Alignment of Estimated Acquisition Costs in U.S. Gene Therapy Cost-Effectiveness Analyses with Standard Price Benchmarks

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Background

Gene therapies (GTs) are promising treatments for serious life-threatening conditions. However, their high upfront costs often exceed $1 million and reach $4.5 million, posing major challenges for U.S. payers.

Cost-effectiveness analysis (CEA) is used to guide reimbursement for GTs. Yet, studies assessing the alignment of estimated acquisition costs (EAC) used in U.S. gene therapy CEA studies with price benchmarks remain limited.

Objectives

The objective of this study was to assess the alignment of EACs for gene therapies used in U.S. CEAs with standard price benchmarks.

Methods

We extracted EACs for GTs from U.S. cost-effectiveness studies published in PubMed through Nov. 2025.

We compared each EAC with the average wholesale price (AWP) and wholesale acquisition cost (WAC) (Red Book), and the average sales price (ASP) from Centers for Medicare & Medicaid Services. Results were summarized descriptively.

Results

Figure 1. U.S. Cost-Effectiveness Analyses of Gene Therapies

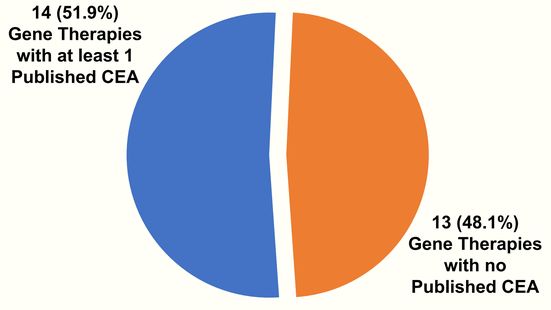


Table 1. Gene Therapies with Published Cost-Effectiveness Analysis

Gene Therapy	Authorization Date	WAC at Market Entry	Number of CEAs
tisagenlecleucel	8/30/2017	$ 475,000	3
axicabtagene ciloleucel	10/18/2017	$ 373,000	18
voretigene neparvovec	12/19/2017	$ 1,020,000	2
onasemnogene abeparvovec	5/24/2019	$ 2,125,000	2
brexucabtagene autoleucel	7/24/2020	$ 373,000	2
lisocabtagene maraleucel	2/5/2021	$ 410,300	3
idecabtagene vicleucel	3/26/2021	$ 419,500	1
ciltacabtagene autoleucel	2/28/2022	$ 465,000	2
betibeglogene autotemcel	9/1/2022	$ 2,800,000	1
etranacogene dezaparvovec	11/22/2022	$ 3,500,000	1
nadofaragene firadenovec	12/16/2022	$ 240,000	1
valoctocogene roxaparvovec	6/30/2023	$ 2,446,875	1
lovotibeglogene autotemcel	12/8/2023	$ 3,100,000	1
eladocagene exuparvovec	11/13/2024	$ 3,950,000	1

There were 79 CEA studies listed in PubMed, of which 34 (43.0%) were original research studies reporting 43 EAC observations. Fourteen GTs (51.9% of 27 FDA-authorized GTs) had a published CEA (Figure 1); 7 studies assessed multiple GTs and 7 were evaluated in only one CEA (Figure 2). Axicabtagene ciloleucel, with 18 CEAs, was the GT with the highest number of published CEAs.

Figure 2. Estimated Acquisition Cost Used in CEAs as a % of the Wholesale Acquisition Cost

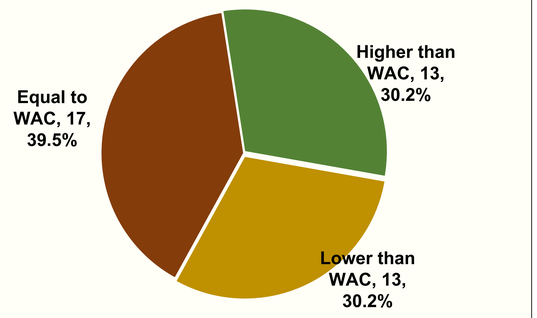
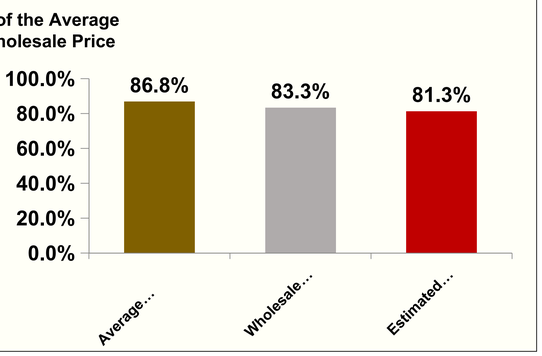


Figure 3. Estimated Acquisition Cost Used in CEAs as a % of U.S. Price Benchmarks



The WAC was 83.3% of the AWP (Figure 3). The AWP and WAC were available for all gene therapies included in the CEAs. The EAC represented a mean ± stdev of 81.3% ± 9.8% (range 59.1%-99.7%) of the AWP and 97.4% ± 11.9% (69.7%-119.7%) of the WAC. There were 13 (30.2%) EACs lower than the WAC, 17 (39.5%) equal, and 13 (30.2%) higher.

The ASP was available for six GTs and represented 86.8% ± 3.0% of the AWP and 96.2%±2.5% of the WAC.

The ASP is the U.S. price benchmark that most closely represents the actual acquisition cost of gene therapies. However, the ASP is only available for GTs administered in physician offices and outpatient clinics.

Additionally, the ASP is not readily available at market entry for new drugs and, therefore, it was unavailable for inclusion as an EAC at the time the CEAs were conducted.

The WAC for GTs was close to the ASP. Considering the high cost of GTs, small percentage differences in EACs may result in changes in CEA results.

Conclusions

The estimated acquisition costs of GTs were generally similar to the WAC. The ASP is the preferred price for establishing the EAC of gene therapies; however, given its limited availability at market entry, the use of the WAC is recommended.

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