health equity challenges, underserved rare disease patients would benefit from a program similar to the Ryan White HIV/AIDS Program, which provides a comprehensive system of primary medical care, medications, and essential support service for low-income people.

SPECIALTY PHARMACY AND PROVIDER PERSPECTIVES

The second panel featured specialty pharmacy representatives with perspectives from organizations of different size, specialization and type. All feature high-touch patient services and care coordination in support of rare disease patients and their medication use.

Patient management and support

Specialty pharmacies support patients with rare disease through a variety of high-touch services that are centered around specially trained pharmacists and care teams. Many pharmacies have staff who are assigned to specific patients to optimize the coordination of their care across multiple providers and therapies.

Other ways pharmacies support patients include embedding pharmacy services into clinics, when possible (most often via integrated health systems), engaging patients in conversations about issues and follow-up care, asking about medication side effects, and providing routine reminders about refills. Specialty pharmacies strive to get medications to patients quickly because time-to-treatment affects quality.

To help improve quality, specialty pharmacies would benefit from broader access to patients' health records and expanded access to medications. Access to patient health records enables specialty pharmacies to help patients navigate medication approval processes, coordinate care and provide needed services. Also, limited distribution networks for certain medications can present challenges for coordinating patient care and ensuring timely, convenient access when patients must use new or multiple pharmacies for their medications.

Addressing barriers to care and medication access

The cost and burden of rare disease makes accessing care and medications more challenging for many patients. Specialty pharmacy's high-touch model is well suited to identify and help address the barriers patients face. Through screening, pharmacies can help connect patients with home health services, support services, patient advocates and peer support groups.

Understanding the needs of the whole patient are important for high quality care. First, acknowledge and look for the barriers their patients face, including the stress of dealing with chronic conditions and accessing medications. It's important to remember patients are more than their conditions, and they shouldn't be seen only by their conditions. Pharmacies and other providers need to build relationships with the patients they serve.

Specialty Pharmacy and Provider Perspectives

panelists

CHRISTOPHER R. ANTYPAS | *PharmD, President, Perigon Pharmacy 360*

WILLIAM TROMBATT | *PharmD, CSP, Manager, Clinical Programs, Walgreens Specialty Pharmacy*

DUSTIN DONALD | *PharmD, CSP, Clinical Pharmacy Team Lead – Rare Disease, Vanderbilt Specialty Pharmacy, Vanderbilt University Medical Center*

MICHAEL DUTEAU | *RPh, President, Noble Health Services*

CHRIS KOTSCHEVAR | *PharmD, CPHQ, Director, Stakeholder Engagement, PQA (moderator)*

Key Quality Insight

Access to patient health records enables specialty pharmacies to help patients navigate medication approval processes, coordinate care and provide needed services.

In rural areas, case management is even more important because patients are traveling long distances for visits and may need information and options on accessing components of their follow-up care closer to home. Similar challenges are faced by patients from lower socioeconomic backgrounds in urban and suburban areas.

Collaborations to expand pharmacy services

Payment models can be a barrier to pharmacy services, as fee-for-service models around medication dispensing do not support robust care management. Collaborations, however, with biopharmaceutical industry partners and employer-sponsored plans can help address this. One example is arrangements that integrate case management with home infusion services.

Another collaboration opportunity is for pharmacy information to be integrated into patients' electronic health records (EHR) within health systems. This can help fill information gaps and connect care teams. However, this approach also brings challenges when health systems use different EHR platforms. Working with multiple EHR platforms creates additional work and reduces efficiencies.

Finding HIPAA-compliant means of communications – within the regulations that determine which communication methods are secure and which are not – can make collaborations challenging. These regulations often delay communications and make it more difficult to discuss a patient's care plan. Very often, pharmacies are still using fax communications, and current technologies could enable faster and easier information sharing.

Opportunities for progress

As pharmacies continue to focus on providing quality care, we need to remember the patient experience is a key factor in whether a patient feels they have received high quality care.

- A decrease in time to treatment, so patients can access medication therapy faster, is one area to focus on improving the patient experience. To achieve this, it's not just about shortening the pharmacy turnaround time, it's also about how the pharmacies are partnering with stakeholders.

