Specialty Pharma:

Cost and Risk Management Opportunities

Written By Mary Paquette and Kristi Bohn

Specialty pharma – the insurance industry category of medication and treatments for rare, complex diagnoses that often require special handling and administration – has been growing at a record-breaking pace. Incentives are leveraging the latest technologies and spurring the development of treatments such as biologics, biosimilars, and gene- and cell-based therapies, which is increasingly challenging insurers' ability to predict, control, and manage the costs of these treatments.

Today's specialty drugs treat a wide and growing range of conditions and needs, from rare diseases to innovative and targeted treatments for cancers, inflammatory diseases, and more. This particular niche launched in earnest in 1983 with the passage of the Orphan Drug Act, which incentivized pharmaceutical companies to investigate treatments for diseases affecting fewer than 200,000 individuals in the U.S.

Additional incentives introduced over the years, including the Accelerating Rare disease Cures Program (ARC), established in 2022 by the U.S. Food & Drug Administration's Center for Drug Evaluation and Research, and the 2023 establishment of the Office of Therapeutic Products (OTP), which aims to improve oversight of and expertise in gene and cell therapies and other biologics, have also accelerated the development of these drugs.

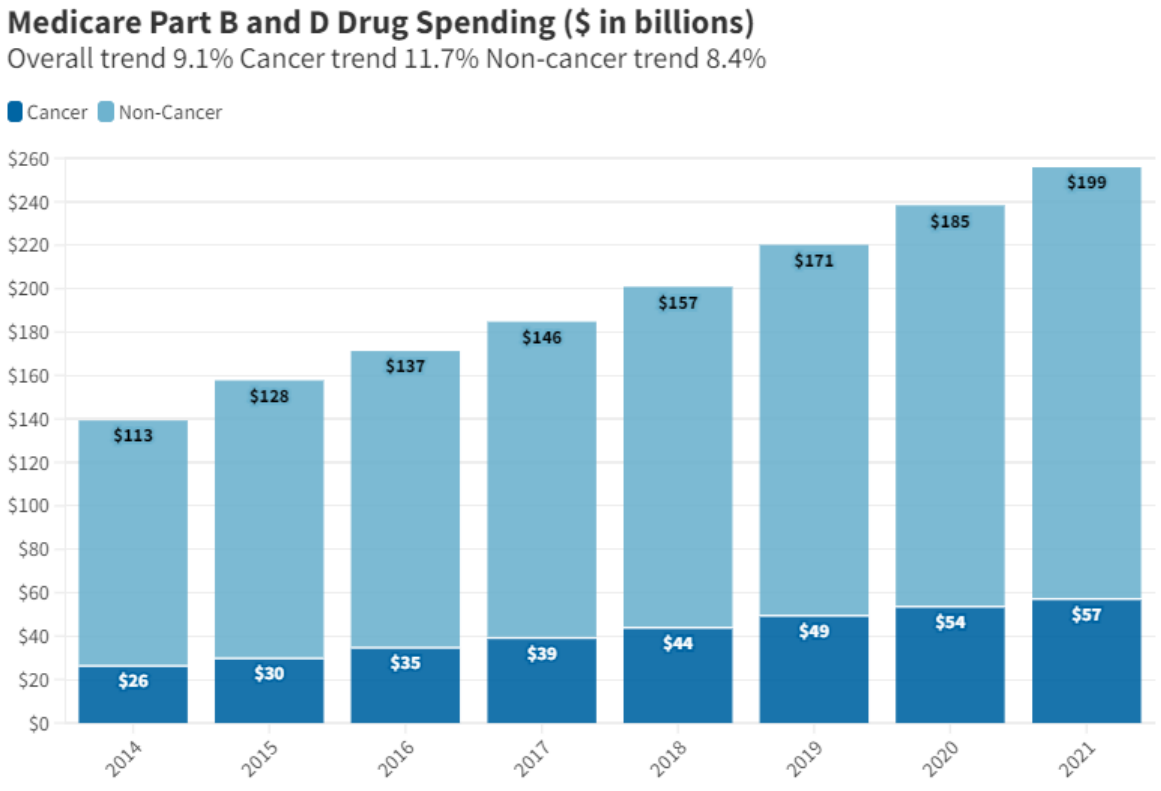
Not surprisingly, claims related to the rare and non-rare (but difficult to treat) conditions for which these drugs are being developed have been rising. In 2022, according to CVS Health Payor Solutions, 50% of new drug approvals were for drugs to treat orphan diseases, and at least half of all payor pharmacy costs currently derive from the 2.5% of insureds on specialty medications.

