Review of Gene Therapy Access Landscape

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OBJECTIVES

Gene therapies are often met with optimism and considered "breakthrough innovations" and the "future of medicine." However, gene therapy costs are high, and their budget impact concerns payers. Payers may mitigate the financial impact of gene therapies by restricting eligible patient populations through stringent access criteria, potentially impacting uptake. To mitigate aggressive market access restrictions, 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 favorable access. 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 2024 were extracted from 7 national and 13 regional health plans (201.9M 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 2023, USD in millions)	Indication
Vyjuvek (2023)	$0.024M*	$50.7	Patients 6 months and older with dystrophic epidermolysis bullosa with mutations in COL7A1 gene
Luxturna (2017)	$0.85M	Not available	Patients with confirmed biallelic RPE65 mutation-associated retinal dystrophy
Zolgensma (2019)	$2.1M	$372	Patients less than 2 years of age with spinal muscular atrophy
Zynteglo (2022)	$2.8M	Not available	Pediatric and adult patients with B-thalassemia
Roctavian (2023)	$2.9M	$3.5	Adults with severe hemophilia A (congenital factor VII deficiency)
Skysona (2022)	$3M	Not available	Boys aged 4-17 with early, active cerebral adrenoleukodystrophy
Elevidys (2023)	$3.2M	$200.4	Duchenne muscular dystrophy (DMD) for patients ages 4-5
Hemgenix (2022)	$3.5M	Not available	Adults with hemophilia B (congenital factor IX deficiency)

**24,250 per vial, annual cost projected around $630,500; **North America, Q3YTD FY 2023 data*
Cost calculated as of Feb. 2024

RESULTS

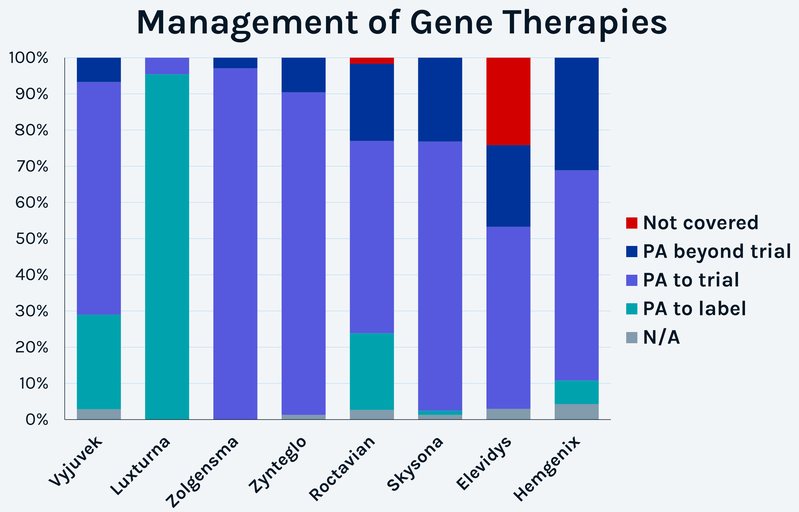
Despite the high prices of gene therapies, coverage is rather favorable. Most policies include language that restricts access to reflect trial criteria, as payers want to ensure that the patients that receive a gene therapy match those who were studied where it was shown to be safe and effective (a common practice for high-cost rare diseases treatments). For gene therapies that are lower cost (i.e., Luxturna), payers are more willing to manage to indication statement, while higher priced therapies like Hemgenix may be managed beyond trial criteria (i.e., requiring specific step therapies) to manage budget impact.

Definitions of Coverage Criteria

SUBHEADING	Definition
N/A	No coverage criteria found
PA to label	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. PA: Prior authorization

~25% of lives do not cover Elevidys, which is likely due to its recent FDA approval via the accelerated approval pathway. Despite its new approval, majority of payers are covering the treatment to trial criteria, showing promising reactions for new-to-market gene therapies.



n=20 plans, n=201.9M lives analysed. Graph percentages are calculated as proportions of total lives managed

Many manufacturers of gene therapies look to employ methods to mitigate payer budget and durability concerns due to the overall high cost and uncertain long-term efficacy and safety. For example, Novartis previously announced attempts for 5-year outcomes-based agreements to support access to Zolgensma. Contracts like these may play a role in Zolgensma's favorable access despite its cost.

CONCLUSIONS

There are many factors that affect the access criteria of a gene therapy 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. From the analysis conducted, it can be hypothesized that payer access criteria is not significantly hindering uptake and sales of currently approved gene therapies. Differences in uptake and commercial success amongst the treatments are likely due to time on market, disease prevalence, level of unmet need at launch, urgency to treat with a gene therapy and efficacy/value of the treatment. Patient and clinician support / advocacy is also helping drive significant uptake with products like Zolgensma and Elevidys due to the high burden and unmet need in those diseases. Gene therapies with low patient uptake are likely due to lack of clinician and patient support and lower unmet need, and not likely driven by payer access issues.

FUTURE IMPLICATIONS

As more gene therapies come to market (including Casgevy and Lyfgenia for sickle cell anemia and Lenmeldy for metachromatic leukodystrophy launched in 2024), patient access remains critical. Even though gene therapies are indicated in rare populations, their high costs can have profound impacts on payer budgets. Payers may look to further limit access as more gene therapies come to market and have a larger cumulative impact. Early market access and value strategy planning are essential to avoid any unnecessary commercialization hurdles.

REFERENCES

For indication/revenue:
1. Vyjuvek: https://www.krystalbio.com/
2. Luxturna: https://sparktx.com/
3. Zolgensma, including contracting: https://www.novartis.com/
4. Zynteglo: https://www.bluebirdbio.com/
5. Roctavian: https://www.biomarin.com/
6. Skysona: https://www.bluebirdbio.com/
7. Elevidys: https://www.sarepta.com/
8. Hemgenix: https://www.csl.com/

For Pricing and Covered Lives Information:
https://www.globaldata.com/
https://www.policytracker.com/

For gene therapy management policies:
1. UHC: https://www.uhc.com/
2. Anthem: https://www.anthem.com/
3. Aetna: https://www.aetna.com/
4. Centene: https://www.centene.com/
5. HCSC: https://www.hcsc.com/
6. Cigna: https://www.cigna.com/
7. Humana: https://www.humana.com/
8. BCBS SC: https://www.southcarolinablues.com/
9. BCBS MI: https://www.bcbsm.com/
10. Molina: https://www.molinahealthcare.com/
11. Highmark: https://www.highmark.com/
12. Horizon BCBS: https://www.horizonblue.com/
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14. Florida Blue: https://www.floridablue.com/
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