HPR69

2026 Review of Gene Therapy Access Landscape

Brooks K[1], Privett B[2], Meersman M[3], Lee K[3]
[1]Red Nucleus, Denver, CO, USA; [2]Red Nucleus, New York City, NY, USA; [3]Red Nucleus, Boston, MA, USA

1 OBJECTIVES

As more 'breakthrough' gene therapies launch, budget impact for payers continues to grow. A common payer strategy for mitigating the financial impact of a given therapy is to restrict the eligible patient population through stringent access criteria. Some manufacturers offer contracts (e.g., outcomes-based agreements, innovative contracts, etc.) to commercial plans to alleviate payer specific concerns (e.g., financial, efficacy, durability, etc.) in exchange for reduced access barriers. This research analyzes the current gene therapy pricing and market access landscape and the commercial success of those treatments.

2 METHODS

Gene therapy management policies with FDA approvals as of December 2025 were extracted from 7 national and 13 regional health plans (202.0M covered lives). Policies were compared to FDA Package Inserts and pivotal clinical trial inclusion/exclusion criteria. Pricing trends, financial reports, and press releases from gene therapy manufacturers were used to assess pricing and market access trends.

GENE THERAPIES ASSESSED

Name (Launch)	Average WAC (USD)	US Revenue (FY 2025, USD in millions)	Indication (Simplified)
Papzimeos (2025)	$0.115	$3	Recurrent respiratory papillomatosis
Adstiladrin (2022)	$0.25M*	Not available	BCG-unresponsive NMIBC w/ CIS
Luxturna (2017)	$0.46M	$46**	Biallelic RPE65 mutation-associated retinal dystrophy
Vyjuvek (2023)	$0.68M*	$389	Dystrophic epidermolysis bullosa with COL7A1 mutations
Casgevy (2023)	$2.2M	$116***	Sickle cell disease with history of VOC
Casgevy (2024)			Transfusion-dependent β-thalassemia
Zolgensma (2019)	$2.6M	$413	Spinal muscular atrophy with bi-allelic mutations in the SMN1 gene
Itvisma (2025)	$2.6M	Not available	Spinal muscular atrophy with mutation in the SMN1 gene
Roctavian (2023)	$2.8M	$36	Severe hemophilia A (congenital factor VII deficiency)
Zynteglo (2022)	$2.8M	$104****	B-thalassemia
Rethymic (2021)	$2.8M	$47	Congenital athymia
Skysona (2022)	$3.0M	Not available	Early cerebral adrenoleukodystrophy
Lyfgenia (2023)	$3.1M	$48****	Sickle cell disease with history of VOC
Elevidys (2023)	$3.2M	$899	Duchenne muscular dystrophy
Hemgenix (2022)	$3.5M	$149***	Hemophilia B (congenital factor IX deficiency)
Kebilidi (2024)	$4.0M	$11	AADC deficiency

Table 1. Summary of gene therapies included in this analysis, cost calculated as of Apr. 2025
Adstiladrin is priced at $15,453 per vial with a projected annual cost around $247,000; Vujuvek is priced at $26,000 per vial with a projected annual cost around $676,000; Roctavian is priced at $94,745 per vial with weight-based dosing assuming 75kg yielding a projected cost of around $2,800,000; ** Average WAC per eye; * World revenue as US not available; **** Annual revenue assumption based on 3 month revenue of ~March 2025 10-Q release*
AADC: Aromatic L-amino acid decarboxylase, BCG: Bacillus Calmette Guérin; CIS: Carcinoma in situ; COL7A1; Collagen type VII alpha 1 chain, NMIBC: Non-muscular invasive bladder cancer; RPE65: Retinal pigment epithelium-specific 65 kDa, SMN1: Survival motor neuron 1; VOC: Vaso-occlusive crises

3 RESULTS

Consistent with analysis from previous years, payer coverage remains generally favorable despite the high cost of gene therapies. Across the landscape, lower cost gene therapies (e.g., Adstiladrin, Papzimeos) tend to receive more permissive management, while higher priced therapies (e.g., Elevidys, Hemgenix) are more likely to face restrictive utilization controls extending beyond trial criteria. In certain high-cost cases (e.g., Rethymic, Kebilidi), higher rates of non-coverage were also observed. These findings indicate a clear relationship between price and the intensity of payer management, though the nature and degree of restriction vary meaningfully between products.

