



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

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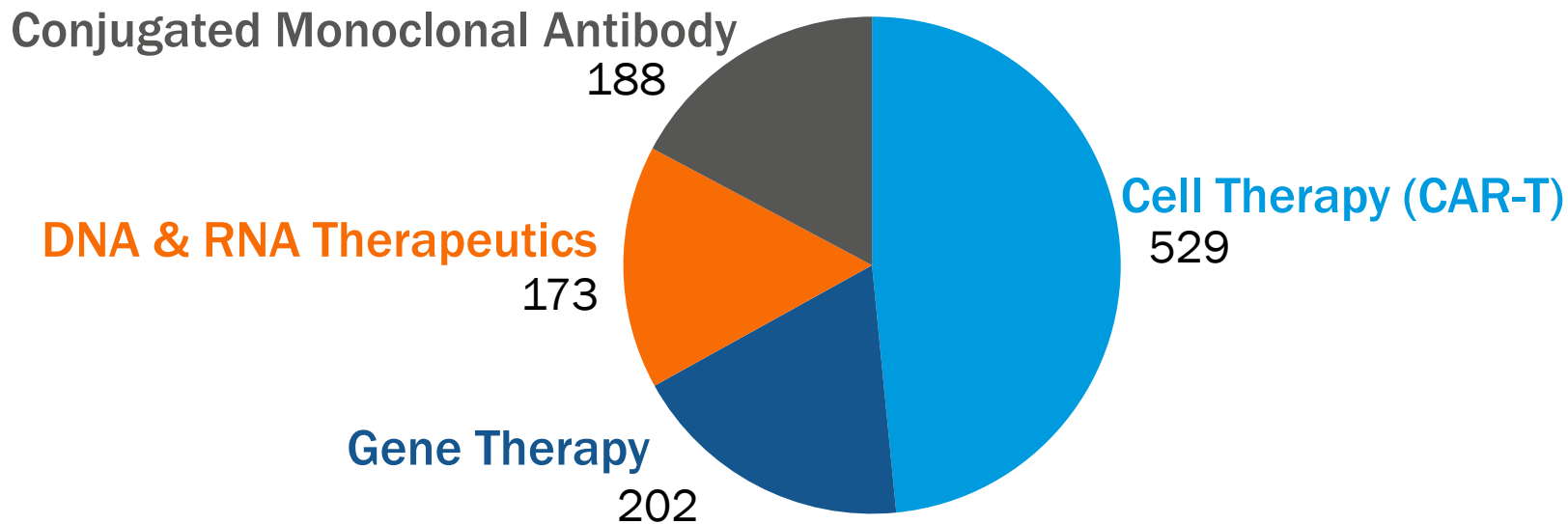
**Chief Medical Officer, Express Scripts**

