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Preparing For the Future: The Rise of Gene and Cell Therapy and its Impact on the Medical Stop Loss Market

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PREPARING FOR THE FUTURE

The Rise of Gene and Cell Therapy and its Impact on the Medical Stop Loss Market

October 24, 2023
Gail Hardin & Charlotte Stabler

1. Introductions
2. What are gene and cell therapies?
3. Financial implications and risk transfer landscape
4. How employers are feeling and preparing

Agenda

Introductions

Today's Speakers

Gail Hardin, FSA, MAAA
Assistant Vice President
Accident & Health
Odyssey Reinsurance Company

Charlotte Stabler
Assistant Vice President, Treaty Broking
Life, Accident & Health Team
Guy Carpenter

What are gene and cell therapies?

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Gene and cell therapy definitions

Term	Definition
Cell Therapy	Transfer of modified live cells into the patient's body
• Autologous	Using cells from the same patient
• Allogenic	Using cells from donor patient
• CAR-T	Chimeric antigen receptor T-cell therapy
• TIL	Tumor infiltrating lymphocyte therapy (targets solid tumors)
Gene Therapy	Addition of new genes into a patient ("gene addition"), or the edit or removal of existing genes
In-Vivo Therapy	Therapies done inside the body – genes or cells modified directly within the body
Ex-Vivo Therapy	Therapies done outside the body – modifications done outside of the body and reinserted back into the patient
Biologic License Application ("BLA")	Request to introduce, or deliver for introduction, a biological product into interstate commerce. The FDA has 90 days from submission of BLA to accept or request additional information. (This is the process of submitting clinical trial data to FDA)
Prescription Drug User Fee Act ("PDUFA")	Once FDA accepts BLA, a PDUFA date is set by the FDA and is provided to manufacturer. This is the date by which the FDA must respond to the BLA – either to approve product, reject product, or request more information.

Gene and cell therapies pose unprecedented cost challenges for plan sponsors

How these therapies work

- *Gene therapies* replace missing or faulty genes, add genes to help the body treat a disease, or turn off genes that are causing a disease
- *Cell therapy* modifies cell function allowing cells to respond to a disease (ex: giving immune cells the ability to fight cancer cells)
- Complex administration process; must be given in specially designated treatment facilities; typically billed through medical benefit

Diseases currently treated

- Gene therapy *currently* targets rare genetic conditions
- Cell therapy targets cancers (primarily blood cancers)

Safety and efficacy

- Tested for safety and efficacy by the FDA prior to approval
- Long term data limited; durability may not be known

Higher price tags

Near or exceed $1M per single treatment course

Less predictable utilization

Typically given as one-time potentially curative treatment

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What makes gene and cell therapies unique?

	Specialty Drugs	Gene and Cell Therapy
Cost	Average annual cost **~$85K - $135K**	One-time cost of **~$250K - $3M+**
Outcomes	**Control or maintenance**	**Cure or sustained response**
Administration	**Self-administered** and/or administered in a **specialized setting**	Must be given in **highly specialized setting** due to additional complexity
Benefit	**Medical and/or pharmacy**	**Typically medical**
Breadth of conditions	**Broad** – wide array of indications	**Limited** – currently targeting rare diseases and cancers
Population applicability	**Wider**	**Narrower**