Hundreds of such new drugs are also currently underway for more than 13 conditions. CVS Payor Solutions is projecting that by 2025, 606 new drugs, 211 supplemental specialty pharmacy indications for existing drugs, 34 new gene therapies, and 30 biosimilars will have received FDA approvals. The specialty pharmacy market overall is also expected to grow at a compound rate of 35% in 2025. Much of the segment's growth, says specialty pharmaceutical consultancy AscellaHealth, will come from gene therapies, drugs for cancers (particularly blood cancers), autoimmune disorders and non-cancer blood disorders, and neurological disorders.

A CROWDED FIELD

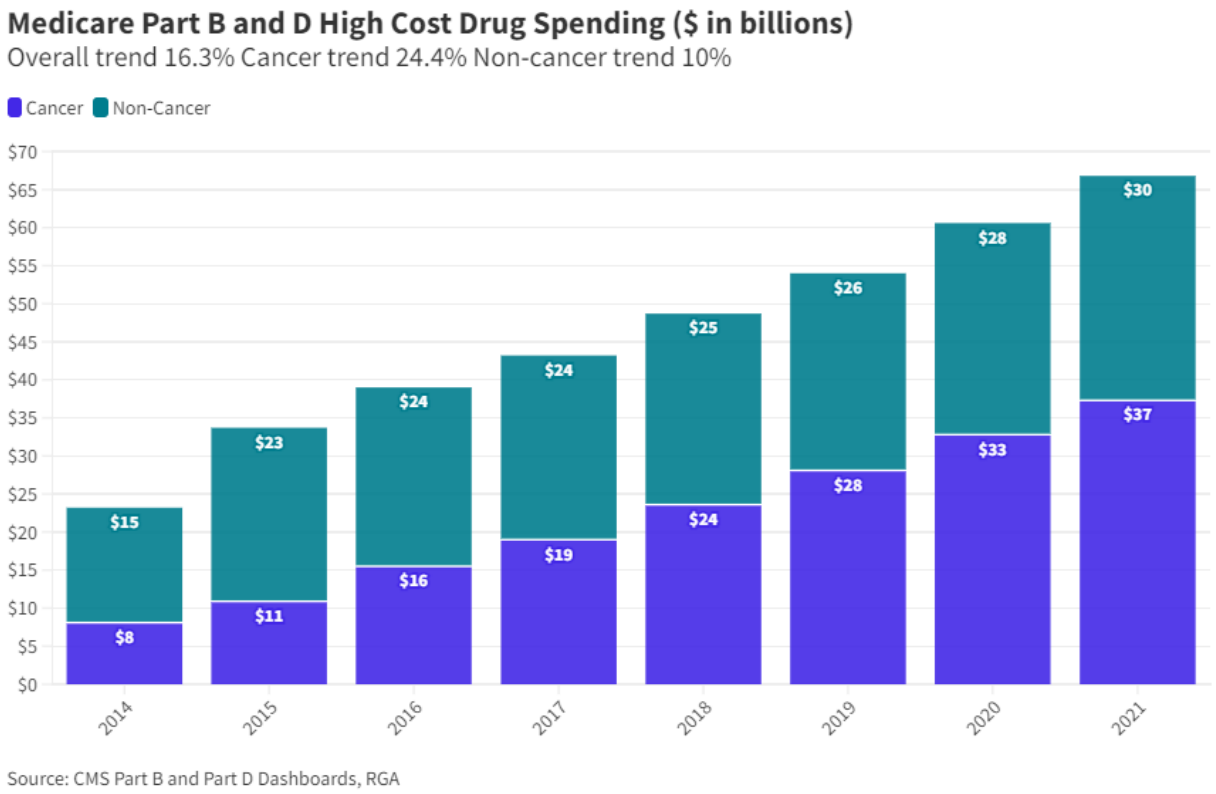
Many of the newest drugs are giving new hope to patients and their families, but in the past ten years, high list prices for these drugs have imposed significant financial risks for payors. Drug utilization data for Medicare Parts B and D from the Center for Medicare and Medicaid Services (CMS) from 2014 to 2021 (Chart 1) showed that annual costs for covering specialty and non-specialty cancer drugs rose by approximately 11.7%, compared with 8.4% for non-cancer drugs.