DEFINITIONS OF COVERAGE CRITERIA

Not found	No coverage criteria found
PA to indication statement	Requirements for coverage do not extend beyond the approved indication
PA to trial	Requirements for coverage do not extend beyond the inclusion/exclusion criteria in the pivotal trial
PA beyond trial	Requirements for coverage extend beyond the inclusion/exclusion criteria in the pivotal trial
Not covered	Medication is not covered by the payer; using the medication would mean completely out of pocket costs

Table 2. Definitions of coverage criteria used in analysis
PA: Prior authorization

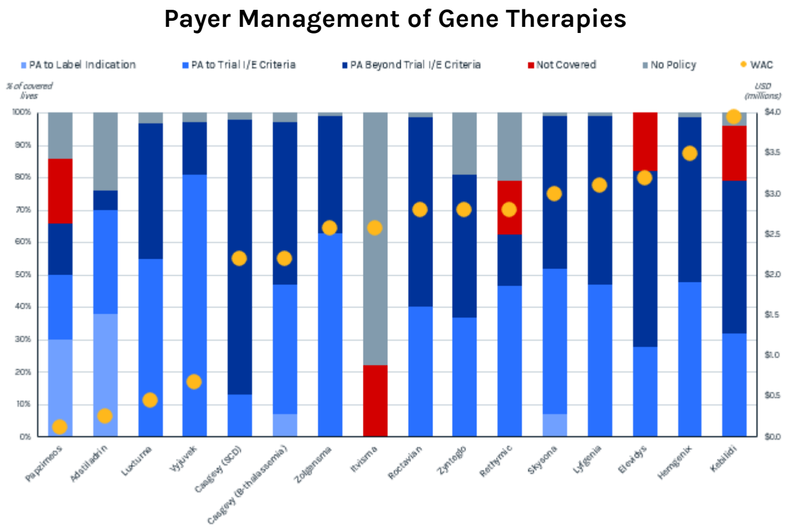

Figure 1. Commercial coverage for gene therapies at twenty of the largest plans by lives
n=20 plans, n=202.0M lives analyzed. Graph percentages are calculated as proportions of total lives managed.
WAC: Wholesale acquisition cost

4 CONCLUSIONS

Payer management policies appear to be influenced by multiple factors including disease burden, unmet need, time on market, competition in the market, the value of a new treatment, and cost of that treatment. Contracting arrangements, including value-based agreements, likely further support adoption by aligning perceived value with financial risk. Ultimately, despite the observed expansion of payer utilization management, this analysis suggests that restrictive payer access criteria is not the primary determinant of gene therapy uptake or sales. While utilization management requirements have expanded relative to 2024, this trend has not translated into measurable revenue pressure Elevidys, for example, generated the highest revenue ($899M USD) among the gene therapies analyzed, driven by high unmet need and limited treatment alternatives, even amid indication restrictions, reinforcing the view that payer controls are not the primary driver of gene therapy commercial performance.
Variation in gene therapy uptake and commercial performance may reflect differences in disease prevalence, unmet need at launch, urgency to treat, perceived efficacy and value, time on market, and the level of patient and clinician advocacy. In cases of lower uptake, factors such as limited clinical support, lower unmet need, treatment center financial dynamics, or recent launch timing may play a greater role than payer access alone.

5 FUTURE IMPLICATIONS

While this analysis suggests that payer access criteria have not been the primary constraint on gene therapy uptake to date, patient access remains critical for long-term commercial success in a rapidly evolving landscape such as gene therapy. Despite targeting low prevalence indications, the high per-patient cost of these therapies can have significant cumulative impact for payers. As launch frequency and price points continue to rise, payers are increasingly incentivized to apply more restrictive management, a trend that is likely to persist. As a result, early market access planning and foundational value strategy planning will become increasingly important to mitigate any future commercialization hurdles.

6 REFERENCES

For indication/revenue:
1. Papzimeos: https://www.nasdaq.com
2. Adstiladrin: www.ferring.com
3. Luxturna: assets.roche.com
4. Vyjuvek: www.biomarin.com
5. Casgevy: www.vrtx.com
6. Zolgensma: www.novartis.com
7. Itvisma: www.itvisma.com
8. Roctavian: www.investors.biomarin.com
9. Zynteglo: www.genetixbiotx.com
10. Rethymic: www.sumitomo-pharma.com
11. Skysona: www.genetixbiotx.com
12. Lyfgenia: www.genetixbiotx.com
13. Elevidys: investorrelations.sarepta.com
14. Hemgenix: www.csl.com
15. Kebilidi: https://ir.ptcbio.com

For pricing and covered lives information:
1. www.pricerx.medispan.com
2. www.policytracker.com

For gene therapy management policies:
1. BCBS SC: www.southcarolinablues.com/
2. BCBS MI: www.bcbsm.com/
3. Molina: www.molinahealthcare.com/
4. Highmark: www.highmark.com/
5. Horizon BCBS: www.horizonblue.com/
6. Independence Blue Cross: www.ibx.com/
7. Florida Blue: www.floridablue.com/
8. BSCA: www.blueshieldca.com/
9. BCBS NC: www.bluecrossnc.com/
10. Carefirst BCBS: member.carefirst.com/
11. BCBS Alabama: www.bcbsal.org/web/
12. BCBS Tennessee: www.bcbst.com/
13. Premera Blue Cross: www.premera.com/
14. Humana: www.humana.com/
15. UHC: www.uhc.com/
16. Anthem: www.anthem.com
17. Aetna: www.aetna.com/
18. Centene: www.centene.com/
19. HCSC: www.hcsc.com/
20. Cigna: www.cigna.com/

Author contact details: Kevin Brooks
Email: kbrooks@rednucleus.com
www.rednucleus.com/macs