AMCP Webinar:
The Challenges and Opportunities in Rare Disease Management

Agenda

- Welcome and learning objectives
- Brief background on Bayer Pharmaceuticals
- Background on rare diseases
- Key challenges with rare disease products
- Panel Discussion
- Conclusion
Introduction of the panel members

**Moderator**

**Ed Feeley**  
VP, Account Management, Market Access,  
Bayer Pharmaceuticals

**Panel Members**

**Kevin Host, PharmD**  
President & COO, Pharmaceutical Strategies Group LLC

**Randy Falkenrath**  
VP, Specialty Strategy, Humana Pharmacy Solutions

**Atheer A. Kaddis, PharmD**  
SVP, Business Development & Strategy, Diplomat Specialty Pharmacy

---

**Learning Objectives**

1. To identify current challenges and trends in rare disease management
2. To identify current and potential rare disease management practices by key stakeholders
3. To discuss opportunities to enhance collaboration and engagement with key stakeholders
A Long Tradition – Over 150+ Years of Innovation

- In the past 150 years, Bayer’s inventions have been designed to improve quality of life for our customers:
  - Aspirin, polycarbonate, polyurethane, and the first synthetic insecticide are among the catalog of chemicals invented by scientists at Bayer.

- This great tradition is also our commitment to the future, consistent with Bayer’s mission: Science For A Better Life
- Bayer has more than 600 collaborations with research organizations around the world

Our Mission: Science for a Better Life

As an innovative healthcare company, we are committed to:

- Shaping the future with innovations that will benefit with health and well-being of people and animals
- Leading the industry in our chosen therapeutic areas and direct research and development to therapeutic areas with a high unmet medical need
- Being respected for our high-performance culture that attracts, retains, and develops the best talents
- Being trusted by our customers
- Delivering on our promises to stakeholders
- Acting in a socially responsible manner

Key Business Areas

- Pharmaceuticals
- Consumer Health
- Crop Science
Bayer Pharmaceuticals - Business Areas

CARDIOVASCULAR DISEASES
- Pulmonary Hypertension
- Heart Failure
- Thrombosis
- Acute Coronary Syndrome
- Cardiorenal Syndrome (CRS)

ONCOLOGY
- Cell Cycle/Survival Signaling
- Immunotherapy/Flawbody-Drug Conjugates
- Tumor Metabolism/Hypoxia
- Chromatin Modulation/OncoGenomics

OPHTHALMOLOGY
- Inflammation
- Magnetic Resonance and Computed Tomography Contrast Media Research

WOMEN’S HEALTH
- Leveraging the full potential of development compounds
- LCM Fertility Control/Menopause-Management
- Ophthalmology

HEMATOLOGY
- Sickle cell anemia and Thalassemia
- Acute and rare bleeding disorders
- Extended half life technology and gene therapy

RADIOLOGY
- Multiple sclerosis
- Infections

OTHER ILLNESSES
- Pulmonary Hypertension/Heart Failure
- Thrombosis/Acute Coronary Syndrome
- Cardiorenal Syndrome (CRS)

Cardiology

Oncology

Hematology

Gynecological Therapy

Common Mechanism Research and Other Areas
- Inflammation
- Magnetic Resonance and Computed Tomography Contrast Media Research

Bayer Pharmaceuticals Research Focus
Developing Products Across 4 Therapeutic Areas
Bayer’s Market Access Organization:
Adapting to Evolving Customer Needs in a Changing Market

Landscape Assessment
• Growing, aging, more diverse population
• Significant growth and impact of specialty pharmacy
• ACA accelerating change
• Payment reform and evolving delivery models

Customer Reaction
• Building capabilities in population-based care
• Consolidation and integration for scale
• Managing growth in specialty spend
• Formalizing clinical decision making via pathways/guidelines
• Quality measure attainment (ACO measurements)
  • Population Health and Preventative Health

Implications for Bayer
• Enhanced value proposition that includes product, services, and outcomes
• Therapeutic density and product differentiation
• National strategies that allow for local decision making and execution
• Deeper customer collaborations

