Mortgages for Medical Care?  Paying for Cost-Effective Therapies with High Up-Front Costs

Steve Miller, MD
Chief Medical Officer, Express Scripts

June 13, 2018
New scientific approaches will lead to more novel but costly medications

Pipeline Products Using Gene-based Mechanisms

Conjugated Monoclonal Antibody
- 188

DNA & RNA Therapeutics
- 173

Gene Therapy
- 202

Cell Therapy (CAR-T)
- 529

Sources: Analysis Group, PhRMA
Fewer patients lead to higher prices

- **Glybera**: <10 patients
  - Estimated cost: $1,600,000

- **Luxturna**: 600 patients
  - Estimated cost: $1,400,000

- **Strimvelis**: <20 patients
  - Estimated cost: $1,200,000

- **Kymriah**: 300 patients
  - Estimated cost: $1,000,000

- **Yescarta**: 7,500 patients
  - Estimated cost: $800,000
First products reach the European market, but few patients receive treatment.

$1.4 million
Failed

$665,000
Money-back guarantee
Inherited retinal dystrophies: Leber Syndrome

Should they be denied care?
First U.S. CAR-T therapies approved for cancers

- **Cost: $475,000**
  - **Treats lymphoblastic leukemia**
    - Lethal blood and bone-marrow cancer
    - Affects children and young adults

- **Cost: $373,000**
  - **Treats large B-cell lymphoma**
    - Aggressive non-Hodgkin lymphoma
    - Indicated after other treatments fail
Gene therapy poses unique challenges

- 4,000 diseases linked to gene disorders
- High cost: $600k-$1.5M per patient
- Single administration
- Very small patient populations
- Durability periods vary

American healthcare system is ill equipped for this model
American healthcare system is ill equipped

It’s not built for one-time or periodic ultra-high-cost medication

Other challenges:

• Distribution
• Reimbursement
• Pricing / Sticker Shock
• Coverage
• Speed / Delays

• Affordability
• Portability
• Market Viability
• Durability / Effectiveness
Stakeholders have varying needs

- **Payers**
  - Patient management
  - Cost management
  - Ensured value
  - Payment mechanisms
  - Guaranteed outcomes

- **Patients**
  - Access
  - Affordability

- **Pharma**
  - Fair pricing
  - Return on investment
  - Coverage
New payment models under consideration

Lump Sum

Pooled Risk

Pooled Risk with Performance Guarantees

Periodic Payments

The right solution will enable collaboration among manufacturers, payers, patients, and policymakers
### Single Solution Provider

<table>
<thead>
<tr>
<th>Single Provider</th>
<th>Multiple Providers</th>
</tr>
</thead>
<tbody>
<tr>
<td>Simplicity</td>
<td>vs Complexity and Fragmentation</td>
</tr>
<tr>
<td>Defined Program Solution</td>
<td>vs Dozens of Custom Programs</td>
</tr>
<tr>
<td>Established Standards</td>
<td>vs Varying Standards</td>
</tr>
<tr>
<td>Single Party Contracting</td>
<td>vs 000’s of Contracts</td>
</tr>
<tr>
<td>Standardized Criteria and Evaluations</td>
<td>vs Fragmented and Varying Degrees of Accuracy</td>
</tr>
<tr>
<td>Streamlined Approvals</td>
<td>vs Multiple Handoffs and Processes</td>
</tr>
<tr>
<td>Established Player with Full Capabilities and Financial Stability</td>
<td>vs Increased Risk and Uncertainty for All Parties</td>
</tr>
<tr>
<td>Portability Programs are Possible</td>
<td>vs Portability Unlikely</td>
</tr>
</tbody>
</table>

#### Benefits
- **Payers**
  - Simplicity in contracting, approvals, plan design
  - Savings through improved management of resources
  - Accountability for results
- **Pharma**
  - Contracting simplicity
  - Improved coverage and payment predictability
  - Established coverage/contracting vs episodic one-offs
- **Patient**
  - Speed to therapy
  - Simplicity
  - Single source of support
## Financial Model Examples:

**Therapy: Product G**

**Price per administration:** $700k  
**Durability:** 5 years

<table>
<thead>
<tr>
<th>Payer Program Options</th>
<th>Price paid up front</th>
<th>Annual payments</th>
<th>Guarantee</th>
<th>Note</th>
</tr>
</thead>
<tbody>
<tr>
<td>No Program</td>
<td>$700k</td>
<td>n/a</td>
<td>None</td>
<td></td>
</tr>
<tr>
<td>Amortization Program</td>
<td>$400k</td>
<td>$60k annually</td>
<td>5 year durability, ESI tracks outcomes vs established criteria. Annual payments cease in the year outcomes fail to meet criteria.</td>
<td></td>
</tr>
<tr>
<td>Portability Program</td>
<td>$300k (up front is discounted $100k vs Amortization Program)</td>
<td>$60k annually</td>
<td>5 year durability, ESI tracks outcomes vs established criteria. Annual payments cease in the year outcomes fail to meet criteria.</td>
<td>Payer agrees to cover outstanding payments for patients transferring from other Portability Program Participating Payers</td>
</tr>
<tr>
<td>Escrow Program</td>
<td>$650k (up front is discounted $50k)</td>
<td>n/a</td>
<td>5 year durability, ESI tracks outcomes vs established criteria. Payer receives Annual payments cease in the year outcomes fail to meet criteria.</td>
<td>Manufacturer receives $300k up front, the balance is escrowed by ESI and paid to manufacturer at rate of $70k per year. If outcomes fail to meet criteria at any time, the balance is refunded to payer.</td>
</tr>
</tbody>
</table>
Key Takeaways

1. Pharmaceuticals are the scalpel of the future
2. Gene therapies are the next frontier in treatment
3. Paying for these new technologies will challenge us all
4. Payment models will need to be as innovative as the therapies