MAY 2026

Pipeline Therapies to Watch – 2026

What's Next in Gene and Cell Therapy & Specialty Drugs

Points to Note in 2026

High-cost biologic, gene, and cell therapies remain drivers of catastrophic claim risk.

Expanding patient populations and earlier-line use are increasing claim frequency.

Rapid approvals and a robust pipeline signal multimillion-dollar therapy approvals through 2027.

Broader Trends and Pipeline Dynamics[1-5]

Continued Shift Toward Highly Specialized Therapies

The drug landscape in the United States continues to shift rapidly toward highly specialized, high-cost therapies, particularly biologics and cellular and gene therapies. In 2024, the U.S. Food and Drug Administration (FDA) cleared 61 novel medicines, and more than 40% were first-in-class treatments often for rare or complex diseases.

This trend persisted through 2025 and was anchored by breakthroughs for ultra-rare conditions in neurology, metabolic disorders, and oncology. The pipeline remains rich in gene therapies and other advanced therapeutics that promise life-changing outcomes at multimillion-dollar price points (not including ancillary expenses like hospital and site-of-care costs).

Broader Patient Populations and Label Expansions

Unlike the first gene and cell therapies that targeted only a few hundred patients nationwide, newer approvals increasingly address conditions with tens of thousands of affected individuals, such as Encelto™, which was approved in 2025 for patients with Macular Telangiectasia Type 2, an eye disorder that affects an estimated 150,000+ patients in the U.S.

Similarly, next-generation cell therapies (like CAR-T cancer treatments) are being tested and approved in earlier lines of therapy. This significantly increases the number of potential eligible patients per therapy. For example, a CAR-T once reserved for last-resort use in refractory cancer may now be used in second-line treatment, which multiplies the potential claim frequency, increasing the spend, even if the per-treatment cost holds steady.

Expanded indications for existing high-cost drugs also are fueling greater utilization. In fact, healthcare plans are seeing a bigger cumulative cost impact from new uses of existing therapies (e.g., when the treatment moves from later lines of care into front-line therapy or when new disease indications are added) than from brand-new product launches alone.

Approvals More Rapid Than Management Tools

Drug innovation cycles are accelerating, with many "breakthrough" therapies reaching market within six to ten months of application. In several instances, utilization management protocols and pricing benchmarks lag behind these swift approvals. This dynamic leaves self-funded employers scrambling to adapt coverage policies and manage patient access on the fly.

Mid-year approvals can be particularly challenging. When a blockbuster therapy is launched just after a plan year begins, it may not be built into Stop Loss premiums or internal projections. This can lead to unanticipated catastrophic claim exposure, reinforcing the importance of proactively monitoring the pipeline and developing forward-looking risk strategies.

Accelerated Adoption, Rising Costs

Initial utilization of new therapies may appear limited, but experience shows that adoption often ramps quickly as providers gain confidence and the treatment capacity expands. What begins as an isolated, one-off claim can evolve into a recurring expense category over several years, materially increasing the long-term trend risk for self-funded plans.

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What's on the Horizon[5-7]

The development pipeline shows no signs of slowing. Multiple high exposure therapies are expected to reach the market in late 2026 and 2027, including gene therapies for rare metabolic and neurodegenerative disorders and allogeneic, off-the-shelf cell therapies for previously untreatable conditions.

Looking ahead, there currently are several therapies with potential approval dates through the third quarter of 2026:

- DTX401 – a one-time gene therapy for Glycogen Storage Disease Type 1a
- Deramiocel – an allogeneic, off-the-shelf cellular therapy for the treatment of cardiomyopathy associated with Duchenne Muscular Dystrophy
- UX111 – a one-time gene therapy for Mucopolysaccharidosis Type IIIa (Sanfilippo Syndrome)

Other rare disease states with potential high-cost therapies up for approval in 2026 into early 2027 include:

- Ornithine Transcarbamylase (OTC) Deficiency – a rare disease that results in toxic ammonia build-up in the body that can lead to brain damage and death
- Retinitis Pigmentosa – a rare, genetic eye disorder that leads to progressive vision loss
- Fabry Disease – an inherited disorder where the deficiency of a specific enzyme (alpha-GAL) results in damage to major organs like the kidneys and heart

Each of these possible therapies carries the potential for multimillion-dollar claims, and some of these diseases affect larger patient populations, which compounds the total financial exposure beyond what historically has been seen with treatments for ultra-rare conditions.

