



## 5 steps to help you prepare for a data-driven discussion on gene therapy

Gene therapies have life-changing potential, but they can be complicated to understand and sometimes stressful for plans that are making proactive, informed decisions on coverage strategies. We'll guide you to a better understanding of gene and cell therapies by:

- + Looking ahead to what's coming in the pipeline
- + Giving you the support you need to move forward
- + Guiding your organization's leaders in making informed decisions for your plan

See the following pages for **five steps** you can take to prepare for a data-driven discussion on a gene therapy coverage strategy.



[Visit the Gene Therapy Learning Center](#)

to learn about updates to the gene therapy landscape, access new content and sign up for a pipeline report.



## STEP 1

# Educate yourself about gene therapies

With so much complex, highly clinical information out there on gene therapies, we've curated and consolidated some key resources for you.

- + [Gene Therapies 101](#): Answers to the most frequently asked gene therapy questions
- + [A Brief History of Gene Therapy](#): How we got here and where drug innovation is going
- + [Gene Therapy vs. Cell Therapy](#): Understanding the differences between these types of therapies and why it matters
- + [Recognizing Patient Challenges](#): The pressures and stressors faced by patients with rare and complex conditions
- + [Near-Term Gene and Cell Therapy Pipeline Overview](#): Review the chart below to understand the speed and scope at which these condition-specific therapies are projected to enter the market
- + [Gene Therapy Learning Center](#): Get updates on the gene therapy landscape and sign up for a pipeline report



**1,000+** gene and cell therapies currently in development

SOURCE :


<https://milkeninstitute.org/report/cell-gene-therapies-2022>


## GENE AND CELL THERAPY PIPELINE AS OF AUGUST 2023


2023


2024


2025


 **(RPL-201)**  
Leukocyte adhesion deficiency


 **Upstaza**  
(*eladocagene exuparvovec*)  
AADC deficiency


 **prademagene zamikeracel**  
(*EB-101*) Dystrophic epidermolysis bullosa


 **afamitresgene autoleucl**  
(*afami-cel*) sarcoma


 **D-Fi**  
(*dabocemagene autoficel*)  
Epidermolysis bullosa


 **dirloctocogene samoparvovec**  
(*SK-8011*; *RG6357*)  
Hemophilia A

 **Engensis**  
(*donaperminogene seltoplasmid*)  
Diabetic foot ulcers


 **Obe-cel**  
(*obecabtagene autoleucl*)  
Acute lymphoblastic leukemia

 **pariglasgene brecaparvovec**  
(*DTX401*) Glycogen storage disease type 1

 **X-SCID Gene Therapy**  
(*MB-107*) X-linked severe combined immunodeficiency


 **Ryoncil**  
(*remestemcel-L*)  
Graft-vs-host disease

 **(MB-106)**  
Non-Hodgkin's Lymphoma

 **(OTL-203)**  
Mucopolysaccharidosis type 1

 **giroctocogene fitelparvovec**  
(*SB-525*) Hemophilia A

 **(XC001)**  
Angina

 **(AAV-RPGR)**  
(*botaretigene sparoparvovec*)  
Retinitis pigmentosa

THERAPY CLASSES



ONCOLOGY



CARDIOVASCULAR



HEMATOLOGY



RHEUMATOLOGY



TRANSPLANT



METABOLIC DISORDERS



DIABETES



OPHTHALMOLOGY



IMMUNOLOGY



DERMATOLOGY



NEUROLOGY



GENETIC DISORDERS



STEP 2

## Understand your coverage options and the decisions you'll need to make





There's a wide spectrum of thoughts regarding coverage strategies for gene therapy. At one end, your plan may choose not to cover gene therapies at all.

While this choice may offer predictability and affordability, it leaves members and their families on their own to cover these costly treatments—sometimes when there are no other or very limited treatment alternatives.

At the other end, your plan could select a per-member-per-month solution (PMPM). These solutions offer a predictable PMPM cost structure, with more drugs and therapies to be added in the future, and can ensure no copay to the patient. But not all PMPM solutions include protection—via no copay to the patient—which can make these therapies unaffordable to families, even when your plan has good intentions in making a coverage decision.

You can also consider stop-loss-based solutions. However, these may prove to be hazardous to your plan and members—rates are not held in place, so you risk them going up as more drugs enter the market. And even more concerning, not all stop-loss solutions cover the expense of gene therapies.

### AVAILABLE COVERAGE STRATEGIES FOR GENE THERAPIES

	Per-member-per-month solution	Specialty pharmacy pay over time	Traditional stop-loss coverage	Client pays out of pocket	Excluding gene therapy from coverage
 <b>Access</b>	✓	✓	✓	✓	
 <b>Affordability for patient</b>	✓*				
 <b>Affordability for client</b>	✓	✓	✓		✓
 <b>Predictability</b>	✓	✓			✓

\*Be sure to check that the PMPM solution you're evaluating offers cost protection for your members



STEP 3

# Utilize sophisticated modeling tools to understand the pipeline and its impact to your plan

Developing a well-informed approach up front is critical for your plan. Although any huge impact may be years off, if you're not considering your organization's strategy on gene therapy, you could be caught by surprise.