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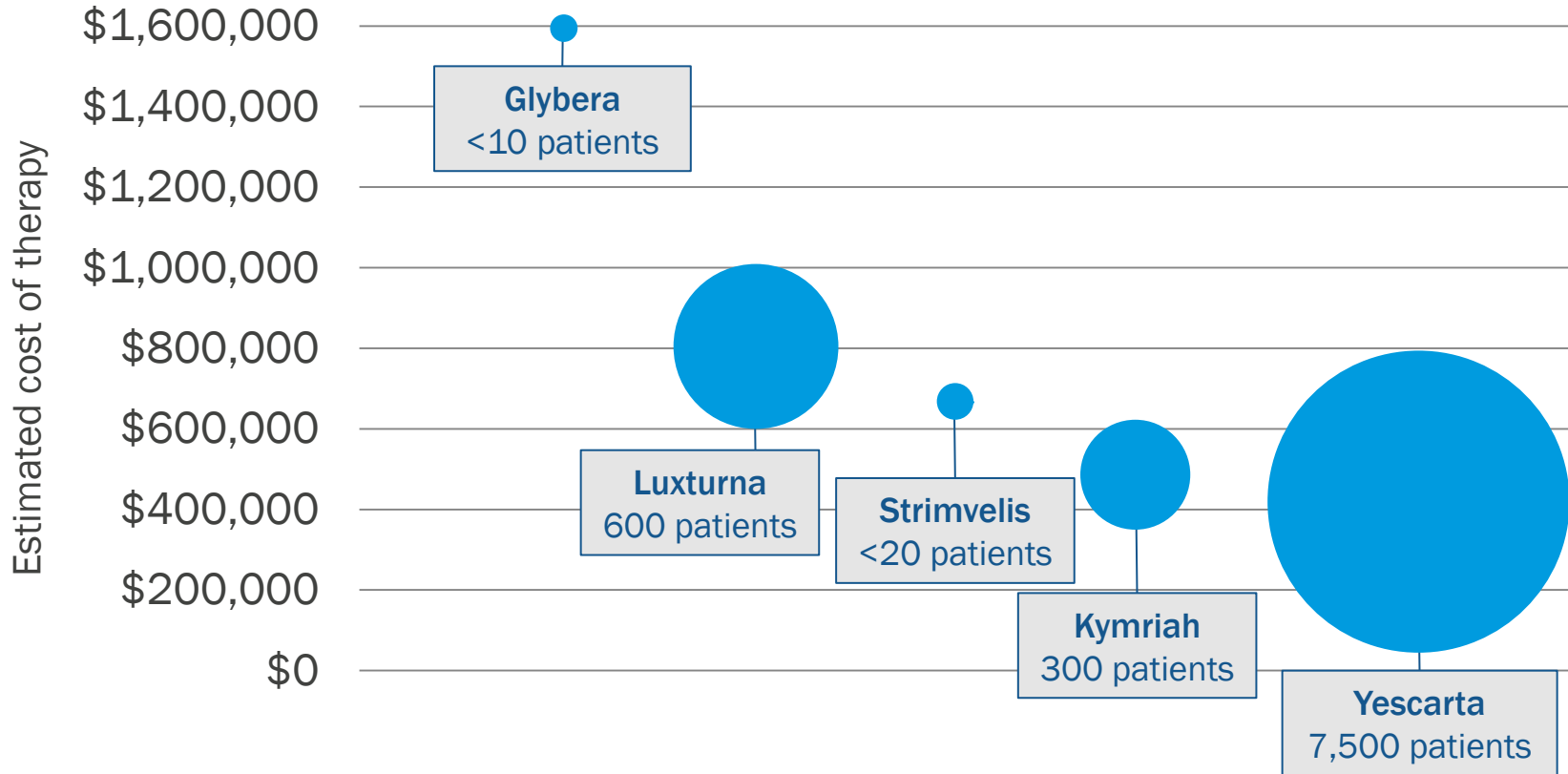
# New scientific approaches will lead to more novel but costly medications

## Pipeline Products Using Gene-based Mechanisms

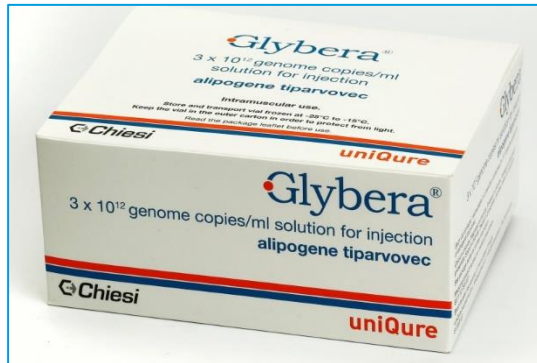


Sources: Analysis Group, PhRMA

# Fewer patients lead to higher prices



# First products reach the European market, but few patients receive treatment



\$1.4  
million

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Failed



\$665,000

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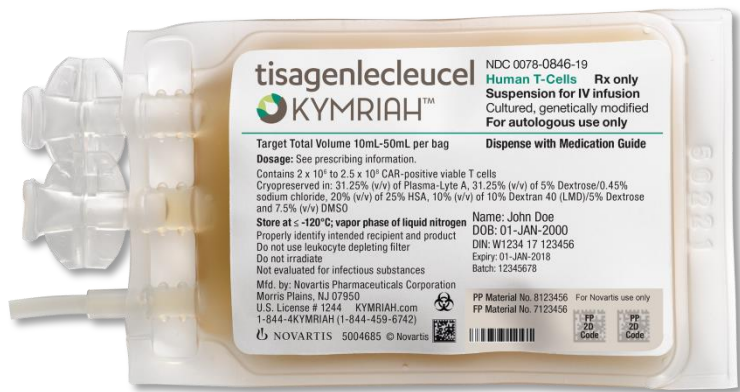
Money-back  
guarantee

