

October 2017

Orphan Drugs in the United States

Providing Context for Use and Cost



Introduction

There are approximately 7,000 rare diseases affecting 25 to 30 million people in the United States. Children make up more than half of those afflicted. Many of these diseases, or conditions, are life-threatening or life-limiting. Patients and caregivers in the rare disease community face many obstacles; one of the most challenging is that few people, including doctors, have heard or know anything about them. Further, treatments are available for just 5% of the estimated 7,000 diseases. Confronted with these unique challenges, the rare disease community avidly works to raise awareness and educate medical professionals and elected officials, as well as funding and supporting research to develop treatments and cures.

The purpose of this report is to provide a historical perspective on the characteristics of rare diseases and their treatment and the role of the Orphan Drug Act of 1983 in advancing rare disease medicines. It describes the characteristics of orphan drug volumes and prices, as well as placing orphan drugs in the context of overall specialty drugs and medicine spending levels and growth.

The research in this report was undertaken independently by the QuintilesIMS Institute, with funding from the National Organization for Rare Disorders (NORD). The contributions of Alana Simone and others at QuintilesIMS are gratefully acknowledged.

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Executive summary

The burden of living with a rare disease is significant, with millions of Americans affected. The number of recognized rare diseases in the United States has risen in recent years and will continue to do so as a result of a combination of scientific advances, such as advancements in genetics and molecular biology and the use of biomarkers to identify diseases, as well as a growing commitment of policy makers to adopt precision medicine.

Orphan drugs can be expensive with an inverse relationship between price and their volume of use, given that they are developed for small patient populations. Although the median annual cost for an orphan drug in 2016 was over \$32,000, the top ten therapies used by the greatest number of patients averaged less, at \$14,909.

Drug spending in the United States is evolving from an emphasis on high-volume, low-cost drugs for chronic diseases toward drugs with lower volumes and higher patient outcomes value, as a result of a combination of scientific advances and a growing emphasis on precision medicine. Other dynamics affecting drug spending in the United States at this time include the introduction of speciality formulary tiers, greater use of health plans involving pharmacy deductibles and patient co-pays, expanded use of specialty pharmacies and limited distribution networks and strengthened negotiating power of pharmacy benefit managers, resulting in greater financial burden for patients.

Of the 449 orphan drugs approved as of 2016, 351 had only orphan indications and 98 had both orphan and non-orphan indications. Of the 98, 54 received a non-orphan indication first and 10 received both orphan and non-orphan indications simultaneously. The orphan drug share of total volume of pharmaceutical use in the United States has declined from a peak of 0.6% in 2003 to 0.3% in 2016.

Of the total drug sales of \$450 billion in the United States in 2016, almost 60% was from non-orphan traditional drugs while one-third was spent on non-orphan specialty drugs. The remaining 7.9% of spending was for orphan indications of approved orphan drugs. During the past five years, while the number of orphan drugs approved has increased from 315 to 449, the share of spending has increased more moderately and these drugs represent only a small part of the overall medicine budget.

Historical perspective and evolution

Rare diseases are serious, chronic illnesses that can become progressively disabling and can limit life expectancy. Although rare diseases are uncommon by definition, in aggregate the number of people with rare diseases is not insignificant: it is estimated that approximately 7% of the population in the developed world have a rare disease and that the number is increasing.¹ In the United States, the National Institutes of Health (NIH) estimates that between 25 million and 30 million people suffer from rare diseases – defined as those affecting fewer than 200,000 people.^{2,3}

As of 2016, there were unique identifiers for 6,084 rare diseases and information on 3,715 genes associated with these rare diseases, although estimates for the number of actual rare diseases could reach up to 7,000.⁴ Some conditions can affect only a handful of people, while others may affect hundreds or thousands, such as cystic fibrosis, which affects approximately 30,000 people in the United States.⁵

Rare diseases are often the result of a genetic mutation; one estimate is that 80% of rare diseases are genetic in nature.⁶ Other causes include exposure to infections or toxins, autoimmune response and adverse responses to therapeutic interventions. Of note is that 50% of patients with rare diseases are children.

The number of reported rare diseases continues to climb. In 2009, there were 5,857 cataloged rare disorders. This number rose to 6,084 in 2016, a rate of approximately 30 new rare diseases per year.^{4,7} A number of factors have contributed to an increase in the number of rare diseases in the past decade. First, there have been noteworthy scientific advances that have allowed for segmentation of disease. For example, the improved understanding of signaling pathways involved in oncogenesis, such as RAS mutations and how they affect the MAPK signaling pathways, have allowed stratification of cancers into specific diseases among unique subsets of patients. A greater understanding and identification of mutations affecting key enzymes in lipid/lysosomal storage disorders has also allowed for greater stratification (e.g., Niemann-Pick disease types A, B, C) as well as greater understanding of the inflammatory pathways in rheumatologic disorders (e.g., familial Mediterranean fever types 1 and 2).

