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10 Most Expensive Drugs in the US

Understanding the Ultra Expensive Drugs

The Ultra Expensive Drugs. Who's paying?

With the entry of Zolgensma into the market in 2019, the prescription drug market changed almost instantly. The world's most costly drug carries a price tag of $2.125 million for a one-time treatment. Other ultra-expensive drugs, such as the self-administered Myalept cost more than $40,000 for a standard monthly supply when processed through a pharmacy; with many of these drugs prescribed for the duration of the patient's life. Drugs which are not processed through a pharmacy and are non-self-administered, including, infusions, cancer treatments, and gene therapies can cost well-over $100,000 per treatment. The question of whether to cover ultra-expensive drugs under pharmacy, medical, or at all, is now at the forefront of many employers' priorities when designing their pharmacy benefit plans.

To properly assess, employers should be well-informed as to what diseases these drugs can be prescribed for, what their annual cost for a typical course of therapy is, and their benefit-risk profile. Below is Confidio's ranking of the top ten most expensive drugs filled at pharmacy (self-administered) as well as prescribed through a healthcare practitioner (non-self-administered drugs) based on the drug's list price. Drug list price is defined as the pharmaceutical company assigned official drug price which is then adjusted for time to create a comparable benchmark.

Drug	Annual Cost
Zolgensma	$2,125,000
Carbaglu	$1,598,822
Procysbi	$1,085,160
Zokinvy	$1,032,480
Danyelza	$977,664
Myalept	$889,904
Luxturna	$850,000
Folotyn	$817,865
Amondys 45, Vyondys 53, Exondys 51	$748,800
Brineura	$730,340

1

Zolgensma ($2,125,000)

Zolgensma is one of the newest drugs on the market. For the third year running, it once again tops our list. With a list price of $2.125 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. Others, however, are citing it as a surprisingly cost-effective treatment as it can cure a once-incurable disease.

Zolgensma is approved to treat spinal muscular atrophy (SMA), a rare childhood disorder that causes muscular erosion which can lead to lung infections and muscle weakness. The incidence of SMA is relatively low at approximately 1 in 10,000 live births, but it is the leading genetic cause of infant mortality. Zolgensma is seen as potentially “curative” and a “one-time” therapy for infants treated early, as many children who have been treated with the drug now show no signs of the disease.

The question now posed by plan managers is how to best structure a pharmacy benefit plan to cost-effectively cover this life-saving drug? Some plans have been able to cover this expensive drug, as the manufacturer of Zolgensma allows certain insurers to pay for the drug in annual installments of $425,000 over five years. Unfortunately, cash-paying patients do not fall under the parameters of this negotiated price and are charged the full price of the drug.

2

Carbaglu ($1,598,822)

Carbaglu is used to treat hyperammonemia, a urea cycle disorder caused by lack of a certain liver enzyme. Carbaglu is usually given with other medications to treat this lifelong disorder. It lowers ammonia levels within 24 hours. While Carbaglu is an oral medication, it is only available from specialty pharmacies.

The incidence of urea cycle disorders in the United States is estimated at 1 in 25,000 live births. It should be noted that the pricing of Carbaglu is dependent on the weight of the patient, as it is dosed per kilogram.

3

Procysbi ($1,085,160)

Procysbi is used to help preserve kidney function and manage kidney damage and other problems in people with an inherited disorder that causes build-up of a certain natural substance (cystine) in the body (nephropathic cystinosis).
Cystine build-up can cause problems such as kidney problems, slow growth, weak bones, and eye problems.

Nephropathic cystinosis is an autosomal recessive metabolic disorder. It is a rare disease with a lifelong impact on the patient. The yearly incidence of nephropathic cystinosis is ~1:150,000 to 200,000 live births and its prevalence is ~1.6 per million population. We priced Procysbi based on a maintenance dose of 2100 mg per day, divided every 12 hours.

4

Zokinvy ($1,032,480)

Zokinvy is a drug used in patients one year of age and older to lower the risk of death in Hutchinson-Gilford Progeria Syndrome (HGPS). HGPS is a rare disease that causes premature aging. HGPS is reported to occur in 1 in 4 million newborns worldwide. Zokinvy is the first and only treatment approved for HGPS. Zokinvy is also used to treat certain types of Progeroid Laminopathies that are “processing-deficient”.

