PARTNERSHIP FORUM
No.1 — 2022

High-Investment Medications

APRIL 26-27, 2022 | RENAISSANCE ARLINGTON CAPITAL VIEW HOTEL
Moderator Welcome

Disclaimer

Organizations may not re-use material presented at this AMCP webinar for commercial purposes without the written consent of the presenter, the person or organization holding copyright to the material (if applicable), and AMCP. Commercial purposes include but are not limited to symposia, educational programs, and other forms of presentation, whether developed or offered by for-profit or not-for-profit entities, and that involve funding from for-profit firms or a registration fee that is other than nominal. In addition, organizations may not widely redistribute or re-use this webinar material without the written consent of the presenter, the person or organization holding copyright to the material (if applicable), and AMCP. This includes large quantity redistribution of the material or storage of the material on electronic systems for other than personal use.

Jennifer Graff, PharmD
Senior Director for Professional Affairs
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

• Provide a voice for stakeholders

• Find common ground and gain consensus

• Identify actionable results

• Amplify to raise visibility
Goals of this Partnership Forum

• Identify stakeholder challenges associated with high-investment medications
• Explore opportunities and financial tools to address those challenges
• Discuss potential policy solutions to improve the predictability, affordability, and accessibility of high-investment treatments
Thank You to Our Sponsors
Our Faculty

Brent Eberle, RPh, MBA
Sr. Vice President, Chief Pharmacy Officer
Navitus Health Solutions

Anne Jackson, FSA, MAAA
Principal
Milliman, Inc

Erin Lopata, PharmD, MPH
Vice President, Access Experience Team
PRECISIONvalue
Agenda

• Key Background

• Forum findings
  • Stakeholder Challenges
  • Existing and Emerging Financial Tools
  • Q&A

• What’s next?
Key Background
High-Investment Medications Encompass Many New Drugs

- Cell and Gene Therapy (Durable Therapies)
- Oncology
Gene and Cell Therapies

- Hemophilia A & B
- Duchenne muscular dystrophy
- Sickle cell disease
- Beta thalassemia
- Choroideremia
- Mucopolysaccharidosis type IIIA
- Leber’s hereditary optic neuropathy
- Severe combined immunodeficiency
- Wiskott Aldrich syndrome
- Von Gierke disease
- Wilson’s disease
- Retinitis pigmentosa
- Metachromatic leukodystrophy
- Urea cycle disorders
- Bladder cancer
- Angina pectoris
- Osteoarthritis
- Macular degeneration
- Diabetic foot ulcers
- Aromatic l-amino acid decarboxylase deficiency
- Ichthyosis
- GM1 gangliosidosis
- GM2 gangliosidosis (Tay-Sachs & Sandhoff disease)
- Achromatopsia
- Congenital adrenal hyperplasia
- Huntington’s disease
- Canavan disease
- Primary immunodeficiency
- Fanconi anemia
- Mucopolysaccharidosis I
- Mucopolysaccharidosis II
- Fabry disease
- Gaucher disease
- Cystinosis
- Phenylketonuria
- Pompe disease
- Parkinson’s disease
- Melanoma
- HIV-1 infection
- Overactive bladder
- Spinal muscular atrophy
- B-Cell lymphoma
- Mantle cell lymphoma
- Non-small cell lung cancer

Source: Navitus Health Solutions and American Society of Gene + Cell Therapy
Avalanche of Indication Approvals Expected

- 54-74 projected US cumulative product-indication approvals by end of 2030

Source: Drug Discovery Today https://doi.org/10.1016/j.drudis.2021.09.001
## Most Expensive Drugs in the US

<table>
<thead>
<tr>
<th>Drug Name</th>
<th>Price</th>
</tr>
</thead>
<tbody>
<tr>
<td>Elivaldogene autotemcel</td>
<td>$3,000,000</td>
</tr>
<tr>
<td>Tebentafusp-tebn</td>
<td>$975,520</td>
</tr>
<tr>
<td>Betibeglogene autotemcel</td>
<td>$2,700,000</td>
</tr>
<tr>
<td>Metreleptin</td>
<td>$929,951</td>
</tr>
<tr>
<td>Onasemnogene abeparvove</td>
<td>$2,125,000</td>
</tr>
<tr>
<td>Voretigene neparvovec-rzyl</td>
<td>$850,000</td>
</tr>
<tr>
<td>Lonafarnib</td>
<td>$1,073,760</td>
</tr>
<tr>
<td>Pralatrexate</td>
<td>$842,585</td>
</tr>
<tr>
<td>Naxitamab-gqgk</td>
<td>$1,011,882</td>
</tr>
<tr>
<td>Cerliponase alfa</td>
<td>$755,898</td>
</tr>
</tbody>
</table>