Another trend that has contributed to the increase in the number of rare diseases is the use of biomarkers to further segment diseases into more specific and smaller patient populations. From 2009–2015, 16% of orphan-designated drugs were based on predictive biomarkers that split a disease into more rare subsets.⁸ One example of biomarkers segmenting a disease is in lung cancer, which comprises about 250 thousand new cases per year in the United States, of which non-small cell lung cancer (NSCLC) represents approximately 85% of cases. Approximately 40-80% of NSCLC patients have epidermal growth factor receptor (EGFR) mutations; this segments the population of patients with the EGFR population to approximately 75 to 150 thousand patients per year.⁹ Similarly, patients with cystic fibrosis can be sub-divided into smaller pools by genetic mutations that then represent unique pathways to treatment.

HISTORICAL PERSPECTIVE AND EVOLUTION

Looking ahead, a broad set of major scientific advances are expected to lead to more diseases being identified, diagnosed and treated, resulting in an increase in the number of diseases able to be defined in terms of patient populations of less than 200,000. The continued uptake of next generation sequencing (NGS) and prioritization of genome-wide studies will contribute towards this trend. Already a number of countries have invested in precision medicine initiatives that involve the widespread genetic screening of large populations, such as the All of Us precision medicine initiative in the United States, which seeks to recruit one million participants to share their genomic and other personal health data, and the United Kingdom's 100,000 Genomes Project. In particular, the 100,000 Genomes Project aims to include rare disease patients and their relatives along with sequences of patients without rare diseases, which will help to identify genetic causes of rare diseases.¹⁰ Other advances in genomics, including but not limited to gene expression analysis, epigenetics and exome sequencing, can help to better understand the causes of rare diseases. Improvements in imaging and bioinformatics analysis will provide a greater understanding of the clinical and biological data behind rare diseases. And finally, improvements in diagnostic technologies, such as microfluidics and nanotechnology, for noninvasive or minimally invasive sampling and detection of atypical functioning, will aid in diagnosis.¹¹

Overall, the combination of scientific advances and the growing commitment of policy makers to adopt precision medicine will increase the number of diseases fitting the definition of "rare." It remains essential to continue to support rare disease patients and to develop and provide therapeutic treatments, especially as the prevalence of rare diseases in the community is high and rare diseases limit the life-span and quality of life of patients. In addition, there is a significant burden of rare disease within the pediatric population, and it remains imperative that research and development continues to help to fill in the gaps around care and support in this vulnerable population.

The Orphan Drug Act of 1983

The Orphan Drug Act has been universally considered a success

The Kefauver-Harris Drug Amendment to the Federal Food, Drug, and Cosmetic Act passed in 1962 introduced a requirement for drug manufacturers to provide proof of the effectiveness and safety of their drugs before approval, required drug advertising to disclose accurate information about side effects and stopped cheap generic drugs being marketed as expensive drugs under new trade names as new “breakthrough” medications.¹²

The resulting increased costs of drug development led manufacturers to increase their focus on chronic diseases affecting large populations. Consequently, therapies for rare diseases were not being developed on the same scale as therapies for more common diseases, leaving patients with few options. One estimate is that from 1967 to 1983, only 34 drugs were approved that would have met the orphan drug definition currently in use. Other estimates are that in the decade prior to 1983, only ten drugs developed by industry would have met the definition.^{11,13}

In the 1980s, rare disease patient advocacy groups formed a coalition that became the National Organization for Rare Disorders (NORD) and, along with Senator Orrin Hatch and with Representative Henry A. Waxman, were instrumental in passing the Orphan Drug Act in 1983, which provided incentives for drug manufacturers to develop therapies for rare diseases. According to the Act, a rare disease was considered one that “occurs so infrequently in the United States that there is no reasonable expectation that the cost of developing and making available in the United States a drug for such disease or condition will be recovered from the sales in the United States of such drug.”¹⁴ Congress has subsequently amended the Act and in 1984 put forth the current definition for a rare disease as one that affects fewer than 200,000 people in the United States. This threshold was an arbitrary ceiling based on the estimated prevalence of narcolepsy and multiple sclerosis.¹⁵ A further amendment in 1985 extended marketing exclusivity to patentable as well as unpatentable drugs, while an amendment in 1988 required sponsors to apply for orphan designation before submitting an application for marketing approval.² Key elements of the Orphan Drug Act are discussed in Exhibit 1.

