



2018

Specialty Drug Trends

TREND HIGHLIGHTS

- 59 total FDA-approved novel drugs in 2018
- 44 specialty drug approvals in 2018
- Specialty drug costs trended upward 10% (2016 to 2017)
- Branded specialty drugs account for roughly 1-3% of branded drug prescriptions filled, but comprise approximately 30% of total branded drug spending
- Of the 44 specialty drug approvals, 19 were Orphan Drugs

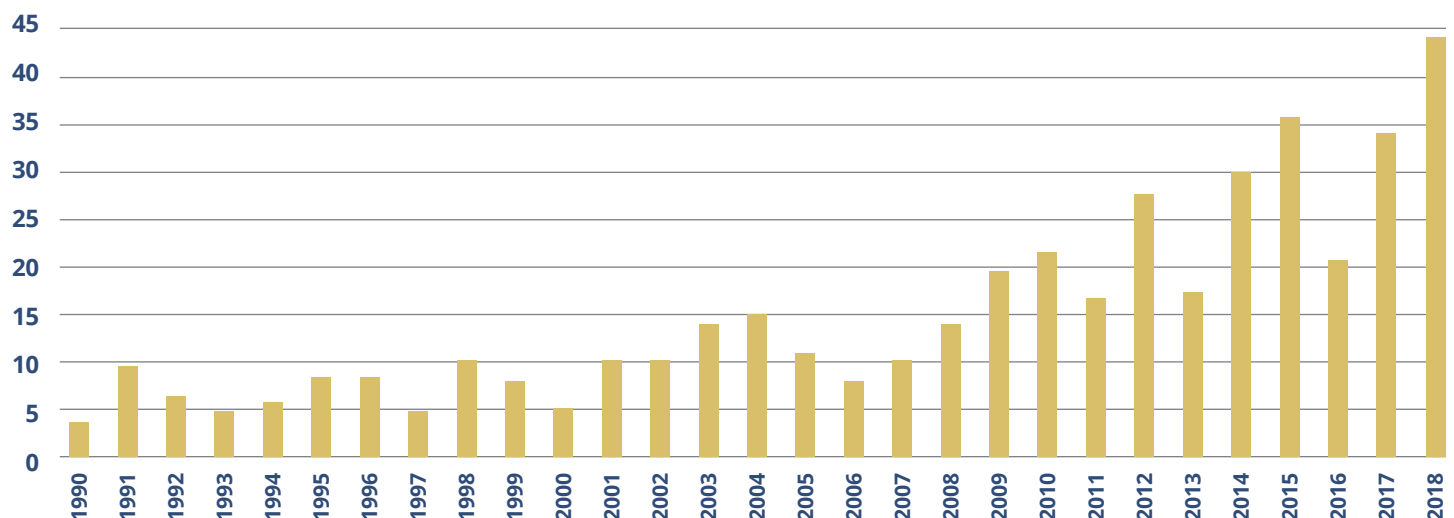
Specialty Drugs

Specialty drugs¹ continue to skyrocket by FDA approvals as well as price. In 2018, high dollar specialty drugs accounted for 44 of the total 59 FDA-approved novel drugs.

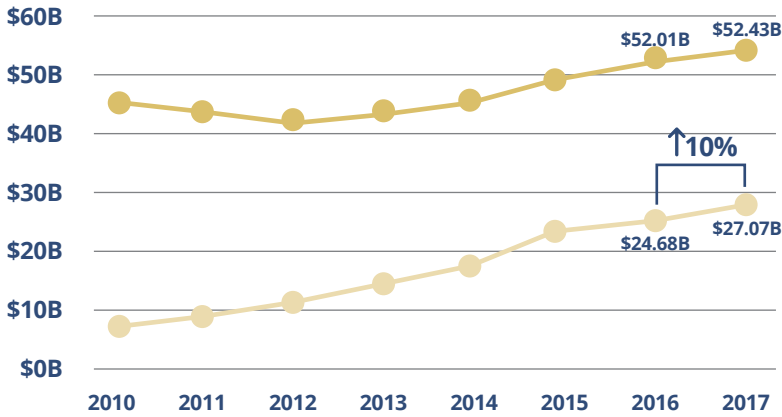
NPR reported on a recent study which found that specialty drug prices rose 21 percent for oral medications and 13 percent for injectable drugs from 2008 to 2016 (based on list prices). Expensive new therapies entering the market have been the predominant factor in pushing specialty pharmaceutical costs as a whole skyward. Overall, their prices have risen 13 times faster than national inflation.