Source: Navitus Health Solutions
Significant Affordability and Access Concerns Anticipated

- Medicaid/Children’s Health Insurance Program Budget Impact Expected ~$1-4B
  - Ultra-orphan pediatric conditions
  - Genetic disorders

- Medicare Anticipated Budget Impact $9-16B
  - Oncology Medications
  - Musculoskeletal
  - Ophthalmological (wet AMD)

Source: Mark Trusheim. NEWDIGS Financing and Reimbursement of Cures in the US
Key Challenges

Predictability
- Treatment benefits
- Costs

Accessibility
- Patient Utilization
- Equity considerations/Centers of Excellence

Affordability
- High up-front costs
- Sustainability
Audience Polling: Which of the following is of greatest concern to your organization?

1. Predictability (Treatment benefits)
2. Predictability (Costs)
3. Affordability (High-up front costs)
4. Affordability (Timing of treatment benefits vs. costs)
5. Accessibility (Patient Access)
Stakeholder Prioritize Predictability, Affordability, and Accessibility in Different Ways
## Stakeholders Prioritize Challenges Differently

<table>
<thead>
<tr>
<th></th>
<th>Predictability</th>
<th>Affordability</th>
<th>Accessibility</th>
</tr>
</thead>
<tbody>
<tr>
<td>Patients</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Employers and Unions</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Health Systems</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Medicaid</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Medicare</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Regional commercial plans</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>National commercial plans</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Pharmacy benefit managers</td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

**Legend:** Low  Medium  High  Priority
No Single Financial Tool Will Meet All Stakeholder Needs
Multiple Tools Were Considered

- **Risk management approaches**
  - Stop-loss/Reinsurance
  - Orphan Reinsurer Benefit Manager
  - Risk-carve out

- **Performance-based**
  - Outcomes-based Contracts
  - Warranty
  - Annuity-based Contract with/without outcomes

- **Contract negotiations**
  - Contract Negotiation
  - Provider Contract Negotiation (e.g., Centers of Excellence)
  - Subscription
<table>
<thead>
<tr>
<th>Model</th>
<th>Predictability</th>
<th>Affordability</th>
<th>Accessibility</th>
</tr>
</thead>
<tbody>
<tr>
<td>Stop-loss</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Orphan reinsurer</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Risk-carve out</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Outcomes-based contracts</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Annuity with outcomes annuity</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Annuity</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Warranty</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Subscription</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Contract negotiation</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Provider contract negotiation</td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