Exhibit 1: Key Elements of the Orphan Drug Act		
Orphan Drug Act Element	Description	Impact
Rare disease definition	<ul style="list-style-type: none">< 200,000 patients in the United States or> 200,000 patients but with no reasonable expectation that the cost of development will be recovered*	The intent of the Orphan Drug act is to provide incentives for drug manufacturers to provide treatment for rare diseases

THE ORPHAN DRUG ACT OF 1983

Orphan Drug Act Element	Description	Impact
Market exclusivity	<ul style="list-style-type: none"> Seven year market exclusivity for sponsors of approved orphan drugs or products 	The market exclusivity for a new chemical entity in the United States is typically five years after FDA approval; for orphan drugs the FDA will not award market authorization for a generic drug for the rare disease for seven years post-approval; this incentive is superior to traditional IP patent protection and a substantial incentive
Tax incentives	<ul style="list-style-type: none"> The Orphan Drug Tax Credit (ODTC): sponsors who have orphan designation can collect tax credits for expenses occurred subsequent to issue of the designation for U.S. clinical trial costs on the orphan indication 	The ODTC lowers the cost of drug development. A study from NORD estimates that 33% fewer orphan drugs would be developed without the ODTC
Clinical research subsidies	<ul style="list-style-type: none"> Orphan Product Grant program provides funding for clinical testing of new therapies to treat and/or diagnose rare diseases** 	<p>The grant program lowers the cost of drug development. According to the FDA, the Office of Orphan Products Development (OOPD) has received over 2,500 applications, reviewed over 2,200, funded over 590 studies and helped 60 products gain marketing approval.</p> <p>Receiving a grant from the Orphan Product Grant program eases the likelihood of marketing authorization.</p>
Other regulatory incentives	<ul style="list-style-type: none"> Orphan drugs and products are exempt from the usual new drug application or “user” fees charged by FDA (i.e., PDUFA) 	These regulatory incentives lowers the cost of drug development and enables therapies to reach patients sooner

Note: *Only three therapies have received orphan drug designation under this second definition for rare disease.* **Grants are modest and can run approximately 500 thousand/year

Sources: FDA.gov; Cheung R, Kohler J, Illingworth P. Health Law Journal. 2004; NORD Impact of the Orphan Drug Tax Credit on treatments for rare diseases 2015; oig.hhs.gov. For full references see: 2, 16, 17, 18, 19

The Orphan Drug Act has been universally considered a success. From 1983–2016, there have been a total of 5,792 orphan drug designation requests, ranging from 16 in 1983 up to 472 in 2015.²⁰ As of 2016, there were 449 approved orphan therapies for 549 orphan indications, and 36% of novel drug approvals in 2016 were orphan drugs.^{21,22} The number of orphan drugs has been climbing steadily since 1983, with 2017 set to outpace 2016 for marketing approvals for orphan products (see Exhibit 2 and Exhibit 3).

THE ORPHAN DRUG ACT OF 1983

Orphan approved drugs and biologics are now available to treat rare diseases across numerous therapy areas and patient populations of which cancer has the highest number of therapies. From 1983 to 2015, there were 1,391 orphan drug designations to treat rare cancers of which 177 gained marketing approval amounting to 36% of all orphan drug approvals by 2015.²³ Inborn errors of metabolism, gastrointestinal (GI) disorders and neurological conditions make up most of the remaining indications.²⁴