- Removing the barriers pharmacies face in the distribution process, expanding access to new medications from the beginning of the launch period, and getting prescriptions in the right place faster to shorten patient wait times are other areas ripe for improvement.
- Performance should dictate access to distribution networks; an objective measurement of pharmacy performance should factor into deciding where patients can access medications. It's a privilege to care for patients and pharmacies should be held accountable to standards.
- Collecting and sharing data on pharmacy's impact on turnaround times, hospitalization rates, adherence, and clinical outcomes is important for determining quality. In addition, qualitative data on patient experience and satisfaction is needed.

BIOPHARMACEUTICAL INDUSTRY PERSPECTIVES

The third panel featured biopharmaceutical industry representatives with perspectives from leaders involved in the research, development and evaluation of medications. The role of data from therapy development to real world use is critical for understanding the quality of medication use.

Biopharmaceutical Industry Perspectives
panelists

DAVID LITWACK | *PhD, Associate Vice President, Scientific Strategy and Communications, Prevail Therapeutics, a wholly owned subsidiary of Eli Lilly*

NANA NUMAPAU | *PharmD, Director, US Health Economics & Outcomes Research, Alexion Pharmaceuticals*

RICHARD CHAPMAN | *PhD, MS, Chief Science Officer, Center for Innovation and Value Research*

LOREN KIRK | *PharmD, CPHQ, CAE, IOM, Senior Director, Strategic Partnerships, PQA (moderator)*

Ongoing data collection is critical to understanding quality and establishing value

One of the challenges in identifying the full value of treatments in rare disease is limited data due to small populations. From small clinical trials to limited real-world data, rare disease medication use is often marked by uncertainty.

Key Quality Insight

Ongoing collection of rich data that captures the full patient experience is critical to establishing the quality and value of rare disease medications.

Ongoing collection of rich data that captures the full patient experience is critical to establishing the quality and value of rare disease medications. Connecting early and often with the whole patient and including family and caregiver perspectives is critical. Snapshots of patients with rare diseases never tell the whole story. And for patients who have progressive diseases, they will have changing needs.

Central data repositories can answer questions, but when it's not rich data there are a lot of missed aspects. Registries help us follow patients longer and provide more data points. Even de-identified data still gives the opportunity to follow patients through their care. Data silos create challenges for data collection and real-world use. Patient registries, surveys and data aggregation by trusted, third parties can help mitigate these issues and support the connection of data that tells a more complete patient story.

Diagnostic journey and importance of education

The journey to diagnosis often takes years for rare disease, and delayed diagnoses are affected by gaps in physician knowledge and education on rare disease. Unfortunately, misdiagnoses are common in patients with rare disease because some of the symptoms are similar to those of more common conditions. For example, frontal lobe dementia can often be confused with Alzheimer's disease based on the patient's symptoms, and often as a result of a general practitioner's limited knowledge of rare conditions.

There are opportunities to shorten the time to diagnosis through increased screening for newborns, genetic testing, and the involvement of genetic counselors. Some providers need to be convinced there is value in genetic testing, so there is a place for broad education of its role, appropriate use and benefits. Additional opportunities include sponsoring the genetic testing needed to gain

access to trials and identifying technologies to make it cheaper and easier to conduct genetic testing.

In the current state of rare disease management, biopharmaceutical researchers and patients have more knowledge than most physicians. But there are ample opportunities to support provider knowledge. We can utilize advocacy groups to create more physician awareness of rare diseases and the common instances of confusion. They also can provide access to patient and caregiver education, as well as tell patient stories.

We can utilize telemedicine more to provide easier access to expert providers, which can also help patients better manage their care by eliminating long travel times for appointments.

Access to care and medications

Accessing care, trials and therapies is an incredibly complicated equation. When considering patient access, we have to recognize access encompasses many issues including cost, affordability, care site locations, and the availability of specialists.

We must put the patient first. We can decrease as many roadblocks as we can for individuals, have ongoing discussions, and strive to make drugs more affordable. We can cut costs to patients by improving processes and reducing costs in manufacturing without stifling innovation.