On a state level, the California Department of Managed Health Care (DMHC) just released its Prescription Drug Cost Transparency Report. One of its key findings stated that while specialty drugs accounted for only 1.6 percent of all prescription drugs, they accounted for over half (51.5 percent) of total annual spending on prescription drugs. In addition, specialty drugs made up a mere 1 percent of the top 25 most frequently prescribed drugs, and yet accounted for 25.4 percent of total spending.

Since 2000, the number of specialty drug approvals has increased by 780%.

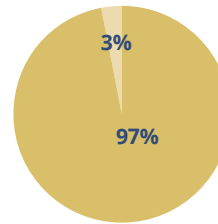


Spending

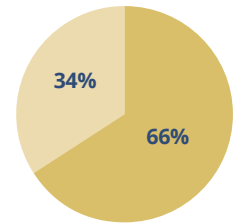


■ Branded Specialty Drugs ■ Branded Non-Specialty Drugs

Total Prescriptions



Share of Spending



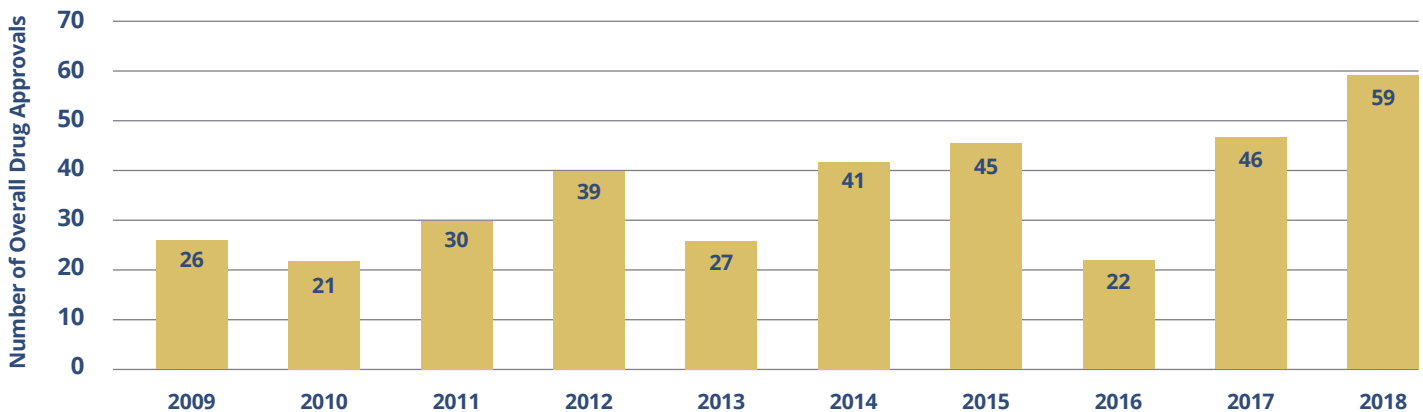
2017 Health of America Report: Prescription Drug Costs Trend Update © 2018 Blue Cross Blue Shield Association. All Rights Reserved.

According to a Blue Cross/Blue Shield (BCBS) prescription drug report, branded specialty drugs made up a small share of the total branded prescription pharmaceuticals that were filled in 2017 at just 3 percent. However, they accounted for 34 percent of branded drug spending. Specialty drugs are the fastest growing branded drug type in terms of spending—rising 10 percent in just the past year². Of the top 30 drugs BCBS spends the most on nationally, over 2/3 are specialty pharmaceuticals.

FDA Approvals

The FDA approved 59 new drugs in 2018, beating the record of 53 set in 1996. The list of novel drugs—which encompasses new molecular entities (NMEs) and new therapeutic biologics under Biologics License Applications (BLAs)—does not include approximately 30 specialty drugs that were approved for additional diseases/conditions.

