

The 10 Most Expensive Drugs in the U.S., Period

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Key takeaways:

- Zolgensma is the most expensive drug in America, at a one-time cost of \$2.1 million.
- Since March, prices for the most expensive drugs have remained stable, with only one medication seeing an increase in price.
- Folotyn increased in price by 3% from April to July.

Many drugs are expensive — but some drugs are *crazy* expensive. That's what this analysis by the GoodRx Research team reveals.

The GoodRx Research team regularly tracks the <u>most expensive medications</u> filled at local pharmacies in the U.S., and drugs on that list have only gotten more expensive. The top 3 drugs — Zokinvy, Myalept, and Mavenclad — all cost over \$60,000 for a typical monthly supply. However, these are the most expensive drugs filled at pharmacies, and patients self-administer them on a regular basis.

With more approvals of expensive infusions, cancer treatments, and gene therapies, we wanted to track the most expensive drugs, including those that you can't get from a pharmacy. We call these drugs "healthcare practitioner-administered drugs" because only a medical professional can administer them.

Many of the most expensive drugs are taken for an extended period of time, and costs quickly add up. This analysis ranks drugs by their annual cost for a typical course of therapy. Prices are based on the drug's list price — which is the price that the pharmaceutical company assigns as an official price to a drug — then adjusted for time to create a comparable benchmark.

The following are the 10 most expensive drugs, period, in the U.S., taking into account both drugs you can get at a pharmacy and healthcare practitioner-administered drugs. Relatively few people take most of these drugs — but their outsized costs can translate into higher premiums and more restrictive formularies for everyone.

The Most Expensive Drugs, Period

July 2021

	Drug	Manufacturer	Annual cost based on length of therapy
1	Zolgensma	AveXis, Inc	\$2,125,000
2	Zokinvy	Eiger BioPharmaceuticals	\$1,032,480
3	Danyelza	YmAbs Theraptuics, Inc	\$977,664
4	Myalept	Aegerion Pharmaceuticals	\$889,904
5	Luxturna	Spark Therapeutics	\$850,000
6	Folotyn	Acrotech Biopharma	\$817,865
7	Brineura	BioMarin Pharmaceuticals	\$730,340
8	Blincyto	Amgen, Inc	\$712,672
9	Ravicti	Horizon Therapeutics	\$695,970
10	Soliris	Alexion Pharmaceuticals, Inc	\$678,392

^{*}This list includes prescription drugs and drugs only administered by a healthcare professional. Quantity of dose may depend on weight or body surface area of the individual, meaning that prices can vary.

Source: GoodRx

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1) Zolgensma – \$2,125,000

Since Zolgensma received FDA approval in May 2019, it has remained the most expensive drug in the U.S., with a one-time cost of \$2.1 million for a course of treatment.

Zolgensma is approved to treat spinal muscular atrophy, a rare childhood disorder that causes muscular erosion that can lead to lung infections and muscle weakness. Zolgensma is a one-time curative therapy, and many children who have been treated with the drug now show no signs of the disease.

Paying for Zolgensma isn't easy, as <u>insurers may not cover it</u> or may have certain requirements in order for patients to qualify for coverage. To encourage insurers to cover this gene therapy, manufacturer Novartis Gene Therapies has allowed certain insurers to pay for the drug in annual installments of \$425,000 over 5 years. Novartis Gene Therapies also offers the <u>OneGene Program</u> to provide guidance and help to navigate insurance requirements.

2) Zokinvy – \$1,032,480

Zokinvy, an orphan drug that treats Hutchinson-Gilford progeria syndrome, was approved by the FDA in November 2020, and the cost for Zokinvy is staggering at over \$1 million a year. It is the first and only treatment approved for Hutchinson-Gilford progeria syndrome, a rare genetic disease that causes premature aging.

Although the dosage depends on body surface area, patients typically take around 200 mg of Zokinvy a day, and, at \$717 per 50-mg capsule, this adds up to \$86,040 a month. While there is no copay card available for this drug, the <u>Eiger OneCare Program</u> can help assist patients with insurance coverage and financial support options.

3) Danyelza – \$977,664

The FDA approved <u>Danyelza</u> in November of 2020 to treat pediatric and adult patients with neuroblastoma in the bone or bone marrow. Danyelza was under an <u>accelerated approval program</u> and is considered an orphan drug and breakthrough therapy.

However, the medication is not cheap. Danyelza costs \$20,368 per vial, and patients typically use around 48 vials per year, bringing the annual cost close to \$977,664. Manufacturer Y-mAbs offers <u>Y-mAbs Connect</u>, a program that can help patients navigate insurance, as well as a patient assistance program for eligible uninsured patients.