Background on Rare Diseases

Incidence & Prevalence
• In the US, a rare disease is a condition that impacts less than 200,000 Americans\(^2\)
  • This definition was created by Congress in the Orphan Drug Act of 1983\(^1\)
• There may be as many as 7,000 rare diseases\(^2\)
• Total number of Americans living with a rare disease is estimated at 25-30 million\(^2\)

Tracked Rare Diseases in the US (Upon Diagnosis)\(^2\)

- Infectious Diseases
- Birth Defects
- Cancers
- Diseases on State newborn Tests

Since most rare diseases are not tracked, it is hard to determine the exact number of rare diseases or how many people are affected\(^2\)

Background on Rare Diseases

Rare Disease Products

- Since 1983, The FDA (through The Office of Orphan Products Development) has helped to bring to market more than 400 drugs and biologic products for rare diseases²
  - The FDA can grant orphan status to drugs or biologics that treat a rare disease⁴
- As of 2016, there are currently 566 products in development for rare diseases⁴

US Drugs Approvals with Orphan Drug Designation³

<table>
<thead>
<tr>
<th>Year</th>
<th>2013</th>
<th>2014</th>
<th>2015</th>
<th>2016</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>28</td>
<td>45</td>
<td>49</td>
<td>37</td>
</tr>
</tbody>
</table>

Challenges facing rare disease products

- Cost to patients
- Cost to payers
- Appropriate clinical support for patients
- Distribution models and network
- Coordination across stakeholders

References:
Challenges facing rare disease products: Cost To Patients

Economic Considerations:
- Out of pocket costs, copayments, coinsurance, deductibles, premiums

Discussion Questions
1. How do payers balance the need to control costs with patient out of pocket costs?
2. How have treatment costs impacted rare disease patients?

Challenges facing rare disease products: Cost To Payers

Cost To Payers
- Payer sensitivity to the cost of orphan drugs is rising as some orphan drugs can cost payers hundreds of thousands of dollars annually per patient
- Additionally, Orphan drugs are increasingly subject to formulary restrictions

Discussion Questions
1. How are plan sponsors responding to the challenges in rare disease management?
2. What changes are needed to effectively manage specialty drugs covered under the medical benefit?
Challenges facing rare disease products: Coordination across stakeholders

Coordination across stakeholders

- Manufacturers, healthcare professionals, payers, and other stakeholders need to coordinate to provide optimal patient care

Discussion Questions

1. How are Manufacturers and Specialty pharmacies and other stakeholders (i.e. treatment centers) working to improve patient care?

2. How are treatment centers (ex: cystic Fibrosis, Hemophilia, etc) being incorporated into rare disease management models?

Key Takeaways

1. An increase in drugs approved for the treatment of rare disease is causing key stakeholders to reevaluate traditional methods for managing access and care for patients

2. Management of rare disease requires a holistic approach that is both financially prudent and patient centric

3. Collaboration and engagement between key stakeholders is critical to providing appropriate access to pharmaceutical and biologic products as well as optimal patient care
Thank You

Appendix
### Challenges facing rare disease products: Additional Discussion Points

<table>
<thead>
<tr>
<th>Category</th>
<th>Discussion Points</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Cost To Patients</strong></td>
<td>What are some recent trends in FDA approvals for specialty pharmaceuticals to treat rare diseases? How do more treatment options impact patient costs?</td>
</tr>
<tr>
<td><strong>Coordination across stakeholders</strong></td>
<td>What type of clinical and financial support do specialty pharmacies provide to patients diagnosed with rare diseases?</td>
</tr>
<tr>
<td><strong>Cost To Payers</strong></td>
<td>What are the main condition management differences and constraints across the plan sponsor types (Medicare, Commercial, Medicaid, ACA exchanges)?</td>
</tr>
<tr>
<td><strong>Distribution Models and Networks</strong></td>
<td>With most medications for distributions being limited distribution drugs, how do pharmaceutical manufactures choose which pharmacies are included in limited distribution networks?</td>
</tr>
</tbody>
</table>

---

Page 19  Bayer