Key gene and cell therapies on the market

As of 09/15/2023

Drug *	Condition	Type	Manufacturer	Cost **
ABECMA	Multiple myeloma	Cell	Celgene Corporation, a Bristol-Myers Squibb Company	$419,500
ADSTILADRIN	Bladder cancer	Gene	Ferring Pharmaceuticals A/S	Unable to determine
BREYANZI	Large B-cell lymphoma	Cell	Juno Therapeutics, Inc., a Bristol-Myers Squibb Company	$410,300
CARVYKTI	Multiple myeloma	Cell	Janssen Biotech, Inc.	$465,000
ELEVIDYS	**Duchenne muscular dystrophy**	**Gene**	**Sarepta Therapeutics, Inc.**	**$3,200,000**
GINTUIT	Mucogingival conditions	Topical	Organogenesis Incorporated	Unable to determine
HEMGENIX	**Hemophilia B**	**Gene**	**CSL Behring**	**$3,500,000**
IMLYGIC	Lesions resulting from melanoma	Topical	BioVex, Inc., a subsidiary of Amgen Inc.	$65,000
KYMRIAH	Follicular lymphoma	Cell	Novartis Pharmaceuticals Corporation	$475,000
LANTIDRA	Type 1 diabetes	Cell	CellTrans Inc.	$300,000
LAVIV	Nasolabial fold wrinkles	Cell	Fibrocell Technologies	$3,000
LUXTURNA	**Retinal dystrophy**	**Gene**	**Spark Therapeutics**	**$425,000 per eye**
MACI	Knee/ joint defects	Cell	Vericel Corp.	$40,000
OMISIRGE	Blood cancers	Cell	Gamida Cell Ltd.	$338,000
PROVENGE	Prostate cancer	Cell	Dendreon Corp.	$93,000
RETHYMIC	**Congenital athymia**	**Cell**	**Enzyvant Therapeutics GmbH**	**$2,700,000**
ROCTAVIAN	**Hemophilia B**	**Gene**	**BioMarin Pharmaceutical Inc**	**$2,900,000**
SKYSONA	Cerebral adrenoleuko-dystrophy	Gene	blubird bio, Inc.	$3,000,000
STRATAGRAFT	Burns	Topical	Stratatech Corporation	Unable to determine
TECARTUS	Acute lymphoblastic leukemia	Cell	Kite Pharma, Inc.	$373,000
VYJUVEK	Dystrophic epidermolysis bullosa	Topical	Krystal Biotech, Inc.	$631,000 per year
YESCARTA	Large B-cell lymphoma	Cell	Kite Pharma, Inc.	$373,000
ZYNTEGLO	**ß-thalassemia**	**Gene**	**bluebird bio, Inc.**	**$2,800,000**
ZOLGENSMA	**Spinal muscular atrophy**	**Gene**	**Novartis Gene Therapies, Inc.**	**$2,100,000**
Cord Blood Therapies			Multiple	
ALLOCORD (SSM Cardinal Glennon Children's Medical Center), CLEVECORD (Cleveland Cord Blood Center), DUCORD (Duke University School of Medicine), HEMACORD (New York Blood Center), HPC (Clinimmune Labs, Univ. of Colorado Cord Blood Bank), HPC (MD Anderson Cord Blood Bank), HPC (LifeSouth Community Blood Centers, Inc.,) HPC (Bloodworks)				

Source: FDA

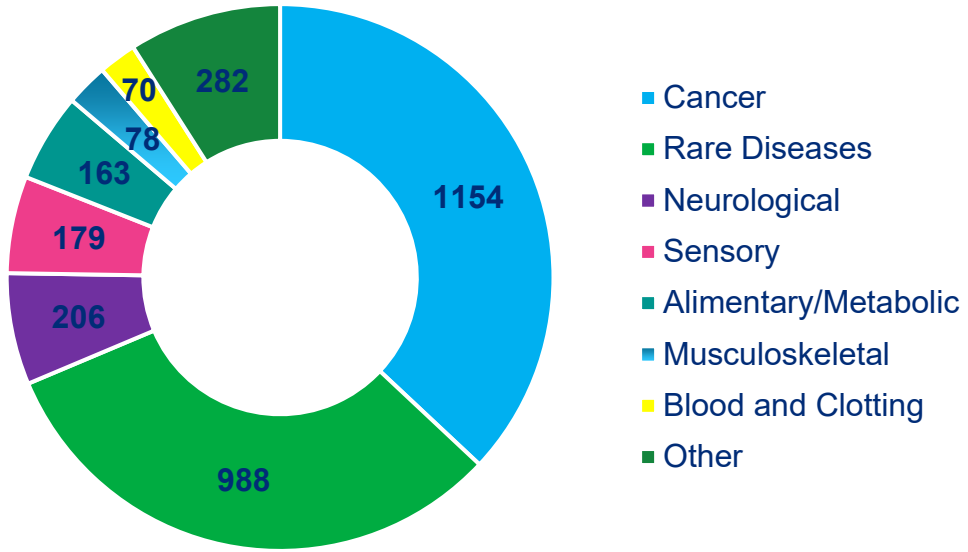
*Note: all drugs are trademarked or have copyright by their respective manufacturers

**Drug costs reflect approximate wholesale list prices. Sources are listed in appendix. Listed prices do not include any administration costs associated with therapies.