*Participant Rating: Low Potential to Impact, Medium Potential to Impact, High Potential to Impact
*Terms/Details Matter*
<table>
<thead>
<tr>
<th>Model Type</th>
<th>Strengths</th>
<th>Challenges</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Stop-loss</strong></td>
<td>• Known,</td>
<td>• Recurring events not covered</td>
</tr>
<tr>
<td></td>
<td>• Works with random high-costs events (care accidents)</td>
<td>• Diminishes other value-contracting</td>
</tr>
<tr>
<td><strong>Orphan reinsurer</strong></td>
<td>• Pools risk</td>
<td>• Adds cost to system</td>
</tr>
<tr>
<td></td>
<td>• Ability to learn about Product value</td>
<td>• Lose ability to learn</td>
</tr>
<tr>
<td></td>
<td>• Reduced administrative Burden</td>
<td></td>
</tr>
<tr>
<td><strong>Risk-carve out</strong></td>
<td>• Requires carving out full condition</td>
<td>• Potential network challenges</td>
</tr>
<tr>
<td></td>
<td>• Reduced plan/employer burden</td>
<td>• Lose ability to learn</td>
</tr>
<tr>
<td></td>
<td>• Improved cost predictability</td>
<td></td>
</tr>
<tr>
<td><strong>Outcomes-based contracts</strong></td>
<td>• Shared risk between manufacturer</td>
<td>• Administrative burden</td>
</tr>
<tr>
<td></td>
<td>• Ability to collect real-world evidence</td>
<td>• Agreement on and ability to collect outcomes</td>
</tr>
<tr>
<td></td>
<td></td>
<td>• Ability to achieve outcomes may be dependent on care</td>
</tr>
<tr>
<td></td>
<td></td>
<td>• Patient portability challenges</td>
</tr>
<tr>
<td>Strengths</td>
<td>Challenges</td>
<td></td>
</tr>
<tr>
<td>-----------</td>
<td>------------</td>
<td></td>
</tr>
</tbody>
</table>
| **Annuity** | • Improved predictability  
  • Addresses high up-front costs  
  • Works when have good disease understanding |
|           | • Nor relevant for Medicaid  
  • Patient portability challenges |
| **Warranty** | • Manages Medicaid Best Price challenges  
  • Potential for patient out-of-pocket relief |
|           | • Requires third-party  
  • Would need a treatments with large enough population  
  • Ability to achieve outcomes may be dependent on care |
| **Subscription** | • Predictable  
  • Potential for a portfolio of products |
|           | • No impact on outcomes  
  • Need sufficient understanding of condition |
| **Contract negotiation and data management** | • Reduced staff time for analysis |
|           | • Requires finding the right third-party  
  • Reduced analytic learning |
| **Provider contract negotiation** | • Provider differentiation  
  • Potential for improved care for rare conditions through |
|           | • Breadth of centers of excellence or networks |
Uptake of New and Innovative Payment Models is Modest But Growing
Methodology and respondent demographics

• PRECISIONvalue recruited 40 respondents from a proprietary network of market access decision-makers.
• Respondents had responsibility or influence for decisions related to the financing of high-investment medications. The survey consisted of 15 questions and was fielded via the Qualtrics platform between April 1 and September 12, 2022.
Stop-loss Insurance is the Favored Model for High-investment Medications among both Payers and Employers

Payers have more likely to implement financial models, and to have plans in place to implement financial models in the future.

**Financial models for high-investment medications (payer)**

<table>
<thead>
<tr>
<th>Model</th>
<th>Currently use</th>
<th>Plan to implement within 18 months</th>
<th>Plan to implement beyond 18 months</th>
<th>No plans to implement</th>
</tr>
</thead>
<tbody>
<tr>
<td>Stop-loss/reinsurance</td>
<td>65%</td>
<td>10%</td>
<td>25%</td>
<td>5%</td>
</tr>
<tr>
<td>Provider contract negotiation</td>
<td>55%</td>
<td>10%</td>
<td>10%</td>
<td>25%</td>
</tr>
<tr>
<td>Risk carve-out</td>
<td>50%</td>
<td>15%</td>
<td>10%</td>
<td>25%</td>
</tr>
<tr>
<td>Outcomes-based rebates</td>
<td>45%</td>
<td>15%</td>
<td>25%</td>
<td>15%</td>
</tr>
<tr>
<td>Contract negotiation/management</td>
<td>45%</td>
<td>25%</td>
<td>30%</td>
<td></td>
</tr>
<tr>
<td>Performance/outcomes-based payments</td>
<td>40%</td>
<td>15%</td>
<td>25%</td>
<td>20%</td>
</tr>
<tr>
<td>Milestone-based contracts</td>
<td>35%</td>
<td>25%</td>
<td>20%</td>
<td>20%</td>
</tr>
<tr>
<td>Outcomes-based annuity</td>
<td>25%</td>
<td>25%</td>
<td>30%</td>
<td>20%</td>
</tr>
<tr>
<td>Annuity/amortization/installment</td>
<td>20%</td>
<td>15%</td>
<td>40%</td>
<td>25%</td>
</tr>
<tr>
<td>ORBM</td>
<td>20%</td>
<td>10%</td>
<td>25%</td>
<td>45%</td>
</tr>
<tr>
<td>Subscription model</td>
<td>10%</td>
<td>10%</td>
<td>20%</td>
<td>60%</td>
</tr>
<tr>
<td>Warranty</td>
<td>5%</td>
<td>25%</td>
<td>30%</td>
<td>40%</td>
</tr>
</tbody>
</table>