Exhibit 2: Number of Approved Orphan Designated Therapies 1983–2016

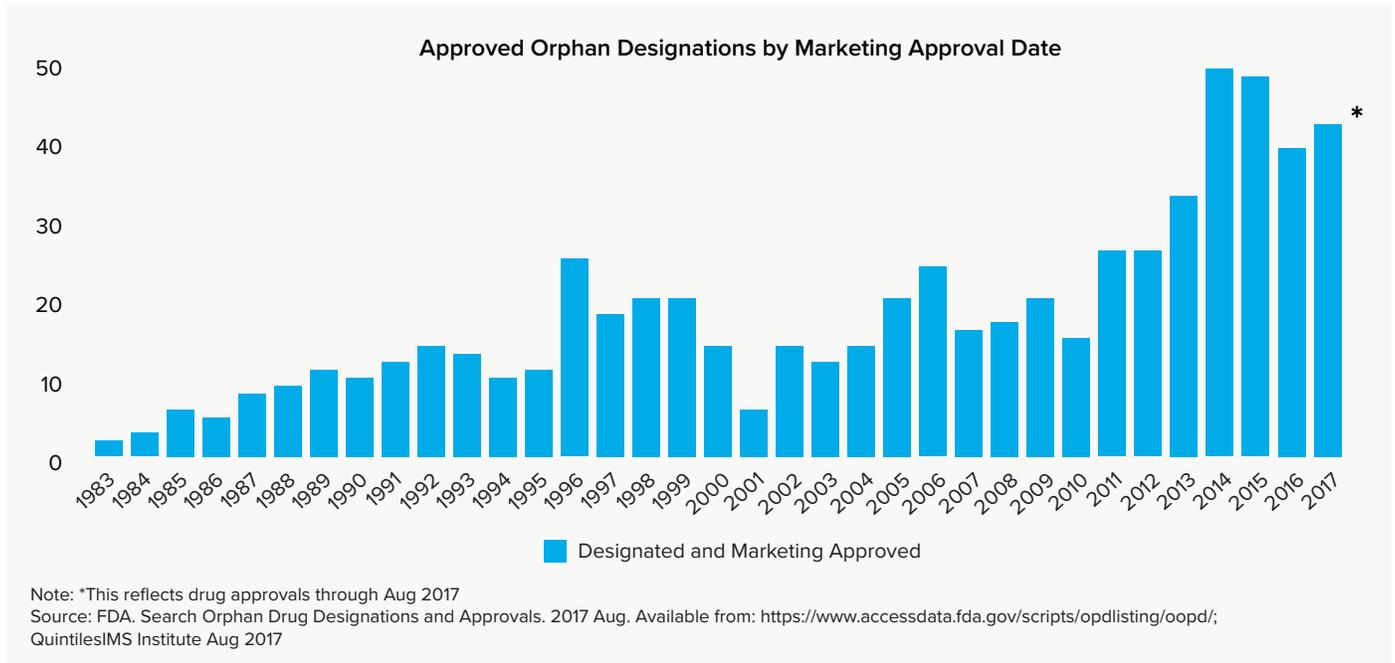
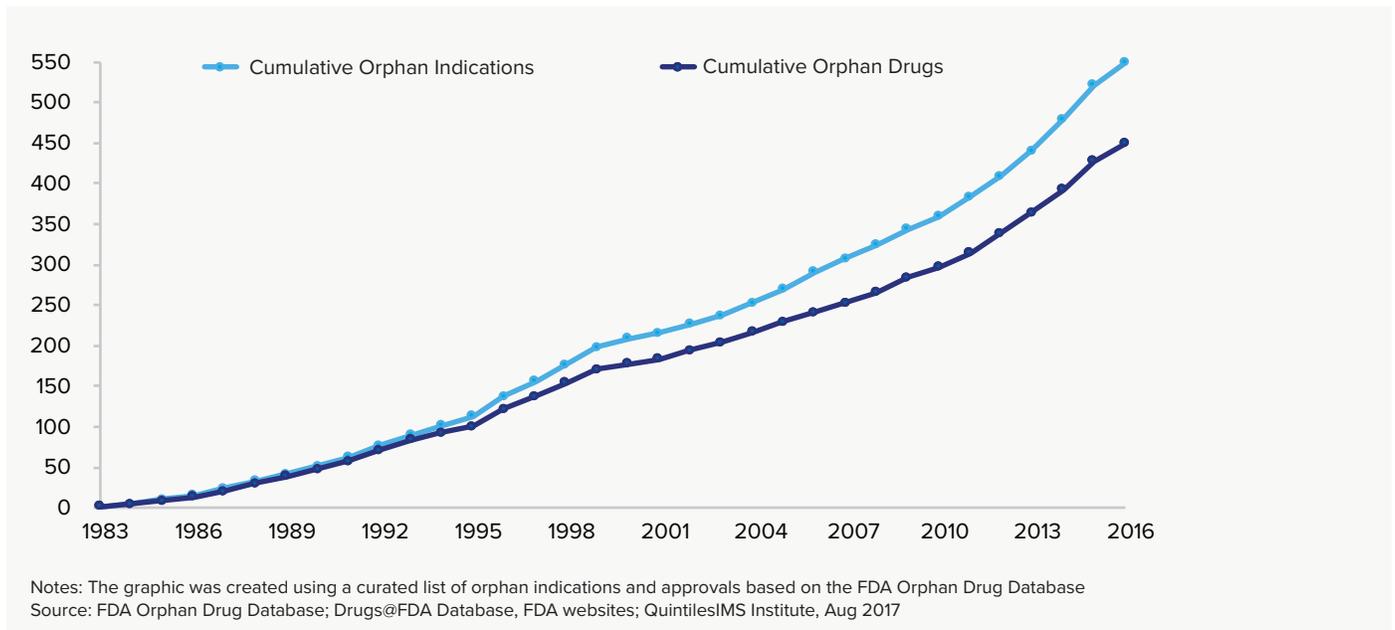


Exhibit 3: Number of Orphan Indications and Orphan Drugs 1983–2016



Orphan drugs approved under the Act include a very wide variety of medicines, many of which are used to treat pediatric and genetic rare diseases. See case studies below for illustrative examples.