Breakthroughs in gene, cell, and specialty therapies will continue to reshape the cost profile of self-funded health plans in 2026 and beyond. Broader adoption, expanded indications, rapid approval timelines, and cumulative cost effects are driving heightened Stop Loss risk. Employers that remain proactive by monitoring late-stage pipelines, aligning coverage policies, and ensuring they have appropriate Stop Loss protection in place will be best positioned to balance access to life-changing treatments with long-term financial sustainability.

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High-Cost Therapies Pipeline[5-7]

Disease	Therapy*	Anticipated FDA Review Timing
Leukemia	Orca-T	7/6/2026
Duchenne Muscular Dystrophy	CAP-1002 (deramiocel)	8/22/2026
Glycogen Storage Disease Type 1	DTX401 (pariglasgene brecaparvovec)	8/23/2026
Mucopolysaccharidosis Type IIIA (Sanfilippo Syndrome)	UX111 (rebisufligene etisparvovec)	9/19/2026
Ornithine Transcarbamylase Deficiency	DTX301 (avalotcagene ontaparvovec)	Second Half of 2026
Retinitis Pigmentosa	MCO-010 (sonpiretigene isteparvovec)	Second Half of 2026
Fabry Disease	ST-920 (isaralgagene civaparvovec)	Second Half of 2026
Liposarcoma/Sarcoma	Lete-cel (letetresgene autoleucel)	Second Half of 2026
Stiff Person Syndrome	KYV-101 (mivocabtagene autoleucel)	Second Half of 2026
Duchenne Muscular Dystrophy	RGX-202	Second Half of 2026 / First Half of 2027
Hereditary Angioedema	NTLA-2002 (lonvoguran ziclumeran)	Second Half of 2026 / First Half of 2027
Limb-Girdle Muscular Dystrophy	SRP-9003 (bidridistrogene xeboparvovec)	Clinical Trial Hold as of 7/2025
Mucopolysaccharidosis II	RGX-121 (clemidsogene lanparvovec)	Clinical Trial Hold as of 1/2026
Post-Transplant Lymphoproliferative Disorder	Ebvallo (tabelecleucel)	Complete Response Letter on 1/20/26

**If common names for therapies are listed, such names are not guaranteed to remain the same upon market approval.*

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Cost Containment Considerations

As part of its HMConnects™ cost containment program, HM Insurance Group (HM) works to support cost management opportunities around the use of gene and cell therapies and other high-cost pharmaceutical treatment options that can impact our clients' bottom line. The Pharmacy Operations (RxOps) team watches the market – and our book of business – to anticipate how current and future advancements will impact financial risk levels for HM's client base. Standard practices include reviewing, auditing, and collaborating on the content of current policies, monitoring trends, and implementing appropriate cost savings techniques. Additional practices include the prevention of stockpiling, working to ensure prescriptions are filled via in-network pharmacies, and assessing to determine if patients are properly dosed based on weight and lab values when appropriate. All these services are provided to HM's clients at no additional cost to them.

About HM Insurance Group

HM Insurance Group (HM) works to protect businesses from the financial risk associated with healthcare costs. A recognized leader in Employer Stop Loss, the company delivers protection for a range of group sizes. HM also offers assumed Accident and Health reinsurance, Provider Excess insurance, and Captive solutions through HM Specialty.

HM Life Insurance Company, HM Life Insurance Company of New York, and Bridge City Insurance Company are all rated "A" (Excellent) by AM Best Company.* Through its insurance companies, HM Insurance Group holds insurance licenses in 50 states and the District of Columbia and maintains sales offices across the country.

For more information, please contact **HMPharmacyServices@hmig.com.**

*AM Best Company, August 2025.

Sources: [1]American Society of Gene and Cell Therapy. (2026, April). Gene, cell & RNA therapy landscape report: Q1 2026.; [2]U.S. Food and Drug Administration. (2025). Compilation of CDER new molecular entity and new biologic approvals (2000–2024). https://www.fda.gov/drugs/drug-approvals-and-databases; [3]U.S. Food and Drug Administration. (2026). FDA approvals of specialty drugs, 2000–2024. Health Affairs Scholar, 4(2), qxag035. https://doi.org/10.1093/haschl/qxag035; [4]IPD Analytics. (2026, January). Drug pipeline and trend impact report: First half 2026; [5]IPD Analytics. (2026). RxInsights: 2025 rare disease drug approvals—Year in review.; [6]IPD Analytics. (2026, February). Data STACK trend report: January 2026 drug price changes; [7]Emerging Therapies Solutions (ETS). (2026, Q1). Gene and cell therapy forecast 2026

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