Plan-specific forecasting can help you get started. However, some forecasting methods are more helpful than others as you prepare. Our forecasting method with a detailed methodology will best achieve realistic estimates on drug costs for gene therapies in the pipeline.

Many forecasting tools available today use disease prevalence and incidence data only. Instead, consider a more sophisticated modeling tool that incorporates assumptions around actual utilization and is based on clinical trial information and potential approved indication parameters.

Not every approved gene therapy will be appropriate for every patient, especially for those therapies serving larger populations. Each drug needs an evaluation that also considers patient demographics, disease severity, clinical trial data and literature, and client-specific mix. When you utilize these parameters during a forecasting exercise, and allow for dynamic client-specific inputs, the output summarizes potential impact at the pipeline drug level while incorporating client-specific detail. In other words—you'll get a highly customized report, tailored to your organization. See the differences in modeling strategy in the table below.

## ACCURATE PREDICTIONS NOW MAY SAVE YOU COSTLY PAIN LATER

### TYPICAL MODELING TOOLS

Some marketplace estimates are broad, often based purely on disease prevalence



 **Disease prevalence**





**OUTCOME: BROAD ESTIMATE**

### SOPHISTICATED MODELING TOOLS



Sophisticated tools create layers of adjustment to provide a finely tuned estimate of potential gene therapy utilization

 **Pipeline evaluation** 



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 **Disease prevalence and patient demographics** 



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 **Clinical trial data and literature** 

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 **Drug-specific requirements** 

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 **Client-specific mix** 

**OUTCOME: MORE ACCURATE ESTIMATE**



### Not all specialty pharmacies have access to and experience with gene therapies.

Accredo, part of Evernorth, is the only specialty pharmacy with access to the majority of approved gene replacement therapies to date—and one of the only pharmacies with experience in handling, packaging and shipping these sensitive medications. To deliver a better payer, patient and physician experience, Accredo has implemented innovative operational technologies and a dedicated GeneAXS™ team to support the needs of gene therapies.



STEP 4

## Leverage your PBM or specialty pharmacy account team to help you prepare

**Your PBM or specialty pharmacy Account Executive can help you forecast prevalence within your plan and prepare for the conversation with your leaders.**

Forecasting can be further customized by a specific gene therapy and by use of your plan's medical data. For example, if you have additional information, such as medical diagnostic numbers for your member population, it will help us fine-tune your forecast even further.

Additionally, there are other decisions to make about gene therapy benefits beyond coverage approach, including which channel (medical or pharmacy) you want to cover each therapy under and what specialty pharmacy (or pharmacies) you want supporting these costly therapies and unique patients.





## STEP 5

# Connect your coverage strategy recommendation to your enterprise strategy, key metrics and goals

Planning for gene therapy coverage can help you meet your organization's goals. Identify your organization's primary goal and make your recommendation accordingly.

Review the goals below to help you get started.



### Is your goal better cost management?

Planning now for your approach to gene therapies will help you manage costs more predictably in the future.



### Is your goal advancing health care and access to health care?

Making a coverage plan for gene therapies can help support health care technology that can engineer bodies to fight cancer and reverse rare diseases—all while giving hope to so many who didn't have it before.



### Is your goal competitive benefits?

In today's competitive workforce, benefit plans are an increasingly important part of compensation considerations. Including gene therapy coverage in your benefit offering can help set your organization apart by illustrating your deep commitment to the health of your employees and their families.



**Taking these first steps will help you be better prepared as an organization and set the stage to provide a better experience and important drug access for your members and their loved ones.**

By using the information available to you—including conversations with our experts—you can begin developing a clear vision for how you want to approach gene therapy coverage.

In the coming years, your plan will need protection from bills for million-dollar therapies, while your members will expect these drugs to become more accessible and affordable. **We're here to help you find the right solution for your organization.**



# Evernorth Embarc Benefit Protection<sup>®</sup>

As the industry's first solution to build a pathway to pay for the coming wave of expensive and potentially curative therapies, Embarc Benefit Protection is changing the future of health care.

Embarc Benefit Protection shields members from the high costs of gene therapies, and assures that people who need these treatments can get them by offering:



Financial protection for high-cost therapies



Access to quality, cost-effective, in-network providers



Support from a dedicated gene therapy case management team



For 99 cents per member per month, an employer with 1,000 lives would have a monthly payment of \$990, with zero cost to members. If one employee needed gene therapy, the employer would ensure access to that therapy while paying virtually nothing against the cost of a **potential \$2M claim**.

Learn more about [Embarc Benefit Protection](#).