CASE STUDIES IN RARE DISEASE MEDICINES	
Case Study	Examples
Breakthrough rare disease medicines for pediatric populations	Sapropterin dihydrochloride (Kuvan) was approved in December 2007 and is indicated to reduce blood phenylalanine levels in patients with hyperphenylalaninemia (HPA) due to tetrahydrobiopterin- (BH4-) responsive Phenylketonuria (PKU). ²⁵ PKU is an inborn error of metabolism that is now routinely screened for at birth; it is characterized by the absence or deficiency of an enzyme critical for metabolism of phenylalanine. Without an intensely monitored and restricted diet, infants with PKU can suffer from neurological problems, such as seizures and intellectual disabilities. In the United States, the reported incidence of PKU ranges from one in 13,500 to 19,000 newborns. ²⁶ Sapropterin dihydrochloride is the first and only therapy approved for patients with PKU to be used in conjunction with a restricted phenylalanine diet and helps to improve quality of life for PKU families.
	Sebelipase alfa (Kanuma) was approved as a breakthrough therapy for patients with a rare disease known as lysosomal acid lipase (LAL) deficiency. Patients with LAL deficiency have little or no LAL enzyme activity, resulting in a build-up of fats within the cells of various tissues that can lead to liver and cardiovascular disease and other complications. The most severe form of this disease presents during infancy and is rapidly progressive. A milder form presents in early childhood or later. This therapy provides an rhLAL protein that functions in place of the missing, partially active or inactive LAL protein in the patient.
Breakthrough therapy for adult population with no previous treatment	Sirolimus (Rapamune) was approved as a breakthrough therapy for lymphangioleiomyomatosis (LAM), a rare, progressive lung disease affecting primarily women of childbearing age. It was the first drug approved to treat the disease. LAM is characterized by an abnormal growth of smooth muscle cells that invade lung tissues, including the airways, and blood/lymph vessels that cause destruction of the lung. LAM is a very rare, life-threatening disease, with only two to five women per million worldwide affected. This was an added indication for sirolimus, which was originally approved as an immunosuppressive agent to help prevent organ rejection in patients receiving kidney transplants.

<p>Rare disease medicine affecting a larger rare disease patient population</p>	<p>Ivacaftor (Kalydeco) is one of three therapies approved for the symptomatic treatments of cystic fibrosis. Mutations in the cystic fibrosis transmembrane conductance regulator (CFTR) gene causes an imbalance of sodium and chloride across the cell membranes resulting in abnormally thick mucus that can impair airways, the GI tract, pancreas, sweat glands and genitourinary system. Cystic fibrosis remains one of the more common rare diseases, with approximately 30,000 patients in the United States. Prior to the approval of ivacaftor in 2012, only one other therapy was available to cystic fibrosis patients. Ivacaftor is effective in patients who harbor the G551D mutation and R117-H-CFTR mutation, among others; by 2017 ivacaftor had approval for a total of 33 CFTR mutations.²⁷ The availability of ivacaftor, along with ivacaftor/lumacaftor (Kalydeco) and dornase alfa (Pulmozyme®) has increased the life expectancy of cystic fibrosis patients.</p>
<p>Rare disease medicine affecting a smaller rare disease patient population</p>	<p>Alglucerase (Ceredase) was approved by the U.S. Food and Drug Administration (FDA) in 1991 for the treatment of Gaucher disease type 1. Gaucher affects only 6,000 patients in the United States.²⁸ Gaucher disease is caused by a deficiency of the enzyme glucocerebrosidase which results in the accumulation of harmful quantities of certain lipids throughout the body causing pain and damage to tissue particularly in the liver, spleen, lungs and bone marrow. Gaucher disease type 1 is treated with enzyme replacement therapy (ERT); there is currently no cure for the disease although ERT may help to slow the progression of the disease. Alglucerase was one of the first effective ERT available to patients; derived from human placenta tissue the manufacturer later introduced a synthetic form in 1994 (imiglucerase [Cerezyme]).</p>
<p>Essential rare disease medicine</p>	<p>Coagulation factor IX (Benefix/Rixubis/Alprolix) is approved to help control and prevent bleeding in people with hemophilia B, also called congenital factor IX deficiency or Christmas disease. Hemophilia B is the second most common type of hemophilia and is caused by mutations in the <i>factor IX</i> gene; it occurs in 1 in 25,000 male births.²⁹ Mutations in <i>factor IX</i> can lead to deficient levels of functional factor IX protein, which is a part a series of enzymes needed for blood clotting. Treatment of hemophilia B involves replacing factor IX using recombinant proteins or blood products. Coagulation factor IX reached the U.S. market in 1997 and represented a shift in standard of care. Prior to recombinant coagulation factor IX, patients received factor IX from human plasma, which had the risk of passing on infectious disease. Coagulation factor IX is on the WHO list of essential medicines and has one of the highest volumes of orphan drugs in the United States.</p>

