

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

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GoodRx Research August 2020 Key takeaways:

- Brineura, a drug that treats a specific form of Batten disease, is a new addition to the list, coming in as the fifth most expensive drug.
- Takhzyro was bumped off the list due to Brineura's addition, although its annual cost remains a high \$591,035.
- The list price for a vial of Folotyn increased in April from \$5,524 to \$5,880.

Many drugs are expensive — but some drugs are *crazy* expensive. That's what a new 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 United States, and the same drugs continue to make the top of the list. Myalept, Ravicti, and Mavenclad all cost over \$50,000 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 translate into higher premiums and more restrictive formularies for everyone.

The Most Expensive Drugs, Period

August 2020

Rank	Drug Name	Manufacturer	Annual cost based on length of therapy
1	Zolgensma	AveXis, Inc	\$2,125,000
2	Myalept	Amryt Pharma	\$855,678
3	Luxturna	Spark Therapeutics	\$850,000
4	Folotyn	Acrotech Biopharma	\$793,870
5	Brineura	BioMarin Pharmaceuticals	\$716,040
6	Soliris	Alexion Pharmaceuticals, Inc	\$678,392
7	Blincyto	Amgen, Inc	\$672,968
8	Ravicti	Horizon Therapeutics	\$664,092
9	Lumizyme	Sanofi Genzyme	\$643,243
10	Actimmune	Horizon Therapeutics	\$633,325

*This list includes prescription drugs and drugs only administered by a healthcare professional

Source: GoodRx

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1. Zolgensma - \$2,125,000

Since <u>Zolgensma</u> received FDA approval in May 2019, it has remained the most expensive drug in the U.S. With a list price of \$2.1 million for a course of treatment, Zolgensma has caused an uproar in the prescription drug community, with many blasting it for its astronomical price tag. However, many are <u>citing it</u> as a surprisingly cost-effective treatment, as it can cure a once-incurable disease.

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.

So, how can anyone afford this life-saving drug? Unfortunately, cash-paying patients may be on the hook for the full price of the drug. Luckily, some insured patients may be able to get coverage through their plan, as the manufacturer has allowed certain insurers to pay for the drug in annual installments of \$425,000 over 5 years.

2. Myalept - \$855,678

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.

The price for a monthly supply of Myalept is a shocking \$71,306. Patients self-administer Myalept once a day and typically use 14 vials per month, bringing the yearly price tag to \$855,678. Because Myalept is the only treatment available to control this rare condition, there are no other cost-saving alternatives.

Aegerion Pharmaceuticals offers a <u>copay card</u> to help some patients afford Myalept.

3. Luxturna - \$850,000

At \$850,000 a year, <u>Luxturna</u> is the third most expensive drug on the list but is over \$1 million cheaper than Zolgensma. 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.

4. Folotyn - \$793,870

<u>Folotyn</u> 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 45 vials of the drug annually.

In April, the list price for a vial increased from \$5,524 to \$5,880, bringing the total annual cost to \$793,870. Despite its high price tag, Folotoyn's effectiveness has been called into question. In

fact, the European Union has <u>not yet been approved</u> it due to insufficient evidence of health gains.

5. Brineura - \$716,040

<u>Brineura</u> 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.

Brineura is the first treatment for CLN2, and because of that, it comes with a high price tag. The cost for 150 mg of Brineura is \$27,500. The recommended dosage is 300 mg every 2 weeks, meaning that over the course of one year, costs add up to \$716,040.

6. Soliris - \$678,392

Priced at \$678,392 per year, <u>Soliris</u> 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 <u>Risk Evaluation</u> <u>Mitigation Strategy</u> (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.

7. Blincyto - \$672,968

The seventh most expensive drug, <u>Blincyto</u>, has an annual list price of \$672,968. 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 per vial is \$4,006.

8. Ravicti - \$664,092

Used to treat urea cycle disorders, <u>Ravicti</u> has an annual list price of \$664,092. 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, each with a list price of \$5,031.

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

9. Lumizyme - \$643,243

<u>Lumizyme</u> is used to treat Pompe disease, an inherited disorder that causes a buildup of complex sugars in the body. It affects the liver, heart, and muscles, and leads to muscle weakness and trouble breathing.

Patients must get Lumizyme from their doctor, and the drug is not available in pharmacies. Dosing for Lumizyme is based on a patient's weight; patients are given 20 mg of Lumizyme per kg of body weight through an IV infusion. For example, a person weighing 75 kg will likely use 60 vials a month of Lumizyme, each vial with a list price of \$824.67.

Like many drugs on this list, manufacturer Sanofi also has a copay assistance <u>program</u> to help patients save on their prescription.

10. Actimmune - \$633,325

Actimmune is approved to treat osteopetrosis and chronic granulomatous disease, rare disorders that cause the immune system to malfunction. Actimmune can be dispensed in a pharmacy, and patients typically use about 132 vials in a year, each with a list price of \$4,797.92.

Horizon Therapeutics offers a <u>patient assistance program</u> to help uninsured and low-income patients get Actimmune at no cost.

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Previous reports

• <u>March 2020</u>