Pharmacy Focus:
Pipeline Therapies to Watch Through 2025

Key Takeaways

- Specialty medications for rare diseases lead the way in the approval trend, with gene and cell therapies remaining a core focus.
- Gene therapies continue to be exceptionally high cost (generally between $2 million and $3 million for one-time infusions, though two approved in 2024 go beyond that mark — one at nearly $4 million and the other exceeding it).
- There are three new gene or cell therapies anticipated in the second half of 2025, and other high-cost therapies for rare diseases seek expanded indications.

What's in the Pipeline?[1-8]

In 2024, the United States Food and Drug Administration (FDA) approved 61 novel drugs — new and innovative treatments that often fulfill an unmet medical need. Twenty-six of the 61 therapies approved were first-in-class, meaning there were no other similar alternatives to these treatments prior to their approval.

Approvals have been trending toward specialty medications for rare diseases for several years, and 2024 approvals continued that trend. Gene and cell therapies continue to be a major focus, with treatments for ultra-rare hereditary diseases like mucopolysaccharidosis types II and IIIa (devastating inherited lysosomal storage disorders) awaiting approval. Many of the therapies in the pipeline have received an expedited review designation, which ultimately means they could be approved within six to 10 months after the completed application is submitted for FDA review.

The costs associated with gene therapies remain exceptionally high, with prices generally set between $2 million and $3 million or more for the one-time infusions. In 2024, however, two of the most expensive gene therapies in the world (to date) were approved — Lenmeldy™ for metachromatic leukodystrophy and Kebilidi™ for aromatic L-amino acid decarboxylase (AADC), carrying price tags of $4.25 million and $3.95 million, respectively. These cost estimates do not include any ancillary charges, such as hospitalizations or the ongoing acute care and monitoring that is needed following administration. The potential monetary impact of these therapies can be huge, which is why it is important to closely monitor the pipeline.

Looking ahead, there currently are several therapies with potential approval dates through the third quarter of 2025:

- Etuvetidigene Autotemcel – a one-time therapy for Wiskott-Aldrich syndrome (a rare, inherited disorder causing excessive bleeding, frequent infections, and eczema)
- UX111 – a one-time gene therapy for mucopolysaccharidosis type IIIa (Sanfilippo syndrome)
- Deramiocel – an allogeneic, off-the-shelf cellular therapy for the treatment of Duchenne muscular dystrophy cardiomyopathy

Other rare disease states with potential high-cost therapies up for approval in 2025 and into early 2026 include mucopolysaccharidosis type II (Hunter syndrome), an inherited lysosomal storage disorder that often results in a shortened life span of 10 to 20 years, and Fanconi anemia, a blood disorder.

There also are several high-cost therapies in the rare disease field seeking approval for expanded indications, such as Gamifant® for hemophagocytic lymphohistiocytosis (HLH)/macrophage activation syndrome (MAS) in Still's disease that is recurrent or not responding to glucocorticoids.

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High-Cost Therapies Pipeline[2,8,9]

Disease	Therapy*	Anticipated Review Timing
HLH/MAS in Still's Disease	Gamifant® (emapalumab)	6/27/2025 (expanded indication)
Multiple Myeloma	REGN5458 (linvoseltamab)	7/10/2025
Melanoma	RP1 (vusolimogene oderparepvec)	7/22/2025
Mucopolysaccharidosis Type IIIA (Sanfilippo Syndrome)	UX111 (rebisufligene etisparvovec)	8/18/2025
Recurrent Respiratory Papillomatosis	PRGN-2012 (zopapogene imadenovec)	8/27/2025
Duchenne Muscular Dystrophy	CAP-1002 (Deramiocel)	8/31/2025
Mucopolysaccharidosis II	RGX-121 (clemidsogene lanparvovec)	Second Half 2025
Wiskott-Aldrich Syndrome	Etuvetidigene autotemcel	Q4 2025
Fanconi Anemia	RP-L102	Q4 2025

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

Number of Pipeline Therapies for Additional Diseases[2,8,9]

The diseases noted below all have potential therapies in the late phases of clinical trials. The number listed represents the possible drugs/therapies (including expanded indications for current FDA-approved drugs) that could receive market approval in 2025 for the listed indication.

Disease	Number of Therapies
Breast Cancer	7
Bladder Cancer	3
Chronic Immune Thrombocytopenia	2
Endometrial Cancer	5
Friedreich's Ataxia	1
Glioblastoma	3
Hemophilia A/B	1
Hepatocellular Carcinoma	6
Hereditary Angioedema	3
Liposarcoma	1

Disease	Number of Therapies
Mucopolysaccharidosis (All Types)	3
Multiple Myeloma	6
Myasthenia Gravis	3
Myelodysplastic Syndromes	1
Non-Small Cell Lung Cancer	16
Paroxysmal Nocturnal Hemoglobinuria	1
Prostate Cancer	7
Skin Cancer (all types)	8
Spinal Muscular Atrophy	2

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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.

Pharmacy Focus provides valuable information about pharmaceutical industry developments and their associated costs that can impact the growing claims trend in the self-funded insurance market. Be aware of influences and gain insight into approaches that may help to contain costs. Please share topic suggestions or feedback with **HMPharmacyServices@hmig.com**.

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Resources: [1]FDA Novel Drug Approvals – 2024 Year in Review, IPD Analytics, March 2025; [2]Drug Pipeline Report: 1H 2025, IPD Analytics, January 2025; [3]Cellular & Gene Therapy Products: Approved Cellular and Gene Therapy Products, FDA.gov, https://www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/approved-cellular-and-gene-therapy-products, accessed April 7, 2025; [4]Mucopolysaccharidosis Type 3, U.S. Department of Health and Human Services, retrieved from https://rarediseases.info.nih.gov/diseases/3807/mucopolysaccharidosis-type-3, April 2025; [5]National Organization for Rare Disorders, September 6, 2019, Mucopolysaccharidosis Type II, retrieved from https://rarediseases.org/rare-diseases/mucopolysaccharidosis-type-ii/, April 2025; [6]Orchard Sets $4.25M U.S. Price for Gene Therapy Lenmeldy on Heels of Approval," Biospace, https://www.biospace.com/article/orchard-sets-4-25m-us-price-for-gene-therapy-lenmeldy-on-heels-of-approval/, accessed March 20, 2024; [7]Fast Track, Breakthrough Therapy, Accelerated Approval, Priority Review, FDA.gov, www.fda.gov/patients/learn-about-drug-and-device-approvals/fast-track-breakthrough-therapy-accelerated-approval-priority-review, accessed April 7, 2025; [8]2025 Gene Therapy Pipeline Q4 2024-Q3 2027: Projected Treatments and Launch Timelines, CVS Caremark. [9]Prime Therapeutics, January 2025; Quarterly Drug Pipeline: January 2025, retrieved from https://primetherapeutics.com/quarterly-drug-pipeline-january-2025/, April 2025.

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