More than 130 cases have been reported in the scientific literature since the condition was first described in 1886. Most patients die before the age of 15 years from heart failure, heart attack or stroke. Compared to untreated patients, the lifespan of HGPS patients treated with Zokinvy increased by an average of three months through the first three years of treatment and by an average of 2.5 years through the maximum follow-up time of 11 years. Before Zokinvy’s approval, the only treatment options included supportive care and therapies directed towards the complications arising from the disease.

5

Danyelza ($977,664)

Danyelza was approved for medical use in the United States in November 2020. It is used to treat a certain type of cancer (neuroblastoma in bone or bone marrow). Danyelza belongs to a class of drugs known as monoclonal antibodies. It works by slowing or stopping the growth of cancer cells.

The incidence of neuroblastoma is 10.2 cases per million children under 15 years of age, and nearly 500 new cases are reported annually. Danyelza is given intravenously by a health care professional and is considered an orphan drug.

6

Myalept ($889,904)

Myalept is used to treat leptin deficiency in patients with generalized lipodystrophy syndromes. These syndromes are a rare body of diseases for which prevalence is currently estimated to be 1 to 5 cases per million people in the United States yearly. Myalept is used to treat a condition of abnormal fat distribution in the body.

It is self-administered once a day, and patients typically use 14 vials per month at a list price of $5,297 per vial, 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 options. To help alleviate some cost, Aegerion Pharmaceuticals offers a copay card to assist commercially-insured patients.

7

Luxturna ($850,000)

Luxturna is a gene therapy which treats an inherited form of retinal dystrophy; a condition that causes vision loss and even complete blindness. It is administered to patients with confirmed biallelic RPE65 mutationassociated retinal dystrophy which affects approximately 1,000 to 2,000 patients in the United States yearly.

It cannot be dispensed at a pharmacy as it requires a doctor to administer one vial of Luxturna into each eye at $425,000 per vial ($850,000 per treatment). Advantageously, patients should only need one dose of Luxturna in their lifetime.

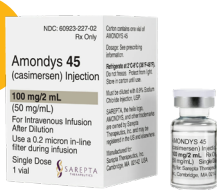
8

Folotyn ($817,865)

Folotyn is approved to treat peripheral t-cell lymphoma, a rare blood cancer that can cause death. The incidence of peripheral T-cell lymphoma is less than 1 case per 100,000 people in the United States. Folotyn is administered by a healthcare professional, and patients are typically given 45 vials of the drug annually. With a list price of $6,058 per vial, the annual cost is $817,615. It experienced a price increase of 3.4% in 2021.

Despite its high price tag, Folotoyn's effectiveness has been called into question. In fact, it has not yet been approved in the E.U. due to insufficient evidence of health gains.

9



Amondys 45, Exondys 51, Vyondys 53 ($748,800)

Amondys 45, Exondys 51, and **Vyondys 53** are indicated for the treatment of Duchenne Muscular Dystrophy (DMD) which is a rare progressive disease caused due to one of more than 2,000 mutations of the dystrophin gene. The disease leads to progressive loss of skeletal and cardiac function and ultimately death. Because all three are priced the same, and for the same indication, we opted to lump them together. All three are administered via intravenous infusion.

The prevalence of DMD is estimated to be 1 in every 3,500 live male births. Age of onset is usually between 3 and 5 years of age.

10

Brineura ($730,340)

Brineura (Cerliponase Alfa) is the first FDA-Approved treatment for a form of Batten Disease, the ultra-rare pediatric brain disorder CLN2, a rare genetic disorder that ravages the nervous system and can cause symptoms ranging from seizures to trouble coordinating muscles to vision loss. CLN2 affects fewer than one in a million people in the U.S., with 20 children born each year with the disorder. The affected children lose their ability to walk and talk by the age of six, and often die before they reach 12.

Brineura is administered into the cerebrospinal fluid (CSF) by infusion via a specific surgically implanted reservoir and catheter in the head. To support early testing for children who experience seizures, BioMarin in partnership with a commercial lab is offering a no cost genetic testing program called *Behind the Seizure*.

Research compiled from the following sources:

OptumRx Drug RxNews/RxHighlights; IPD Analytics; GoodRx.com; National Center for Biotechnology Information, U.S. National Library of Medicine; National Organization for Rare Disorders, Inc.; National Kidney Foundation; US Food & Drug Administration; and National Eye Institute.