Robust gene and cell therapy pharmaceutical pipeline may yield a wave of new products in the coming years

By 2025, the FDA anticipates that it will review and potentially approve 10-20 gene and cell therapies per year[1]

Number of Therapies in Development[2]



[1]Statement from FDA Commissioner Scott Gottlieb, M.D. and Peter Marks, M.D., Ph.D., Director of the Center for Biologics Evaluation and Research on new policies to advance development of safe and effective cell and gene therapies | FDA
[2]Gene, Cell, and RNA Therapy Landscape. Q4 2022 Quarterly Data Report. American Society of Gene and Cell Therapies.

Financial Implications and the Risk Transfer Landscape

Gene and cell therapy will have a significant financial impact to individuals, employers, and stop loss carriers

$25B

Annual cost by 2025

$300B

Total estimated cost from 2020 - 2035

1M+

Individuals

Eligible for treatment within 12 years

$3.5M+

Cost for Hemgenix therapy

48.6%

Cost allocated to Private insurance

4k+

Therapies in development as of mid-2023

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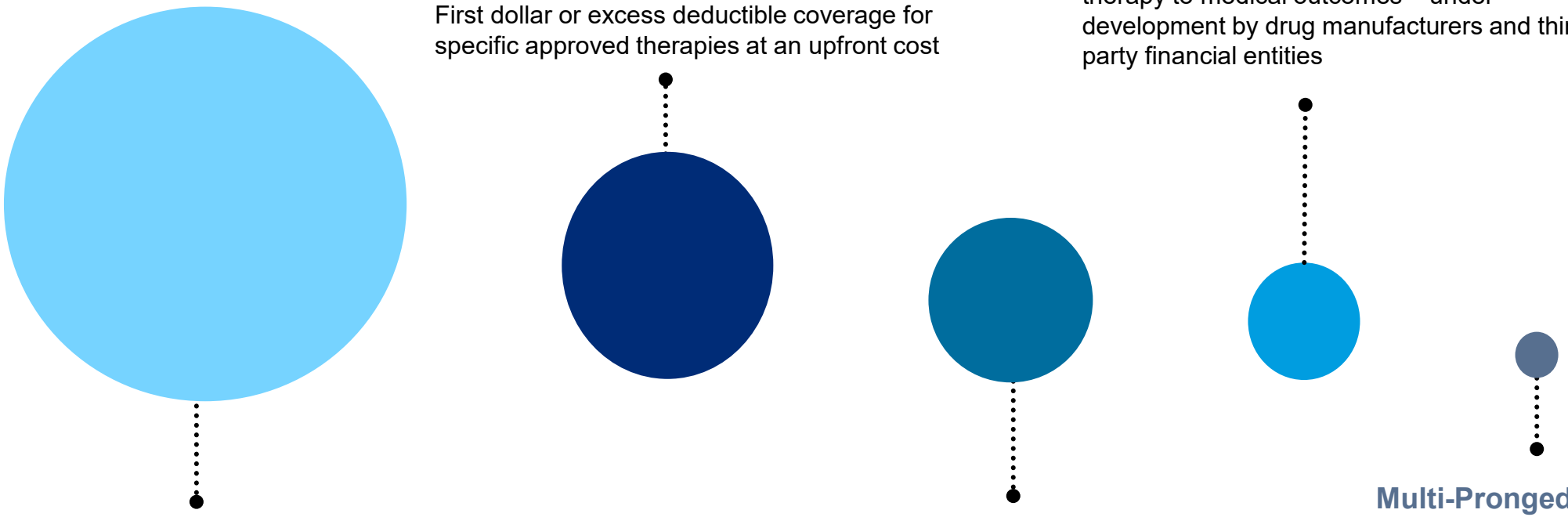
Estimating the Financial Impact of Gene Therapy, medRxiv
Research conducted by Marsh & McLennan Agency (MMA) Rx Solutions
Gene, Cell, and RNA Therapy Landscape. Q4 2022 Quarterly Data Report. American Society of Gene and Cell Therapies.

Current risk transfer market landscape



Employer Stop Loss

Nearly all employer stop loss carriers currently provide gene & cell therapy coverage for approved and pipeline therapies within standard policy forms

Listed Therapy Carve-Out

Zolgensma, Luxturna, Spinraza, etc.