CHART 1: MEDICARE PART B AND D DRUG SPENDING, 2014–2021



Source: CMS Part B and Part D Dashboards, RGA

CHART 2: MEDICARE PART B AND D HIGH-COST DRUG SPENDING



Source: CMS Part B and Part D Dashboards, RGA

Both metrics include unit cost, utilization and enrollment trends but exclude the impact of drug rebates, which are not transparently reported to CMS and other stakeholders. Of these, the highest-cost cancer drugs, which we define here as typically costing commercial markets in excess of $100,000 per year or more, experienced an annual unit cost-plus-utilization-plus-enrollment trend of 24.4%. This particular trend drove the overall average increase in costs for the highest-cost specialty drugs to approximately 16.3%, versus the highest-cost non-cancer drug trend, which was about 10.0%.

While the cancer category is the leading one of continued high specialty pharma trends, certain other rare conditions also have high-cost drug treatments:

- **Hypophosphatasia** is a rare genetic disorder resulting in defective teeth and bone mineralization. Hypophosphatasia treatment Strensiq (asfotase alfa) is typically the highest-costing chronic treatment. In 2023, a course of treatment with Strensiq could cost around $2.5 million. Crysvita, which treats x-linked hypophosphatasia, has typical annual cost levels of around $500,000.
- The many **hemophilia A and B** factors on the market have annual costs that typically range from $400,000 to over $1 million. The recently approved one-time gene therapies – Hemgenix (etranacogene dezaparvovec-drlb) for hemophilia B and Roctavian (valoctocogene roxaparvovec) for hemophilia A, have list prices of $3.5 million and $2.9 million, respectively. New hemophilia treatments also include lifestyle-improving features such as extended-release so patients may require less frequent treatments, which could lower long-term costs despite the higher unit price for these extended-release alternatives.

- Other rare blood clotting disorders, such as **Von Willebrand disease**, continue to have very high-cost treatments, such as Tretten ($1.2 to $1.4 million annually), Coagadex ($1.1 to $1.3 million annually), and Vonvendi ($500,000 to $700,000 annually).

- **Lipodystrophy** is a rare syndrome that causes a person to lose fat from portions of their body and possibly gain abnormal fat deposits in other parts of the body. The lipodystrophy treatment Myalept (metreleptin) typically costs more than $1.2 million for a full-year treatment course.

- Four of the multi-dose treatments for **Duchenne's muscular dystrophy**, one of the most severe forms of inherited muscular dystrophies – Exondys 51 (eteplirsen), Vyondys 53 (golodirsen), Amondys 45 (casimersen), and Viltepso (viltolarsen) – are dosed by patient weight, which means costs can vary widely. Typical one-year costs can range from $700,000 to $2.3 million annually. The one-time gene therapy option Elevidys (delandistrogene moxeparvovec-rokl), approved for ages 4 and 5, has a $3.2 million list price.

- A treatment for **Progeria**, the rare syndrome that causes children to age rapidly, Zokivny (lonafarnib), typically costs more than $900,000 for a full-year course of treatment.

- **Pompe disease**, the glycogen-related condition that causes severe muscle weakness and wasting, has several treatment choices with annual costs ranging from $350,000 to up to $1 million. The lowest to highest cost treatments are: Myozyme (alglucosidase alfa), Nexviazyme, (alglucosidase alfa), Pombliti (cipaglucosidase alfa-atga) plus OPFOLDA (miglustat), and Lumizyme (alglucosidase alfa).

- Patients with **generalized myasthenia gravis**, a disorder characterized by drooping eyelids and facial weakness and fatigue, who have become resistant to corticosteroids or can no longer tolerate their side effects, can seek certain higher-cost treatments. Annual charges for Ultomiris (ravulizumab-cwvz) and Soliris (eculizumab), two frequently used treatments, can range from $400,000 to $750,000, with Ultomiris on this lower end of the range despite its more convenient delivery regimen. Newer treatments available in the U.S. are Vyvgart (efgartigimod), Rystiggo (rozanolixizumab-noli), and Zilbrysq (zilucoplan), which are expected to fall near or below the annual cost of Ultomiris.