CDER’s Annual Novel Drug Approvals: 2009-2018



Data from FDA’s Center for Drug Evaluation and Research (CDER)

Another significant detail: in this year alone, the FDA nearly doubled its roster of biosimilar drugs. These products are very similar, but not identical, to a reference biologic product. Seven new biosimilars received approval this year. Since 2015, only nine biosimilars had been approved, so the total now stands at 16. More competition in the marketplace certainly is a good thing. Better access to therapies and lower costs usually are the result of a wide variety of available products -- "available" being the key word here. That's because despite getting FDA approval, nine of the 16 biosimilars have not been commercially launched in the US as of December 31, 2018. Most delays are due to complex patent issues pertaining to biosimilars that result in lengthy litigation.

Critics have warned that issues of safety and efficacy can be overlooked in the haste of bringing new drugs to market. Thankfully there have been no such issues reported. So far.

One more notable point: researchers are implementing a wider variety of methods in order to defeat diseases. Roughly 15% of 2018's new drugs are not conventional small molecules or biologics. Rare, genetic diseases are being targeted more than ever. As you'll see in the next section, there's a reason for that.

Orphan Drugs

Thirty-four of the 59 CDER approvals were for Orphan Drugs. Of the 44 approved specialty drugs, 19 were designated Orphan Drugs. Orphan Drugs remain a hot button topic in the industry. Because the disorders they treat are rare by definition (usually with patient populations below 200,000 per year) drug manufacturers in the distant past tended to not devote time and resources to them. And, of course, because they weren't profitable.

"In the thirty-five years since the passage of the Orphan Drug Act (ODA) in 1983, the structure of development incentives laid out in the legislation has successfully spurred investment and innovation in rare disease therapies."³ That's the good news.

Here's the bad: Drug makers exploited loopholes in the ODA to massive financial gain. "A number of pharmaceutical companies gamed the system to sell orphan drugs at astronomical prices by using two key strategies: repurposing commonly used drugs and getting approval to use one product for multiple orphan diseases."⁴

Moreover, "in some cases, FDA reviewers failed to show they had checked how many patients could be treated by a drug being considered for orphan drug status; instead, they appeared to trust what drugmakers told them."⁵

Big Pharma hit the Orphan Drug trifecta thanks to the ODA's lengthy exclusivity rights, their own exorbitant price strategies, and lax oversight at the FDA. And still, approximately 95% of the approximate 7,000 total rare diseases remain without any therapeutic options.

Drug Costs

Advanced Medical Strategies (AMS) keeps tabs on all specialty drugs—including those with Orphan designations—in **PredictRx**, its subscriber-based directory that evaluates drug costs and dosing regimens.

For 2018, all the Top 5 most expensive drugs per dose based on AMS Cost Projection (ACP) ⁶ were Orphan Drugs. Luxturna topped the list with an ACP of over a half million dollars per dose. As for non-Orphan drugs, Orfadin's ACP doubled its 2nd place competitor at \$150K per dose.

2018 Most Expensive Specialty Drugs – Overall (Per Dose)

Name	Diagnosis	ACP Price (per Dose)
Luxturna	Inherited Retinal Dystrophy (IRD)	\$566,470.75
Kymriah	Acute Lymphoblastic Leukemia (ALL)	\$547,200.00
Yescarta	Non-Hodgkin Lymphoma (NHL)	\$519,216.00
Spinraza	Spinal Muscular Atrophy	\$174,060.30
Azedra	Pheochromocytoma/Paraganglioma	\$96,021.50

2018 Most Expensive Specialty Drugs – Non-Orphan (Per Dose)

Name	Diagnosis	ACP Price (per Dose)
Orfadin	Treatment of Alkaptonuria	\$151,841.65
Brineura	Treatment of Batten Disease	\$74,972.62
Provenge	Treatment of Prostate Cancer	\$67,976.51
Zevalin	Non-Hodgkin's Lymphoma (NHL)	\$64,915.54
Cuprimine	Treatment of Mercury Poisoning	\$58,283.71

Utilizing ACP to estimate the most expensive specialty drug on a per year basis,
Revcovi took the #1 spot for both Adult and Pediatrics.

