Price Analysis of Gene Therapies Authorized by the U.S. Food and Drug Administration

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EE382

Background

Gene therapies represent a paradigm shift in the treatment of rare and life-threatening diseases. However, studies evaluating factors associated with gene therapy prices remain limited.

Objectives

This study described the clinical evidence available for gene therapies in the U.S., examined price variation across clinical characteristics, evaluated price trends, and compared gene therapy price benchmarks.

Methods

Regulatory data were obtained from the FDA website. Wholesale acquisition cost (WAC) data were obtained from the Micromedex RED BOOK; average sales price (ASP) data from the Centers for Medicare & Medicaid Services; and Federal Supply Schedule (FSS) and Big 4 prices from the U.S. Department of Veterans Affairs (VA) website.

Prices were adjusted to 2025 U.S. dollars using the Consumer Price Index (Bureau of Labor Statistics). Prices were estimated as cost per treatment for single or short-term use therapies and as annual cost for chronic-use therapies. WAC trends were calculated using annual price changes from market entry through January 1, 2026. ASP, FSS, and Big 4 were compared with the WAC.

Results

Figure 1. FDA Authorizations of Gene Therapies, Therapeutic Biologics, and New Molecular Entities, 2010-2025

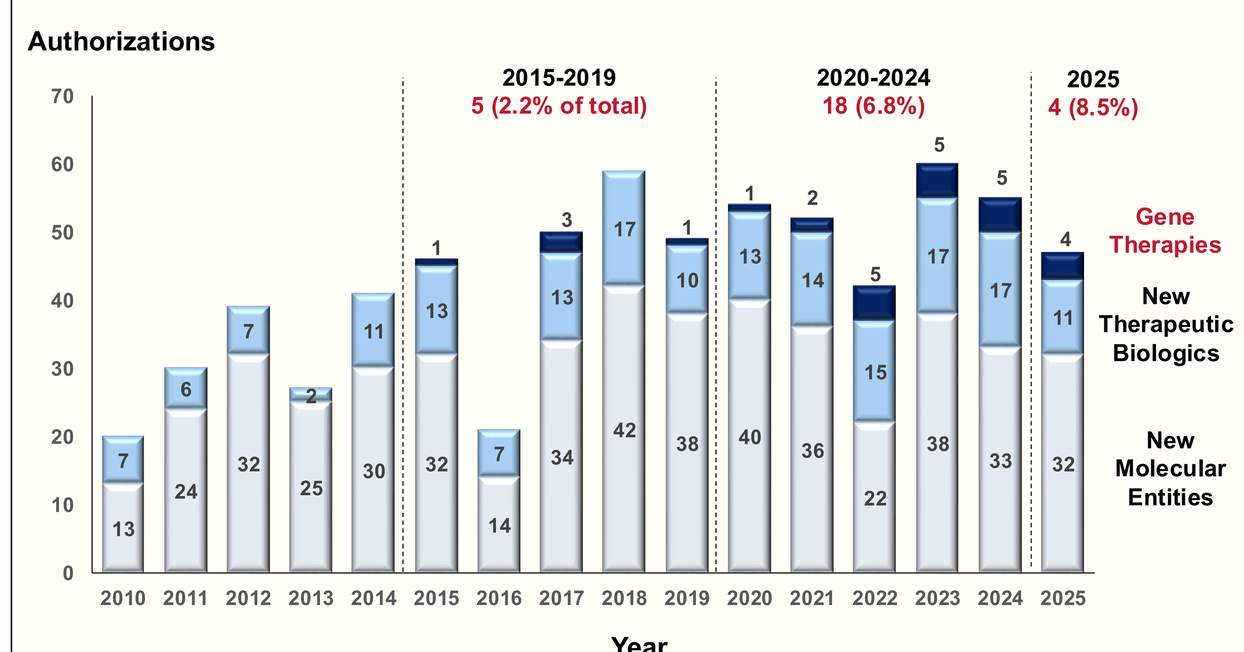
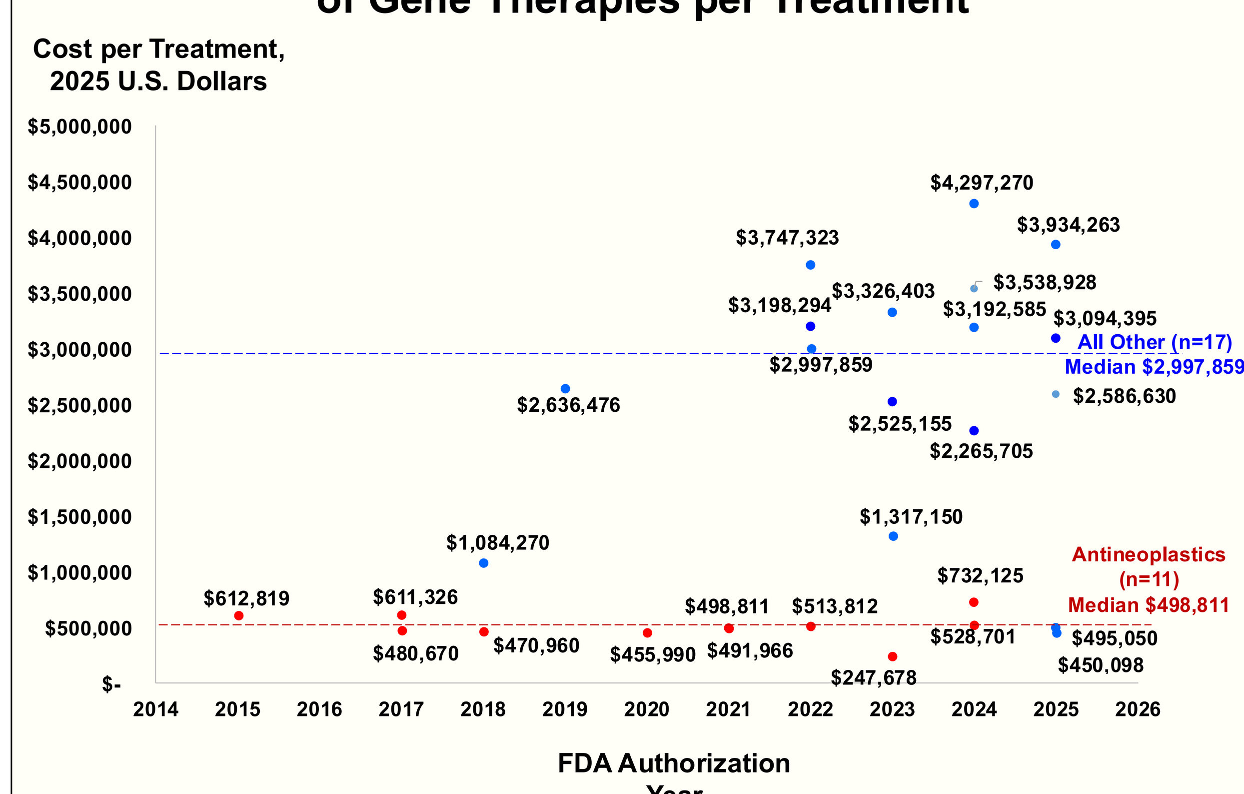


Figure 2. Inflation-Adjusted Wholesale Acquisition Cost of Gene Therapies per Treatment