- Patients with the blood diseases **paroxysmal nocturnal hemoglobinuria** (PNH), **atypical hemolytic uremic syndrome** (aHUS), or the inflammatory disease **neuromyelitis optica spectrum disorder** (NMOSD) who use Soliris often incur high claims costs. Ultomiris is less costly and more convenient to use and is also used for PNH and AHUS. Competition in these conditions is expected to continue as more drugs are approved and more patients change drugs.

- Treatments for **hereditary angioedema**, which causes rapid swelling of the hands, feet, limbs, or face, can range in cost from $500,000 to $1 million annually. They include: Firazyr (icatibant injection), Orladeyo (berotralstat), with Haegarda (C1 esterase inhibitor subcutaneous [human]), Berinert (C1 esterase inhibitor [human]), Cinryze (C1 esterase inhibitor [human]), Takhyzyro (lanadelumab-flyo), Ruconest (C1 esterase inhibitor [recombinant]), and Kalbitor (ecallantide).

In terms of cancer, the highest-costing patients usually receive escalating treatments ending with bone marrow transplants, which sometimes incur adverse reactions requiring further hospitalization. In addition, the costs of cell therapies (also known as CAR Ts), which are increasing in access and use, are akin to

those for bone marrow transplantation, but have further upward financial pressures due to the costs of the CAR T treatment itself.

That said, it is relatively rare to encounter specialty drugs for cancer with costs at the extreme levels seen in the non-cancer conditions. High and rising cancer drug cost levels have, however, recently been experienced for the following:

- Elzonris (tagraxofusp-erzs), a treatment for **blastic plasmacytoid dendritic cell neoplasm**, a rare form of acute leukemia, may cost approximately $3 million for an entire year course
- Kimmtrak (tebentafusp-tebn), a treatment for the eye cancer **uveal melanoma**, may cost nearly $1 million for a full-year course
- Folotyn (pralatrexate) for **relapsed or refractory peripheral t-cell lymphoma** may cost more than $800,000 for a full-year course
- Demser (metyrosine), for symptoms of **pheochromocytoma** (adrenal gland tumors), may cost over $800,000 for a full-year course. A patient who also uses Dibenzyline (phenoxybenzamine) may incur claims costs as high as $1 million.

