Review of Gene Therapy Access Landscape

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OBJECTIVES

As more and 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 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.

METHODS

Gene therapy management policies with FDA approvals as of January 2025 were extracted from 7 national and 13 regional health plans (202.0M covered lives) and then 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 Analyzed

Name (Launch)	Average Wholesale Acquisition Cost (USD)	US Revenue (FY 2024, USD in millions)	Indication
Vyjuvek (2023)	$0.025M*	$291	Dystrophic epidermolysis bullosa with COL7A1 mutations
Adstiladrin (2022)	$0.24M	$70	BCG-unresponsive NMIBC w/ CIS
Luxturna (2017)	$0.85M	Not available	Biallelic RPE65 mutation-associated retinal dystrophy
Casgevy (2023)	$2.2M	$10**	Sickle cell disease with history of VOC
Zolgensma (2019)	$2.4M	$435	Spinal muscular atrophy with bi-allelic mutations in the SMN1 gene
Zynteglo (2022)	$2.8M	$62	B-thalassemia
Roctavian (2023)	$3.0M	$26**	Severe hemophilia A (congenital factor VII deficiency)
Skysona (2022)	$3.0M	$10	Early cerebral adrenoleukodystrophy
Lyfgenia (2023)	$3.1M	$12	Sickle cell disease with history of VOC
Elevidys (2023)	$3.2M	$821	Duchenne muscular dystrophy
Hemgenix (2022)	$3.5M	Not available	Hemophilia B (congenital factor IX deficiency)
Kebilidi (2024)	$4.0M	Not available	AADC deficiency

Table 1. Summary of gene therapies included in this analysis, cost calculated as of May, 2025
**At launch, Vyjuvek was priced at $24,250 per vial with a projected annual cost around $631,000*
***World revenue as US not available*
AADC: Aromatic L-amino acid decarboxylase, BCG: Bacillus CalmetteGuérin; 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

RESULTS

Coverage is generally favorable despite the high cost of many gene therapies, consistent with last year's analysis. Given high per patient budget impact and following common practice in expensive disease states, most policies restrict access to reflect trial criteria to ensure patients receiving gene therapies match those in whom use was proven to be safe and effective. Lower cost gene therapies receive more open management, while higher priced therapies such as Kebilidi or Lyfgenia are more likely to encounter additional criteria (e.g., additional clinical criteria which are not reflected in product label).

Definitions of Coverage Criteria

SUBHEADING	Definition
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

As prices increase amongst listed therapies, payers gradually input more restrictions beyond label to mitigate the increasing price.

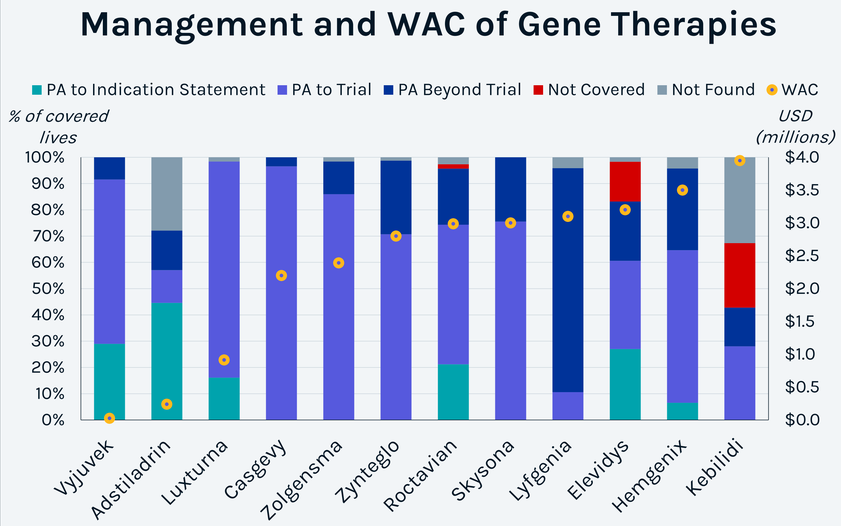

Figure 1. Commercial coverage for gene therapies at twenty of the largest plans by lives
n=20 plans, n=202.0M lives analyzed. Graph percentages are calculated as proportions of total lives managed
WAC: Wholesale acquisition cost

The disparity in management of Casgevy versus Lyfgenia is of particular note as these products share an indication in sickle cell disease. Payer willingness to introduce management criteria beyond trial for Lyfgenia is likely motivated by its expensive price tag in comparison to Casgevy without sufficient demonstration of added benefit.

CONCLUSIONS

Underlying motivations for payer management policies are varied and complex, pulling on several factors including disease burden, unmet need, competition in the market, the value of a new treatment, and cost of that treatment. Contracting (e.g., value-based agreements) also likely plays a role in supporting the value of gene therapies. The analysis conducted appears to reinforce our previous hypothesis that that payer access criteria is not the primary factor hindering uptake or sales of currently approved gene therapies. Specific disparities in uptake and/or commercial success amongst therapies are likely due to disease prevalence, level of unmet need at launch, urgency to treat with a gene therapy, efficacy/value of the treatment, and/or time on market. Patient and clinician support/advocacy may also drive significant uptake in indications with high burden and unmet need. Gene therapies with low patient uptake are likely due to lack of clinician and patient support and lower unmet need rather than payer access issues.

FUTURE IMPLICATIONS

Patient access remains critical for a successful gene therapy launch. Despite treating low-prevalence indications, high per patient costs can have profound impacts on payer budgets. Payers have not yet severely restricted gene therapies but may become more willing as the financial impact of sickle cell and pipeline gene therapies are realized. Early market access and value strategy planning are essential to avoid any unnecessary commercialization hurdles.

REFERENCES

For indication/revenue:
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2. Adstiladrin: https://www.ferring.com/
3. Luxturna: https://sparktx.com/
4. Casgevy: https://www.vrtx.com/
5. Zolgensma: https://www.novartis.com/
6. Zynteglo: https://www.bluebirdbio.com/
7. Roctavian: https://www.biomarin.com/
8. Skysona: https://www.bluebirdbio.com/
9. Lyfgenia: https://www.bluebirdbio.com/
10. Elevidys: https://www.sarepta.com/
11. Hemgenix: https://www.csl.com/
12. Kebilidi: https://www.ptcbio.com/

For Pricing and Covered Lives Information:
https://pricerx.medispan.com/
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For gene therapy management policies:
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4. Centene: https://www.centene.com/
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