First dollar or excess deductible coverage for specific approved therapies at an upfront cost

Carve-Out Approved and Pipeline Therapies

First dollar or excess deductible coverage for approved and pipeline therapies at an upfront cost

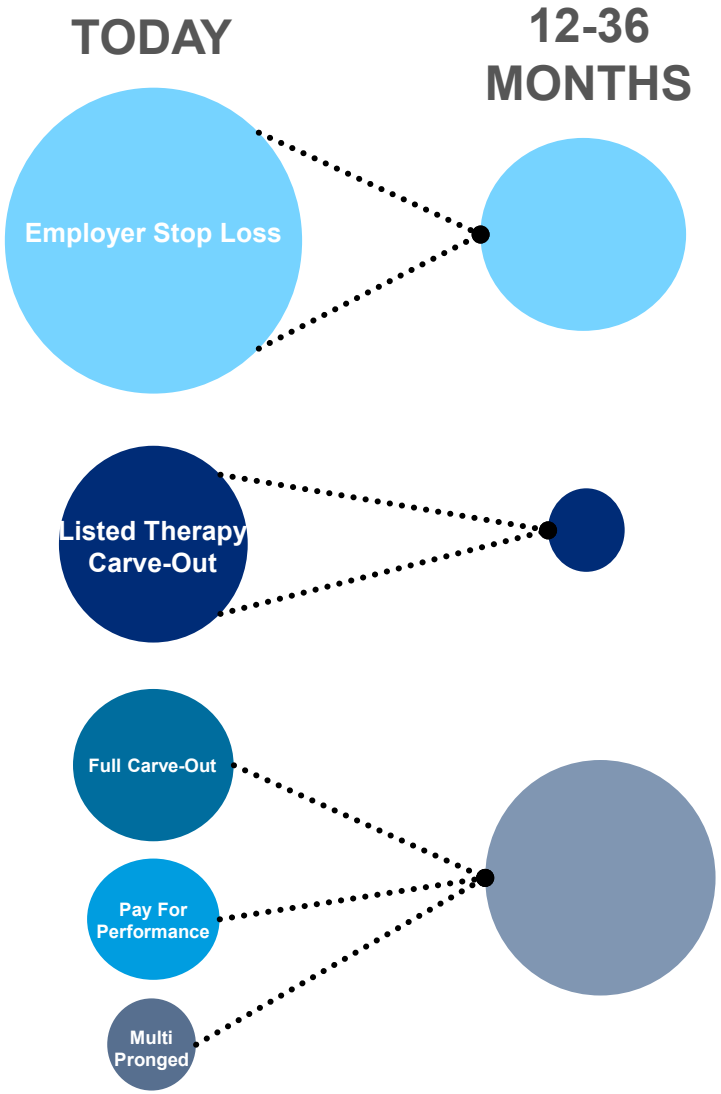
Pay For Performance

Financial mechanism that ties ultimate cost of therapy to medical outcomes – under development by drug manufacturers and third party financial entities

Multi-Pronged Solutions

Attempts to address wide-range of common issues including portability, cost spreading over time, and pay for performance

Anticipating the future risk transfer market landscape



Employer Stop Loss

As additional high-cost therapies are approved, carriers may begin excluding coverage – or pricing coverage such that a market clearing price is not achieved. Expect continued innovation around coverage options in this market.

Listed Therapy Carve-Out

Reduced demand for specific drug carve-outs as more complete solutions enter the market. Drug developers and manufacturers may offer therapy-specific cost management products.

Alternative Cost Management Solutions

Development of new solutions addressing multiple facets of gene & cell therapy risk:

- Structured / Financial mechanisms to spread cost over time
- Pooling arrangements to spread risk amongst multiple parties (payers, employers, reinsurers)
- Value Based Care contracts aligning drug cost with medical outcomes
- Portability options protecting payers from members who receive a therapy and subsequently move plans
- Government-sponsored coverage (e.g. state carve-outs for Medicaid, 1332 Waivers, high risk health pools, etc.)

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Size of bubble represents estimated number of available risk transfer companies

How are employers preparing?