2018 Most Expensive Specialty Drugs – Adult (Yearly ACP)

Name	Diagnosis	Orphan	ACP Yearly Price (Adult)
Revcovi	Severe Combined Immune Deficiency (SCID)	N	\$8,265,793.54
Feiba NF	Routine Prophylaxis in Hemophilia A and B	Y	\$3,168,874.80
Exondys 51	Duchenne Muscular Dystrophy	Y	\$2,892,851.52
Strensiq	Hypophosphatasia	Y	\$2,592,356.83
Vimizim	Mucopolysaccharidosis Type IVA (MPS IVA)	Y	\$2,466,932.83

2018 Most Expensive Specialty Drugs – Pediatric (Yearly ACP)

Name	Diagnosis	Orphan	ACP Yearly Price (Pediatric)
Revcovi	Severe Combined Immune Deficiency (SCID)	N	\$3,542,482.94
Demser	Management of Pheochromocytoma	N	\$2,437,270.27
Feiba NF	Routine Prophylaxis in Hemophilia A and B	Y	\$1,690,066.56
Acthar (HP Acthar Gel)	Treatment of Nephrotic Syndrome	N	\$1,465,048.10
Ravicti	Urea Cycle Disorders (UCD)	Y	\$1,423,605.77

Have Questions? Contact Derek Moore at 781.224.9711 Ext. 115 or Dmoore@mdstrat.com today to get started.

Advanced Medical Strategies
 855.224.9711 | www.mdstrat.com

Drug Cost Trends

PredictRx features a Drug Trend graph that tracks quarterly pricing data based on ACP as well as AMS Risk Threshold (ART).⁷ End of year analysis reveals that over the past year Marqibo and Ravicti were the biggest gainers per dollar for Adults and Pediatrics respectively, while the price for Gleostine had increased the most on a percentage basis.

2017-18 Specialty Drug Trends – Adult (Yearly ACP Dollar Increase)

Name	Diagnosis	Orphan	ACP Yearly Adult Change Dollar
Marqibo	Acute Lymphoblastic Leukemia (ALL)	Y	\$87,562.80
Beleodaq	Treatment of Peripheral T-cell Lymphoma (PTCL)	N	\$69,325.73
Krystexxa	Treatment of Gout	Y	\$56,505.95
Proleukin	(Multiple Treatments)	N	\$46,541.38
Strensiq	Hypophosphatasia	Y	\$45,287.42

2017-18 Specialty Drug Trends – Adult (Yearly ACP Percent Increase)

Name	Diagnosis	Orphan	ACP Adult Change Percent
Gleostine	Treatment of Brain Tumors	N	28.7208%
Lupron Depot	Prostate Cancer	N	26.9045%
Mitomycin/ Mitomycin C	Treatment of Gastric Cancer (Stomach Cancer)	N	21.6758%
Matulane	Treatment of Anaplastic Oligo-dendrogloma/Oligoastrocytoma	N	18.3913%
Bleomycin	Treatment of Non-Hodgkin Lymphoma (NHL)	N	18.2367%

2017-18 Specialty Drug Trends – Pediatric (Yearly ACP Dollar Increase)

Name	Diagnosis	Orphan	ACP Yearly Pediatric Change Dollar
Ravicti	Urea Cycle Disorders (UCD)	Y	\$110,515.26
Actimmune	(Multiple Treatments)	N	\$73,493.97
Sabril	Infantile Spasms	Y	\$25,863.25
Yervoy	Melanoma	Y	\$21,202.37
Sprycel	Chronic Myeloid Leukemia (CML)	Y	\$19,359.46

2017-18 Specialty Drug Trends – Pediatric (Yearly ACP Percent Increase)

Name	Diagnosis	Orphan	ACP Pediatric Change Percent
Gleostine	Treatment of Brain Tumors	N	28.7208%
Matulane	Treatment of Hodgkin Lymphoma	N	17.998%
Intron A	(Multiple Treatments)	N	17.3834%
Lupron Depot	Central Precocious Puberty	Y	16.4219%
Supprelin LA	Central Precocious Puberty	Y	14.3596%

Drug Views

For the 2018 Top 5 viewed drugs in PredictRx, AMS pinpointed Opdivo as the one its members inquired about the most during the past 12 months (1416 views). Industry professionals should be particularly mindful about this drug and the four others when addressing high dollar claims going forward in 2019.

A more comprehensive data sheet detailing the [PredictRx Top 5 Researched Specialty Drugs of 2018](#) can be accessed from the link.