Source: Compiled from U.S. Prescribing Information and Micromedex RED BOOK, 2026

Table 1. Median Inflation-Adjusted WAC (Adj-WAC) by Gene Therapy Characteristics

Category	Median Adj-WAC	IQR	N
Therapeutic Class*			
Alimentary Tract and Metabolism	$3,934,263	$549,488	3
Blood and Blood-Forming Organs	$3,095,222	$809,012	6
Musculoskeletal System	$2,636,476	$369,887	3
Dermatologicals	$2,205,773	$888,622	2
Sensory Organs	$665,898	$418,373	2
Antineoplastic and Immunomodulating Agents	$498,811	$94,198	11
Patient Population			
Pediatrics	$3,198,294	$830,397	3
Adults and Pediatrics	$2,792,244	$1,613,748	10
Adults	$498,811	$208,997	15
Patient Population (Excluding Antineoplastic Agents)			
Pediatrics	$3,198,294	$830,397	3
Adults and Pediatrics	$2,997,859	$926,880	9
Adults	$2,525,155	$3,088,830	5
Duration of Therapy			
Single-Use Treatment	$2,265,705	$2,693,774	25
Chronic-Use Treatment	$612,819	$534,736	3
Line of Therapy			
First-Line	$2,636,476	$2,221,778	13
Second-Line or Later	$528,701	$1,012,597	15
Line of Therapy (Excluding Antineoplastic Agents)			
First-Line	$2,636,476	$2,221,778	13
Second-Line or Later	$3,095,222	$379,192	4
Disease Incidence			
1-5 per 10,000	$2,265,705	$2,373,388	7
1-9 per 100,000	$612,819	$2,699,483	9
1-9 per 1,000,000	$1,951,890	$2,718,973	6
Complexity of Manufacturing Process			
Highest Complexity (Autologous CAR-T/*Ex Vivo*)	$570,013	$2,321,143	14
Very High Complexity (*In Vivo* Gene Therapies)	$2,555,892	$2,413,127	12
High Complexity (Specialized Tissue Engineering)	$1,670,960	$1,423,435	2
Complexity of Manufacturing Process (Excluding Antineoplastic Agents)			
Highest Complexity (Autologous CAR-T/*Ex Vivo*)	$2,997,859	$1,756,924	7
Very High Complexity (*In Vivo* Gene Therapies)	$2,586,630	$2,231,955	11
High Complexity (Specialized Tissue Engineering)	$1,670,960	$1,423,435	2
Costly Alternatives			
Non-Costly Alternatives	$506,312	$139,059	14
Costly Alternatives	$3,046,127	$753,853	14

Adj-WAC: Inflation-adjusted Wholesale Acquisition Cost; IQR: Interquartile range; CAR-T: Chimeric Antigen Receptor T-cell therapy. *Therapeutic class not available for zopapogene imadenovec

Gene therapy FDA authorizations increased from 2015 to 2025 (Figure 1). Therapies were indicated for hereditary conditions (n=15; 55.6%), oncology (n=10; 37.0%), and other (n=2; 7.4%).

WAC data were available for 26 (96.3%) and ASP data for 11 (40.7%) therapies. Onasemnogene and tisagenlecleucel had different adult and pediatric indications, which were analyzed separately, resulting in 28 price observations.

The median adj-WAC was $1,200,710 (IQR $2,621,843; n=28; range $247,678 to $4,297,270). Thirteen (46.4%) therapies were priced under $1 million and 15 (53.6%) between $1 million and $4.5 million (Figure 2).

Gene therapies for pediatric patients, single-use treatments, and those with costly alternatives had higher median adj-WAC. Antineoplastics had the lowest median adj-WAC (Table 1).

Costs were lower for antineoplastic and immunomodulating agents than for other therapeutic classes (Figure 2). After excluding antineoplastics, costs across patient population, line of therapy, and manufacturing process complexity were more similar. When adjusted for inflation, prices remained stable over time, with a mean ± standard deviation annual change of −0.37% ± 2.27%. The FSS price was 93.36% ± 6.20% of WAC (n=16), the Big 4 price was 72.47% ± 3.43% of WAC (n=17), and the ASP was 99.42% ± 4.35% of WAC (n=11).

Conclusions

FDA gene therapy authorizations increased from 2015 to 2025. WACs varied by therapeutic class, patient population, duration of therapy, manufacturing process complexity, and availability of costly alternatives. Antineoplastics appeared to influence several descriptive comparisons, likely due to the availability of alternatives for these therapies.

Prices remained stable over time. ASPs were close to WAC. FSS prices were also close to WAC, suggesting limited discounts for these therapies. Big 4 prices were lower than WAC, consistent with statutory pricing requirements.

Funded by Arnold Ventures