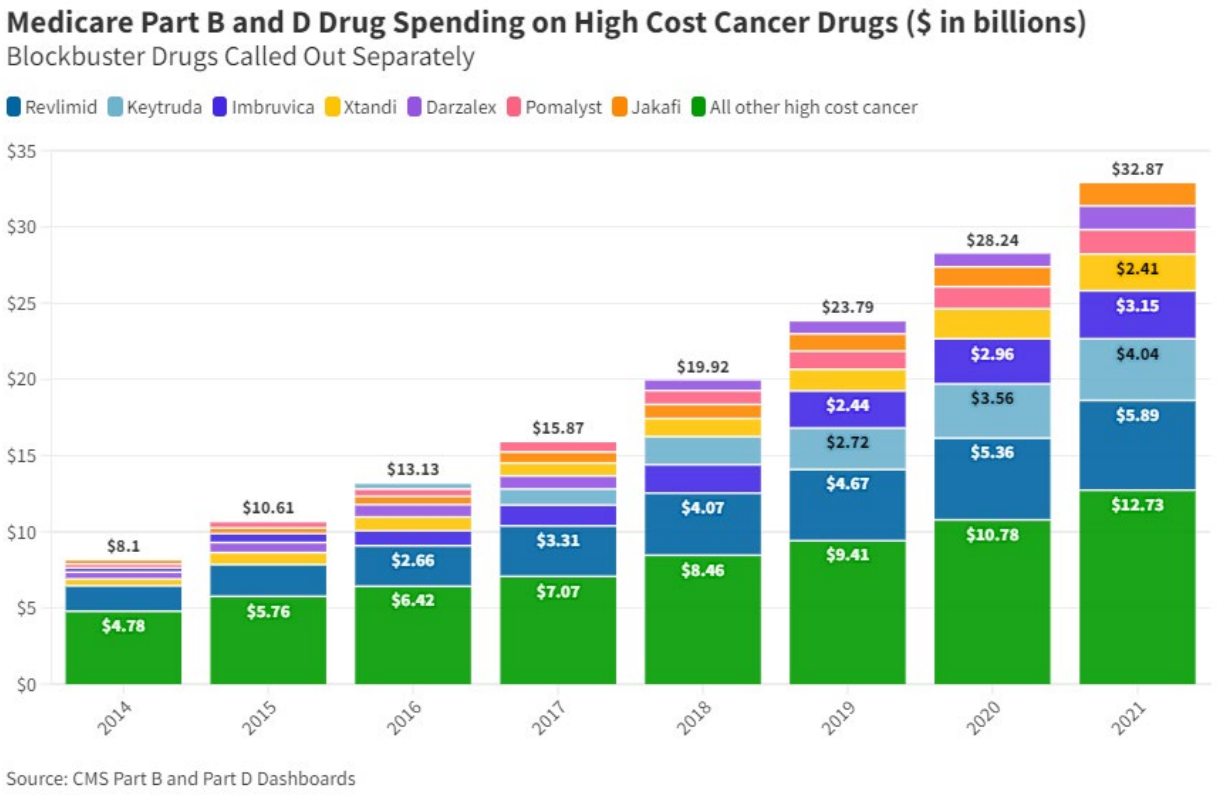


Chart 3: Medicare Part B and D Drug Spending on High-Cost Cancer Drugs, 2014-2021

Chart 3: This chart demonstrates how Medicare spending on ten 10 cancer drugs and other costly cancer drugs – those that typically cost over $100,000 per year per patient commercially – are playing a material role in elevating U.S. healthcare spending. Biosimilar introductions, competition, and Medicare's IRA negotiations may succeed in dampening the current cancer specialty drug spend trajectory.

From RGA's experience with high-cost claims, cancer drugs have higher overall trends due to several factors:

- Higher annual costs for newly approved cancer drugs
- Higher unit cost trends
- More use of drugs to treat cancer in place of surgeries, radiation, and chemotherapy

How can health plans mitigate these costs and risks?

When cases have high excess drug claims, there are strategies to make sure that the health plan is maximizing value:

- **Curating formularies.** Biosimilars and generics are often available to compete directly with certain drugs. Once approved, direct writers should ensure that their formularies adopt generics and biosimilars quickly to make lower-cost alternatives available and encourage price competition. Plan design incentives are also recommended to encourage patient use of lower-cost alternatives, such as lower co-pay tier placement. Sometimes, cutting high-cost brand names from a formulary is worthwhile. For example, candesartan is generically available to treat moderate blood pressure, but brand-name candesartan versions can cost eight to 15 times more than the generic, unnecessarily costing some health plans tens to hundreds of thousands of dollars a year.

- **Address the "where" for certain drugs.** Health plans should examine whether certain drugs are optimally accessed through a retail pharmacy, a specialty pharmacy, or certain hospitals, as the same drug can cost drastically more when administered by certain providers. This is where benchmarking can help health plans cut costs.

- **Look for and react to atypically high charges.** Outlier analysis is a powerful tool for identifying bad actors, human error, or claims system errors. In one case RGA recently encountered, a patient's cost for post-transplant immunotherapy was eight times more than normal but was corrected by working with the third-party transplant network vendor.

- **Watch J Code drug costs.** While Congress and state legislators have increased their scrutiny of pharmacy benefit managers over the past two years, more outlier drug claims are being encountered among physician- and facility-billed drugs (J Code drugs) than retail drugs. Sometimes, these egregious cases should imply network exclusion consequences for certain providers or facilities, or at least provider contract implications and intermediary remedies.

- **Watch General J Codes.** When first approved, most non-retail drugs are billed using the general J codes J3490, J3590, or J9999 until a specific code is assigned. As general codes are usually not flagged by claims systems, charge levels net of discounts can be incorrectly high. Health plans would benefit from reviewing general J Code claims, particularly high-dollar ones.

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MANAGING THE RISK

What is clear is that planning for and executing specialty drugs requires strong and careful oversight. New high-cost treatments and therapies are coming faster than ever and cases with excessive costs are increasing, which is challenging portfolio management.

Most important? Everyone is looking for a magic bullet. Unfortunately, to date, no one has found one yet. For payors, tightening basic practices and relying on good cost and utilization management is the best bet.

Strong prior authorization will validate the right drug for the patient and the most cost-effective treatment administration site. Many specialty drugs can be safely administered in the patient's home rather than at a clinic or hospital, where costs are much higher.

Monitoring for generic J codes that may slip past claim system flags, allowing for higher-than-usual payments, can also help control specialty pharm costs. ■

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