2018 Top 5 PredictRx Views

Drug	2018 Views
Opdivo	1416
Remicade	1327
Keytruda	1304
Rituxan	1215
Humira	1207

2017-18 Top 5 Increased PredictRx Views

Drug	2018 Views
Cuvitru	3033%
Luxturna	800%
Krystexxa	794%
Imfinzi	745%
Ethylol	533%

Among the PredictRx drugs that saw the biggest increase in views from last year Cuvitru soared a whopping 3000%.

Diagnosis Views

For every drug there is a diagnosis that it aims to treat. Sometimes a drug treats multiple indications. Since the two are inexorably linked we have provided in this section (as we did last year) a brief summation of the most viewed diagnoses in [PredictDx](#), AMS' software solution for delivering in-depth clinical information, empirical insight and key financial analytics for the costliest medical diagnoses.

For the 2018 Top 5 viewed diagnoses in PredictDx, Stage I/II/III Breast Cancer was viewed the most during the past 12 months. Here's the companion data sheet to PredictRx which details the [PredictDx Top 5 Researched Diagnoses of 2018](#).

2018 Top 5 PredictDx Views

Drug	2018 Views
Early Stage/Locally Advanced Breast Cancer (Stages I/II/III)	16369
Chronic Renal Failure/End-Stage Renal Disease (ESRD)	13149
Dilated Cardiomyopathy/Congestive Heart Failure (CHF)	7234
Colon Cancer/Cancer of the Appendix	5946
Prostate Cancer	5838

2017-18 Top 5 Increased PredictDx Views

Drug	2018 Views
Diabetes Mellitus (Type I/Type II)	842%
Autoimmune Hepatitis	256%
Giant Cell Arteritis	243%
Bipolar Disorder	237%
Large Bowel/Intestine/Colon/Rectum/Anus Atresia	233%

The diagnosis of Type I/II Diabetes increased the most from the previous year, nearly a 900% jump in views.

Specialty Drug List

Members of Advanced Medical Strategies receive a Newly Predicted email notifying them whenever a specialty drug gets approved by the FDA and is added to the AMS PredictRx directory.

Newly Predicted is a fact sheet which provides the most relevant datapoints about each new drug. First and foremost, Quantity for Cost and ART pricing are listed. Information includes an overview of the drug, physician comments most pertinent to its specific treatment area, available dosages, drug category, HCPCS/J Codes, and Line of Therapy (LOT). It also contains a brief synopsis about its associated diagnosis from PredictDx.

Click below to view all the 44 high dollar pharmaceuticals (listed in chronological order of when approved) that have been added to Advanced Medical Strategies' comprehensive specialty drug directory, PredictRx, in 2018. Drug indications that have n/a in the ACP column have not launched as of press time. If there is n/a in the Yearly ACP column that means those costs cannot be accurately calculated or projected.

[Download the list of 44 High Dollar Pharmaceuticals.](#)

Transparency and clarity are essential for payers within the health and casualty industries to make informed, proactive business decisions concerning specialty drugs, particularly when it comes to pricing, dosing, and treatment analytics. With hundreds of thousands of searches within PredictRx and PredictDx, and associated usage of their respective statistical reporting functionalities, it's clear that the on-demand decision support solutions they both provide have become the gold standard.

¹ There are no current established criteria defining what makes a drug a specialty pharmaceutical. However, Medicare defines any drug for which the negotiated price is \$670 per month or more as a specialty drug.

² <https://www.bcbs.com/the-health-of-america/reports/prescription-drug-costs-trend-update>

³ <https://www.iqvia.com/institute/reports/orphan-drugs-in-the-united-states-growth-trends-in-rare-disease-treatments>

⁴ <https://www.the-scientist.com/features/how-orphan-drugs-became-a-highly-profitable-industry-64278>

⁵ <https://khn.org/news/government-investigation-finds-flaws-in-the-fdas-orphan-drug-program/>

⁶ ACP denotes the usual costs of a given drug within the U.S. healthcare system. This proprietary benchmark is calculated using federally contracted rates for government agencies, incorporating amounts paid by any private-sector purchaser for the pharmaceutical. Discounts, rebates, chargebacks, and other fiscal adjustments are all factored into the price. Simply put, ACP is the expected cost for a particular drug dosage amount.

⁷ ART, an AMS proprietary benchmark, represents a fiscal red line of cost tolerance. ART is the absolute maximum specialists should tolerate as billed on a claim.