The overall cost of care greatly impacts patients' access and their outcomes. Reduced medication costs and increased affordability are not the only solutions since there are other costs for patients. Clinical trials are a way for patients to access new treatment options with little to no direct costs. However, clinical trials often have small enrollments and there are time and travel considerations that can impact participation.

For drugs that are out of the trial phase, we must improve the distribution timing. Some rare disease medications require special monitoring and timing. We need to consider if expanded access would improve distribution speed. Will distribution become easier through utilization of home health monitoring? Will we be able to send information faster?

Opportunities for progress

- We need to decrease the time to the diagnosis of rare diseases because earlier diagnosis can lead to better outcomes.
- The entire health care industry needs to make continuous investments in rare disease research and data collection to ensure we are capturing as many patients and as much diversity in the data as possible. We should strive to gather more real-world evidence.
- Patient goals for disease state management must be considered alongside other measurable health outcomes.

- We can better utilize genetic testing and communicate its value to gain insights and improve diagnostics.
- We should continue to explore the use of artificial intelligence as a tool to find information to fill data gaps. Can it extract information from EHRs? Can it sift through patient records to core patients and find additional patients who should be included in studies or data registries?
- Collaboration is another way to improve quality. Patient advocates, caregivers, and clinicians can collaborate with industry to strengthen clinical trials. There is potential for neutral third parties to unite competing groups to drive progress and produce credible results without commercial bias.

PUBLIC AND PRIVATE PAYER PERSPECTIVES

The fourth and final panel featured payer perspectives with insights from individuals with experience leading commercial, Medicare, Medicaid and state programs and plans and managing prescription drug coverage for health plans. Payers are focused on medication outcomes and the impact of therapies on total costs of care.

Key indicators of quality and value

When medications are prescribed, payers want to make sure the medications are clinically appropriate, that patients are provided supportive monitoring and care management, and have access to additional support to ensure their needs are routinely being met.

When looking for quality and value, payers are looking for safety and efficacy. Payers want to know:

- did the medication do what it was prescribed to do?
- what are those outcomes?
- how does it impact the total cost of care?

Ideally the medications are showing a long-term reduction to the cost of health care.

Payers are always looking at new drugs coming to the market, the clinical implications and impact on whole-person care, as well as for holes in the evidence. Often, there is limited data for new medications or medications for small populations, which makes evaluations of quality challenging.

Public and Private Payer Perspectives
panelists

DAVID KELLEY | *MD, MPA,*
Chief Medical Officer, Pennsylvania Department of Human Services Office of Medical Assistance Programs (Medicaid)

DRAKE REITER | *PharmD,*
Director, Pharmacy Operations, Priority Health

JAY TRAN | *PharmD, MHA,*
Vice President, Clinical Operations, Capital Rx

MICAH COST | *PharmD, MS, CAE,*
Chief Executive Officer, PQA (moderator)

Specialized services, support services and care management

Specialized services and support play a key role in helping patients have a high-quality experience and the best possible outcomes. Payers use a variety of strategies and partners to support complex case management for individuals with rare disease.

Payers look for community-based programs, including waiver programs, special needs units for children and wrap around services to better assist their members to ensure their needs are met. This care management approach includes a focus on maximizing in-network care opportunities, while also tracking issues such as the distance between patients and their pharmacies and providers, which can impact use and outcomes.

Payer size often impacts the approach to case management. Large payers may have the staff and internal structure to provide direct care management, while smaller payers may rely on external services to provide care management or leverage provider partnerships to assist.

Technology is playing an increasing role in care management. Prompts and reminders of upcoming appointments, medication refills and other aspects of care can support patient engagement and timely care.

Some payers are prepared to help patients meet basic needs, such as housing. They are interested in screening patients for social needs to be able to refer individuals to helpful resources. When a crucial resource is missing, the whole care plan may fall apart.

Addressing barriers to care and medication access

The social determinants of health (SDOH) significantly impact the quality of rare disease medication use, given the high burden of these conditions. Payers employ a variety of strategies to address SDOH and help patients achieve the best outcomes.