**Financial models for high-investment medications (employer)**

<table>
<thead>
<tr>
<th>Model</th>
<th>Currently use</th>
<th>Plan to implement within 18 months</th>
<th>Plan to implement beyond 18 months</th>
<th>No plans to implement</th>
</tr>
</thead>
<tbody>
<tr>
<td>Stop-loss/reinsurance</td>
<td>50%</td>
<td>5%</td>
<td>15%</td>
<td>30%</td>
</tr>
<tr>
<td>Provider contract negotiation</td>
<td>30%</td>
<td>5%</td>
<td>20%</td>
<td>45%</td>
</tr>
<tr>
<td>Contract negotiation/management</td>
<td>25%</td>
<td>15%</td>
<td>15%</td>
<td>45%</td>
</tr>
<tr>
<td>Performance/outcomes-based payments</td>
<td>20%</td>
<td>10%</td>
<td>10%</td>
<td>60%</td>
</tr>
<tr>
<td>Risk carve-out</td>
<td>20%</td>
<td>10%</td>
<td>10%</td>
<td>70%</td>
</tr>
<tr>
<td>Milestone-based contracts</td>
<td>10%</td>
<td>15%</td>
<td>10%</td>
<td>65%</td>
</tr>
<tr>
<td>Annuity/amortization/installment</td>
<td>10%</td>
<td>25%</td>
<td>60%</td>
<td>65%</td>
</tr>
<tr>
<td>ORBM</td>
<td>10%</td>
<td>10%</td>
<td>20%</td>
<td>85%</td>
</tr>
<tr>
<td>Subscription model</td>
<td>5%</td>
<td>10%</td>
<td>35%</td>
<td>55%</td>
</tr>
<tr>
<td>Performance/outcomes-based payments</td>
<td>5%</td>
<td>10%</td>
<td>10%</td>
<td>80%</td>
</tr>
<tr>
<td>Warranty</td>
<td>10%</td>
<td>10%</td>
<td>20%</td>
<td>70%</td>
</tr>
</tbody>
</table>

- **Currently use**
- **Plan to implement within 18 months**
- **Plan to implement beyond 18 months**
- **No plans to implement**

ORBM, Orphan Reinsurer and Benefit Manager n=20 payers; n=20 employers Sorted by “currently use”

Q: Does your organization currently utilize or plan to utilize any of the following financial models to support plan affordability for high-investment medications?

Confidential. Limited Distribution to AMCP Members. AMCP/PRECISIONvalue survey March 2022.
Both Payers and Employers Primarily Rely on Existing Partnerships to Execute Financial Models, but Employers are More Likely to Bring in a Third-party

n=20 payers; n=20 employers Q: As it relates to arrangements for financial models for high-investment medications, do you have a preference related to the entity you engage with for contract negotiation?

Confidential. Limited Distribution to AMCP Members. AMCP/PRECISIONvalue survey March 2022.
Multiple Policy Levers Were Considered

DATA, Enhancement, Streamlining and Exchange

- Patient incentives for disease registries
- Regulations to enable state pilots
- High-investment medication reinsurance after catastrophic
- National High-investment medication benefit
- High-investment medication risk pools
- Patient portability credits
- Mandatory value-based contracts for drugs above a $ threshold
Additional Takeaways

• Incremental changes needed now to prepare for the future impact

• Standardize terminology and education to provide awareness of new financial models and potential policy solutions

• Facilitate partnering and innovation across stakeholders
What’s Next?
Next Steps

https://www.amcp.org/Resource-Center/meeting-proceedings-findings/high-investment-medications
Key forum takeaways

• High-investment medications will raise sustainability and affordability concerns
• Stakeholders prioritize needs differently
• Uptake of new and innovative payment models is modest but growing
• No one tool meets all needs
• Incremental changes needed now
• Enhance the collection, sharing and use of data
• Standardize terminology and education
• Partnership and innovation across stakeholders is needed
**Mission**
To improve patient health by ensuring access to high-quality, cost-effective medications and other therapies.