4) Myalept – \$889,904

Myalept is one of the only drugs on this list that is dispensed at the pharmacy. It is used to treat leptin deficiency in patients with generalized lipodystrophy, a condition of abnormal fat distribution in the body.

Myalept costs \$74,159 for a monthly supply. Patients self-administer Myalept once a day and typically use 14 vials per month, bringing the yearly price tag to \$889,904. Because Myalept is the only treatment available to control this rare condition, there are no other cost-saving alternatives.

Aegerion Pharmaceuticals offers assistance programs to help some patients afford Myalept.

5) Luxturna – \$850,000

At \$850,000 a year, Luxturna is the fifth-most expensive drug on the list. Luxturna is a gene therapy that treats an inherited form of retinal dystrophy, a condition that causes vision loss and even complete blindness.

Luxturna cannot be dispensed at a pharmacy, as it requires a doctor to administer 1 vial of the medication into each eye. Patients should only need one dose of Luxturna ever, but the list price is steep, ringing up at \$425,000 per vial.

<u>Spark Therapeutics Generation Patient Services</u> can provide assistance to patients by helping to navigate insurance coverage and connect patients to other financial resources.

6) Folotyn – \$817,865

Folotyn is approved to treat peripheral T-cell lymphoma, a rare blood cancer that can cause death. A healthcare professional must administer Folotyn, and patients are typically given 135 vials of the drug annually.

Manufacturer Acrotech Biopharma increased the list price for a vial by 1.9% in April 2021 from \$5,880 and increased it another 1.5% in July 2021 to \$6,058, bringing the total annual cost to \$817,865.

7) Brineura – \$730,340

Brineura is a first-in-class medication that treats a specific form of Batten disease called late infantile neuronal ceroid lipofuscinosis type 2 (CLN2). Batten disease encompasses a group of rare disorders of the nervous system. Specifically, Brineura helps treat the slow loss in walking ability for pediatric patients 3 years and older.

The list price of Brineura is \$28,090 for a kit of 2 150-mg vials. The recommended dosage is 300 mg every 2 weeks, meaning that over the course of one year, costs add up to \$730,340. Manufacturer BioMarin does provide patient support for insured and cash-paying patients through their <u>copay assistance program</u>.

8) Blincyto - \$712,672

The eighth-most expensive drug, Blincyto, has an annual list price of \$712,672. Manufactured by Amgen, Blincyto is used to treat a rare form of acute lymphoblastic leukemia, a cancer of the blood and bone marrow.

Only a healthcare provider can administer Blincyto, and dosing is done in cycles. The first cycle, known as the induction phase, is aimed at reducing the number of cancer cells. Cycles two

through four, known as consolidation phases, help new healthy cells grow. Patients need different amounts of the medication during each phase but will typically use about 168 vials per year. The current list price is \$4,242 per vial.

9) Ravicti – \$695,970

Used to treat urea cycle disorders, <u>Ravicti</u> has an annual list price of \$695,970. Urea cycle disorders are genetic conditions that result in high levels of ammonia in the blood. If left untreated, they can lead to confusion, coma, or even death.

Ravicti is dispensed in a pharmacy and doesn't require administration from a healthcare practitioner. Patients are typically given 132 bottles annually. The list price of Ravicti increased by 4.8% this January to \$5,273 for one bottle.

Manufacturer Horizon Therapeutics offers a way for some patients to save on Ravicti through the <u>Horizon Cares Patient Assistance Program</u>.

10) Soliris - \$678,392

Priced at \$678,392 per year, Soliris is used to treat paroxysmal nocturnal hemoglobinuria and atypical hemolytic uremic syndrome — blood disorders that result in the destruction of red blood cells.

The dosing regimen for Soliris can differ depending on the age of the patient and the condition being treated. But most patients need a maintenance dose of 1200 mg every 2 weeks, which only a healthcare provider can administer.

Because Soliris comes with a high risk of infection, the drug is part of a risk evaluation mitigation strategy (REMS) program. Medications with serious safety concerns require a REMS program, and healthcare providers who prescribe these drugs often need to undergo specific training to administer them.

There is no copay card available for Soliris, but the <u>OneSource program</u> from manufacturer Alexion can help patients navigate health insurance.

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Methodology

We created a list of the most expensive drugs by list price, including drugs that are administered under the supervision of a healthcare provider, and obtained typical fill quantities using data and information from the drug manufacturer websites. Using the official manufacturer list price, we calculated the total annual cost of a typical course of therapy for each drug. For drugs where there is a large effective dosing range, or where patient weight or body surface area determines dosing, we selected a dosing quantity based on an average weight or body surface area of a typical patient.

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