Given its focus on providing insurance for people with low income, Medicaid is often well positioned to address SDOH, though programs vary by state. Many Medicaid programs are set up to be a patient's medical home and screen enrollees for social needs. The data collected can be mapped and coded using Z codes, which are a set of ICD-10-CM diagnosis codes that identify nonmedical factors that can impact a patient's health. This allows the program to help identify appropriate resources for patients in their area.

Key Quality Insight

Care management is the backbone of quality.

For commercial payers, SDOH remains an area of focus, and through partnerships with other organizations, they can collect the same level of detail as Medicaid. For some payers, there are questions around who should collect the data and how. For example, community pharmacies could be a place to regularly collect data on patients' needs, but many pharmacies are understaffed; and while data is a crucial component, there are few incentives for pharmacies to collect it. It's an issue where commercial payers will continue to focus.

When data is routinely collected, the next step is following up on assessments. Are needs still present or have the gaps been closed? It's not uncommon to see other needs arise as previous needs are met. For example, once housing issues are addressed, we may see transportation become a higher need. Ultimately, the goal is to connect the social needs identified to measurable actions that close gaps and improve outcomes. However, it is important to keep focus on the patient perspective throughout this work. For individuals facing social needs, SDOH screenings aren't always comfortable, so it is payers' responsibility to show patients they can trust us and we will take care of them.

Leveraging centers of excellence

Many payers work with rare disease centers of excellence to drive quality care because of their patient services, data and care coordination. Some payers use third-party vendors to engage with centers to provide wrap around services at competitive rates. For other payers, it's a negotiation initiated by a center to position itself as a go-to provider designed to achieve better outcomes for rare disease patients. Some of these partnerships are born out of geography, and some areas are home to multiple centers of excellence that are effectively leveraged by state or regional plans and programs.

Through working with centers of excellence there are opportunities to use their registries and robust data sets. Data sharing can be challenging, though, because the information is very sensitive, with access and confidentiality issues. Third parties are often engaged to consolidate and manage data in these cases.

Opportunities for progress

- Additional data on long-term medication outcomes and comparative clinical effectiveness would strengthen our ability to evaluate the quality of rare disease medication use.
- Standardizing coverage criteria would benefit patients when they change insurance plans, so that they do not have to repeat certain aspects of the care and authorization processes.
- Access, sharing and analysis of both pharmacy and medical data is critical to effective, coordinated care and evaluating the quality of care.
- Care management is the backbone of quality. Its continued improvement is a top priority.

ABOUT PQA

PQA, the Pharmacy Quality Alliance, is a national quality organization dedicated to improving medication safety, adherence and appropriate use. A measure developer, researcher, educator and convener, PQA's quality initiatives support better medication use and high-quality care. PQA was established in 2006 as a public-private partnership with the Centers for Medicare & Medicaid Services. PQA was created because prescription drug programs were a major area of health care where there was no organization or national program focused on quality improvement. Today, PQA is an independent, non-profit organization with more than 200 diverse members across health care.

About PQA Convenes

PQA Convenes brings together national leaders in medication use quality to build consensus and develop plans of action to promote innovative and timely opportunities for improving patient care and outcomes. A gathering of diverse thought leaders and decision makers, PQA Convenes is designed to:

- Explore how medication use quality and pharmacist-provided care can improve care delivery, patient and provider experiences and patient outcomes.
- Clarify unmet market needs, gaps in care, or interventions that can be realized through research, education and collaboration.
- Provide a collective call to action, which can include white papers or consensus statements, follow-up or expanded convenings and communications and engagement strategies to build broader awareness.

Visit PQAalliance.org to learn more. For questions about this report, email PQA at Info@PQAalliance.org.

ACKNOWLEDGEMENTS

PQA would like to thank the 20 speakers, panelists and moderators, whose experience, perspectives and insights informed our understanding of what constitutes quality in rare disease medication use and how to improve it. PQA members recommended dozens of experts, thought leaders and individuals with lived experience for PQA Convenes, and their input helped make this event relevant for today's rare disease landscape. Caroline Green, MD, MBA, FACP, conducted extensive background research on rare disease medication use in 2023 as part of a PQA MPH Practicum, and her work greatly informed PQA's approach to this event.