<p>Rare disease medicines for oncology</p>	<p>Imatinib (Gleevec) is an oncology therapy with nine orphan drug approvals, including chronic myeloid leukemia (CML), Philadelphia-positive (Ph+) acute lymphoblastic leukemia (ALL) and gastrointestinal stromal tumor (GIST), each of which there fewer than 200,000 cases per year. Imatinib was one of the first members of a class of small molecule tyrosine kinase inhibitors; initially approved for CML in 2001, it has been a paradigm shift in the treatment of leukemia and a success of precision medicine. Prior to imatinib, allogenic stem cell transplantation was the only treatment for long-term control of CML. In one eight year follow up study, the estimated overall survival of all patients randomized to receive imatinib was 85%.³⁰</p>
<p>Rare disease medicine benefiting from advances in precision medicine</p>	<p>Rucaparib (Rubraca) was approved to treat women with a certain type of ovarian cancer. Specifically, it was approved for women with advanced ovarian cancer who have been treated with two or more chemotherapies and whose tumors have a specific gene mutation (deleterious <i>BRCA</i>) as identified by an FDA-approved companion diagnostic test. The diagnostic test was approved simultaneously. The National Cancer Institute estimated that 22,280 women would be diagnosed with ovarian cancer in 2016 and an estimated 14,240 would die of the disease. Approximately 15–20% of patients with ovarian cancer have a <i>BRCA</i> gene mutation. FDA approved rucaparib under its accelerated approval program.</p>

Characteristics of rare diseases and their treatments

Rare disease patients and caregivers often shoulder a considerable burden for their disease and find it necessary to educate physicians about their condition and serve as becoming their own advocates. Results of one survey indicate that approximately 60% of patients and caregivers provided physicians with information on their rare disease.³¹ Although there is increased awareness of rare diseases among healthcare stakeholders, patients often struggle to receive diagnosis and support; delays in diagnosis for rare diseases are common, with patients visiting on average 7.3 physicians before receiving a diagnosis and often experiencing symptoms for 4.8 years until they are diagnosed.³² In addition, the number of rare disease specialists is limited, and it can be difficult to find a specialist, which contributes to the challenges around receiving an accurate and timely diagnosis.³³