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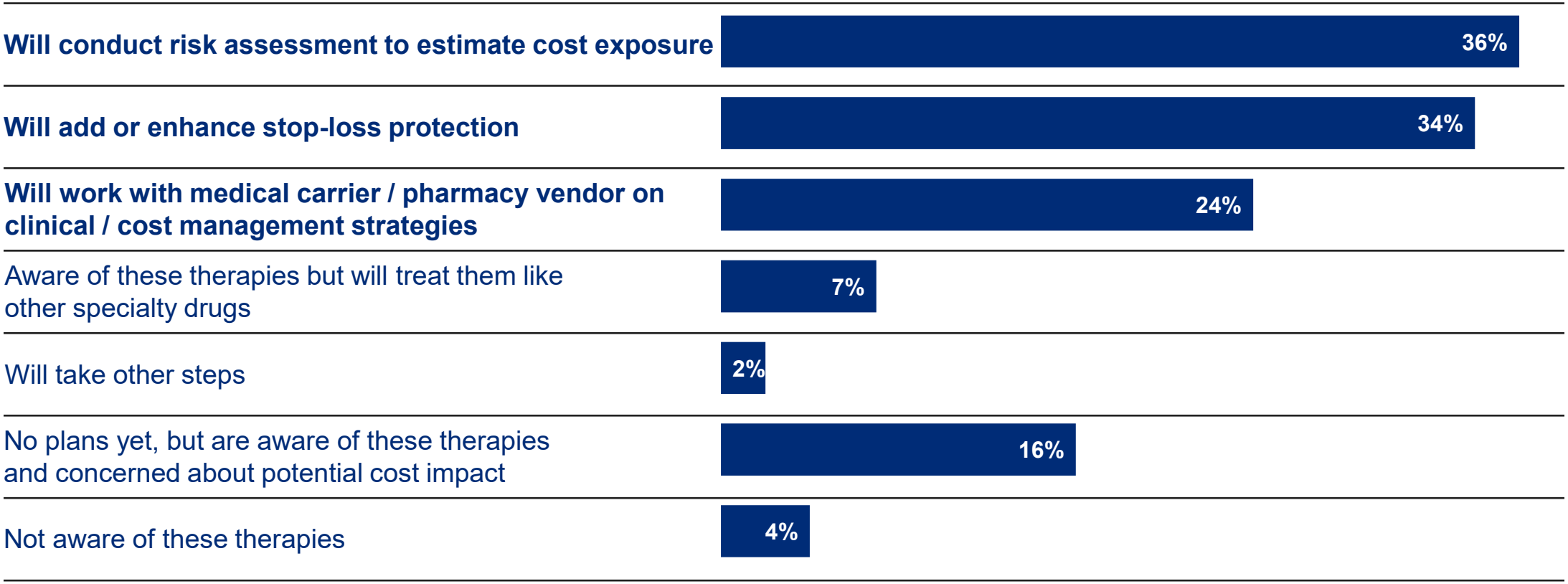
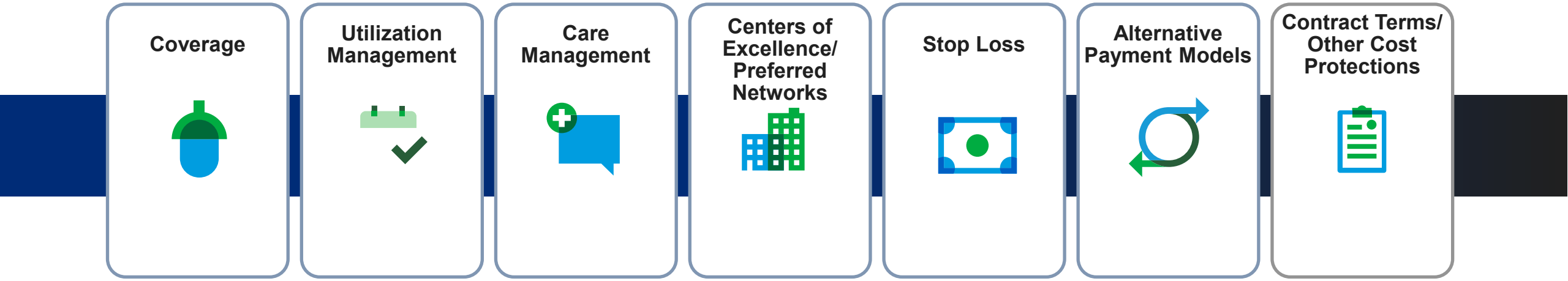
How plan sponsors are preparing for the potential cost impact of gene and cell therapies



Employers with 500 or more employees

Employers need to explore the full spectrum of vendor strategies



Key Takeaways and Questions

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Key Takeaways

1. Gene and cell therapies can have a significant financial impact to self-insured employers
2. Future FDA pipeline is extensive and focused on more common genetic disorders
3. Stop loss market currently provides gene and cell coverage but alternative risk financing strategies will likely be needed in the near future
4. Employers will play an active role in ensuring employees continue to receive access to these life saving therapies

Questions