Leslie Hughan of the Blue Wagon Group acted as writer for this report and synthesized the many complex issues discussed. PQA staff, particularly Rachel Cormier, Richard Schmitz and Virginia Sweeter, were instrumental in the success of this PQA Convenes event.

PQA Convenes: Quality Medication Use in Rare Disease and this report were funded by the generous support of Pfizer, Novo Nordisk and Jazz Pharmaceuticals. PQA does not endorse, recommend or favor any organization, or its products or services. PQA general funds also supported this work.

APPENDIX A

PQA CONVENES: QUALITY MEDICATION USE IN RARE DISEASE EVENT AGENDA

Thursday, November 7, 2024 | 8:00 a.m. - 2:00 p.m. ET
Renaissance Arlington Capital View | Arlington, VA

7:00 a.m. - 8:00 a.m.	**Breakfast**
8:00 a.m. - 8:30 a.m.	**Event Welcome and Overview** MICAH COST | *PharmD, MS, CAE, Chief Executive Officer, PQA* RICHARD SCHMITZ | *Chief Engagement Officer, PQA*
8:30 a.m. - 9:30 a.m.	**Patient, Caregiver and Patient Advocate Perspectives** AMI GOPALAN | *PharmD, MBA, FAMCP, Senior Vice President, Head of Clinical and Editorial Services Market Access, Precision AQ* INGRID HE | *JD, MBA, Director of Research, Autoimmune Registry* AMY MILLER | *PhD, President, PhRMA Foundation* CARL SCHMID | *MBA, Executive Director, HIV+Hepatitis Policy Institute* EMILY EASTMAN | *PharmD, MBA, CSP, Associate Director – Centralized Specialty Pharmacy Services, University of Kentucky, Specialty Pharmacy and Infusion Services (moderator)*
9:30 a.m. - 9:45 a.m.	**Break**
9:45 a.m. - 10:45 a.m.	**Specialty Pharmacy and Provider Perspectives** CHRISTOPHER R. ANTYPAS | *PharmD, President, Perigon Pharmacy 360* WILLIAM TROMBATT | *PharmD, CSP, Manager, Clinical Programs, Walgreens Specialty Pharmacy* DUSTIN DONALD | *PharmD, CSP, Clinical Pharmacy Team Lead – Rare Disease, Vanderbilt Specialty Pharmacy, Vanderbilt University Medical Center* MICHAEL DUTEAU | *RPh, President, Noble Health Services* CHRIS KOTSCHEVAR | *PharmD, CPHQ, Director, Stakeholder Engagement, PQA (moderator)*
10:45 a.m. - 11:00 a.m.	**Break**
11:00 a.m. - 12:00 p.m.	**Biopharmaceutical Industry Perspectives** DAVID LITWACK | *PhD, Associate Vice President, Scientific Strategy and Communications, Prevail Therapeutics, a wholly owned subsidiary of Eli Lilly* NANA NUMAPAU | *PharmD, Director, US Health Economics & Outcomes Research, Alexion Pharmaceuticals* RICHARD CHAPMAN | *PhD, MS, Chief Science Officer, Center for Innovation and Value Research* LOREN KIRK | *PharmD, CPHQ, CAE, IOM, Senior Director, Strategic Partnerships, PQA (moderator)*
12:00 p.m. - 12:45 p.m.	**Lunch**
12:45 p.m. - 1:45 p.m.	**Payer and Policy Perspectives** DAVID KELLEY | *MD, MPA, Chief Medical Officer, Pennsylvania Department of Human Services Office of Medical Assistance Programs (Medicaid)* DRAKE REITER | *PharmD, Director, Pharmacy Operations, Priority Health* JAY TRAN | *PharmD, MHA, Vice President, Clinical Operations, Capital Rx* MICAH COST | *PharmD, MS, CAE, Chief Executive Officer, PQA (moderator)*
1:45 p.m. - 2:00 p.m.	**Event Closing** Summary of day's discussions and perspectives Observations on opportunities ahead