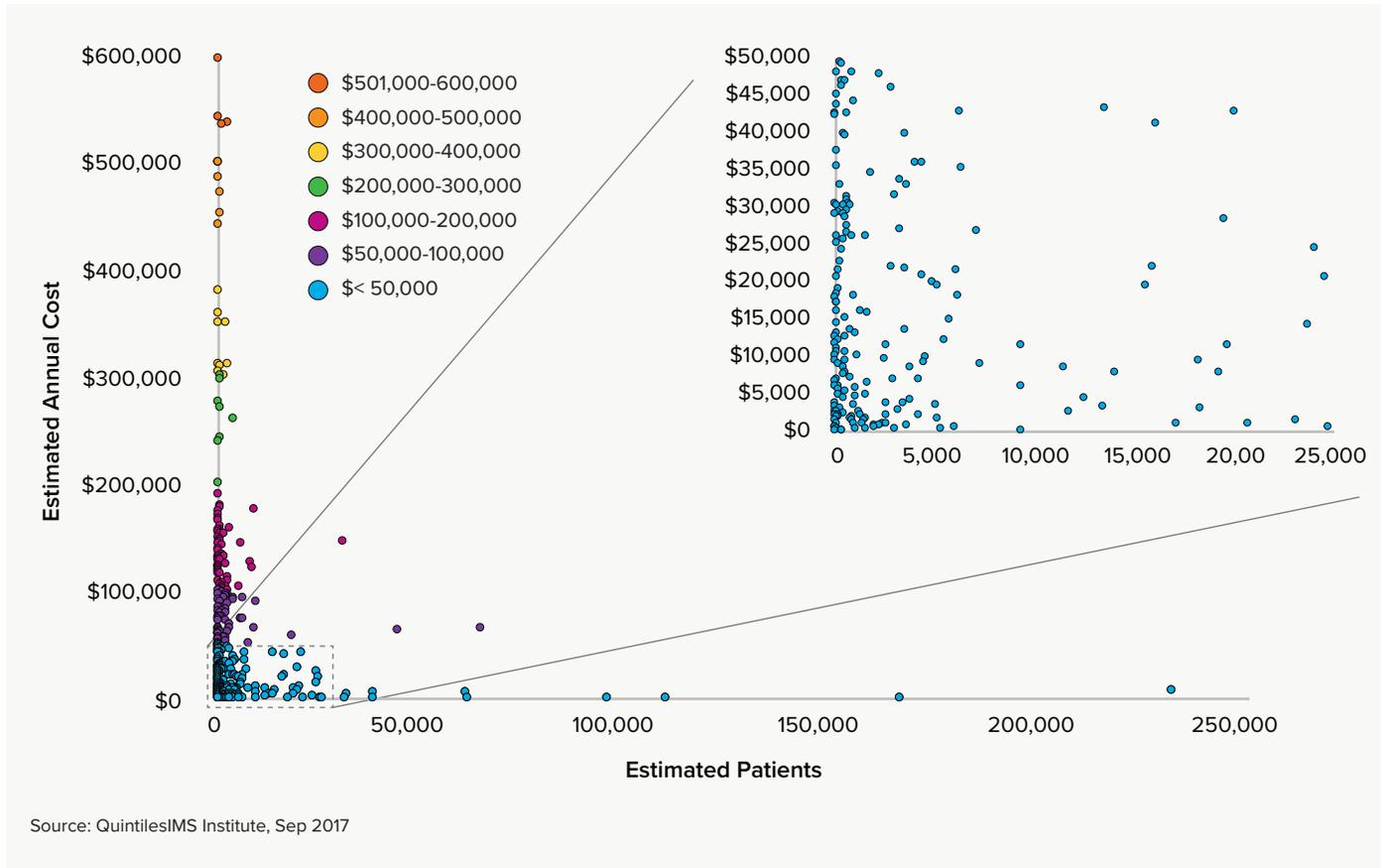
There are relatively few recognized Centers of Excellence (COE) for rare diseases, although individual diseases may have their own COE. The Children's National Health System's Children's National Rare Disease Institute (CNRDI) was designated by NORD in 2017 as its first official COE. The CNRDI will focus on advancing the care and treatment of children and adults with rare genetic diseases, including testing and development of new therapies and providing specific training in rare diseases to physicians and other health professionals.³⁴

A greater understanding of the biological pathways within rare diseases has allowed for development of novel therapies. Improvements in speed, access, and affordability of personalized genomic testing since the realization of the Human Genome Project have allowed for the development of methods to ensure drug response, precise dosing and minimal adverse drug reactions in individual patients.³⁵ Advances in genetics have transformed healthcare by changing the treatment paradigm from broadly treating symptoms within a population towards specifically targeting disease pathways within individuals, such as individuals with specific cancer mutations. These precision medicines provide differential treatments tailored to specific groups of patients. An analysis of 80 therapies, stratified by means of a recommendation or requirement on their label for testing of a specific gene, protein, or hormone prior to use, showed that 49% had orphan drug approval from the FDA.³⁶

Due to smaller populations that they cover, orphan drugs can be more expensive; the median annual cost for an orphan drug in 2016 was over \$32,000 per year. However, a number of orphan therapies have a more modest cost. Exhibit 4 shows an inverse relationship between cost per year and number of patients, with many less expensive therapies being dispensed to relatively few patients. The ten therapies used by the greatest number of patients averaged \$14,909 per year and include therapies such as Avonex and Copaxone for the treatment of MS, the oncology product Gazyva and the anti-infective Alinia.