# 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

 **YESCARTA™**  
(axicabtagene ciloleucel) Suspension  
for IV infusion

**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**

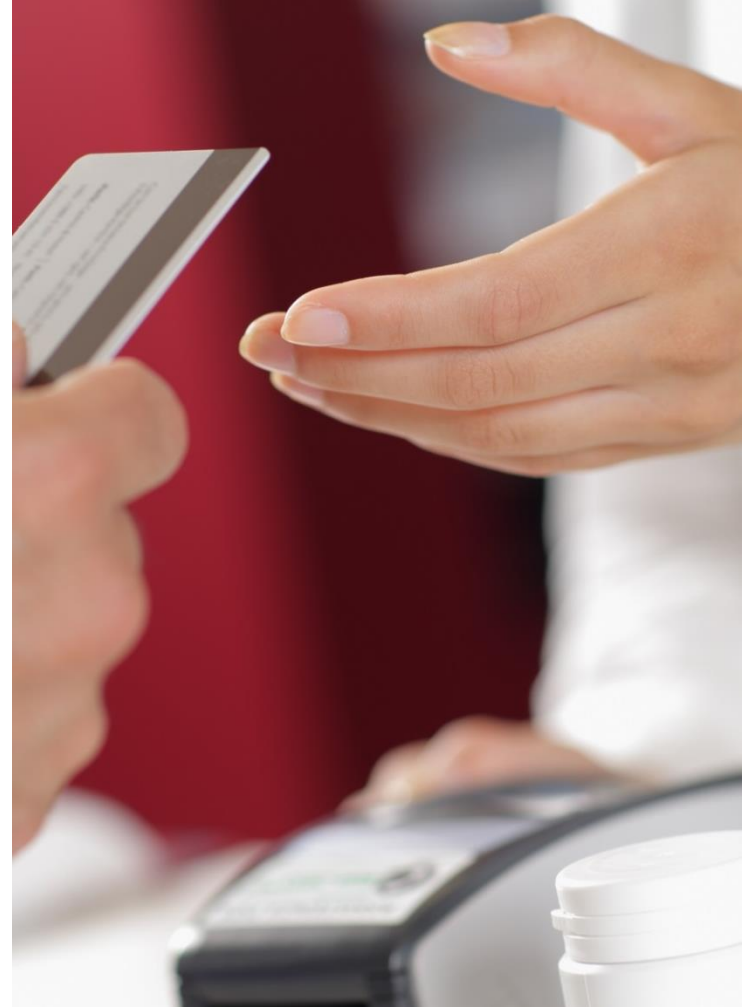


# American healthcare system is ill equipped

**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

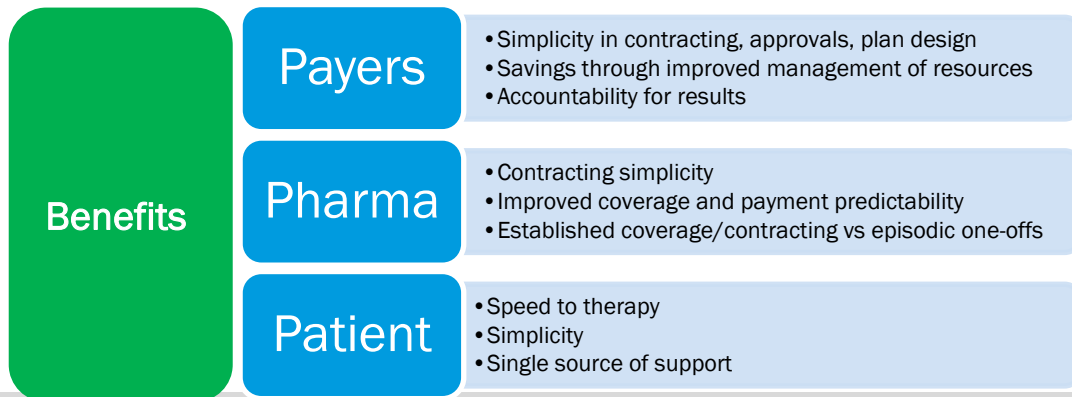
# New payment models under consideration



**The right solution will enable collaboration among manufacturers, payers, patients and policymakers**

# Single Solution Provider

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



# Financial Model Examples:

Therapy: Product G

Price per administration: \$700k

Durability: 5 years

Payer Program Options	Price paid up front	Annual payments	Guarantee	Note
No Program	\$700k	n/a	None	
Amortization Program	\$400k	\$60k annually	5 year durability, ESI tracks outcomes vs established criteria. Annual payments cease in the year outcomes fail to meet criteria.	
Portability Program	\$300k (up front is discounted \$100k vs Amortization Program)	\$60k annually	5 year durability, ESI tracks outcomes vs established criteria. Annual payments cease in the year outcomes fail to meet criteria.	Payer agrees to cover outstanding payments for patients transferring from other Portability Program Participating Payers
Escrow Program	\$650k (up front is discounted \$50k)	n/a	5 year durability, ESI tracks outcomes vs established criteria. Payer receives Annual payments cease in the year outcomes fail to meet criteria.	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.



# 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