CHARACTERISTICS OF RARE DISEASES AND THEIR TREATMENTS

Exhibit 4: Patients and Costs For Orphan Drugs, United States 2016

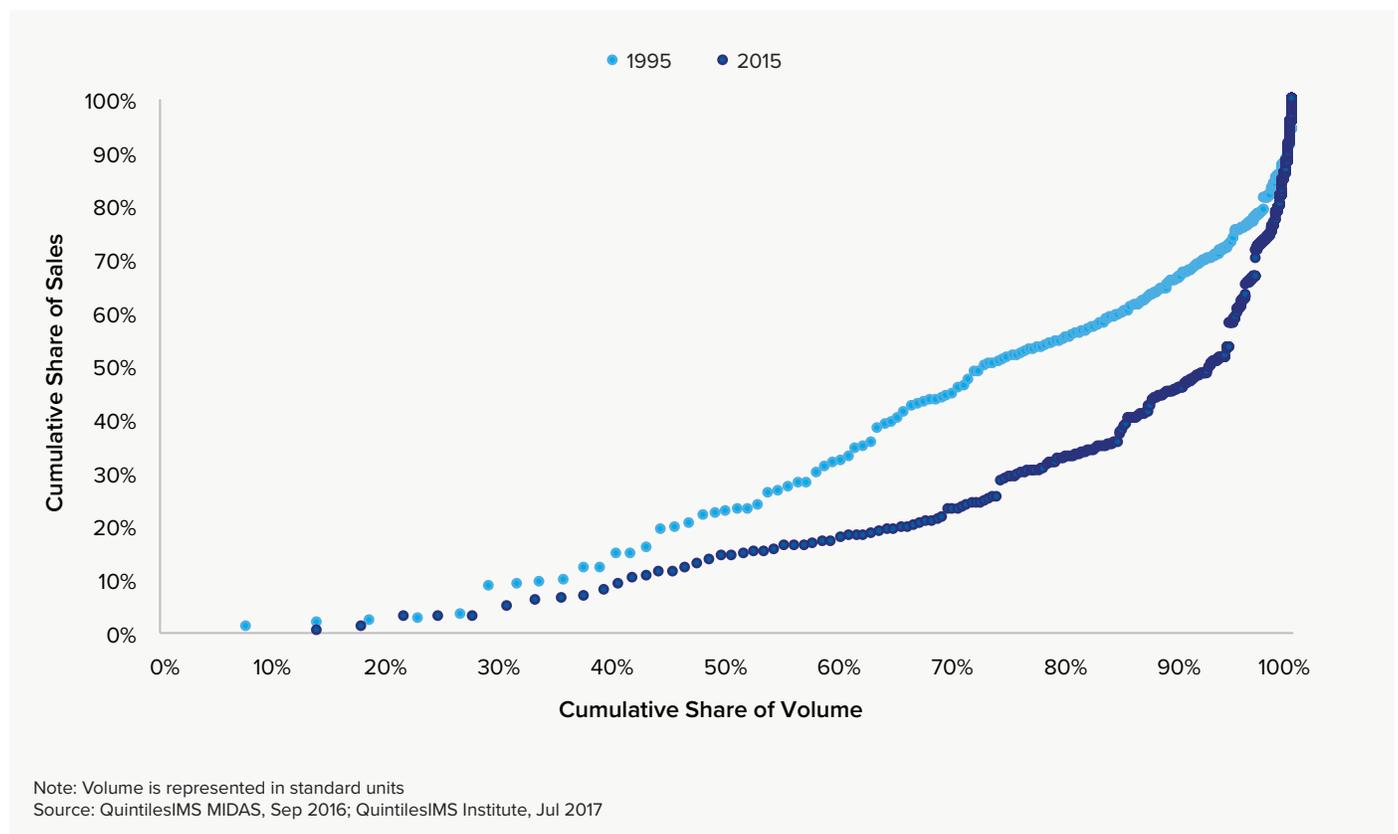


Source: QuintilesIMS Institute, Sep 2017

Evolution of drug volumes and prices and payer responses

Historically, medicines in the U.S. market have mostly been for the treatment of chronic diseases affecting large numbers of patients, such as hypertension, diabetes, high cholesterol and depression. While many of these drugs were available at relatively low prices, they were prescribed in some cases to millions of people. More recently, the introduction of specialty medicines with smaller target patient populations and higher costs has occurred at a time as many of the chronic disease drugs have lost patent protection and therefore become available at lower costs. From 1995 to 2015, the share of spending on the lowest volume drugs accounting for approximately 20% of total medicine volume has increased from 44% to 67%. During the same time period, the share of spending on the highest volume drugs, accounting for 80% of total medicine volume, declined from an average of 56% to 33% (see Exhibit 5). In other words, 80% of the volume of drugs in 2015 accounted for a third of spending; the remaining two thirds of spending is relegated to only 20% of the total volume of drugs.

Exhibit 5: Comparison of Spending and Volume 1995 and 2015



EVOLUTION OF DRUG VOLUMES AND PRICES AND PAYER RESPONSES

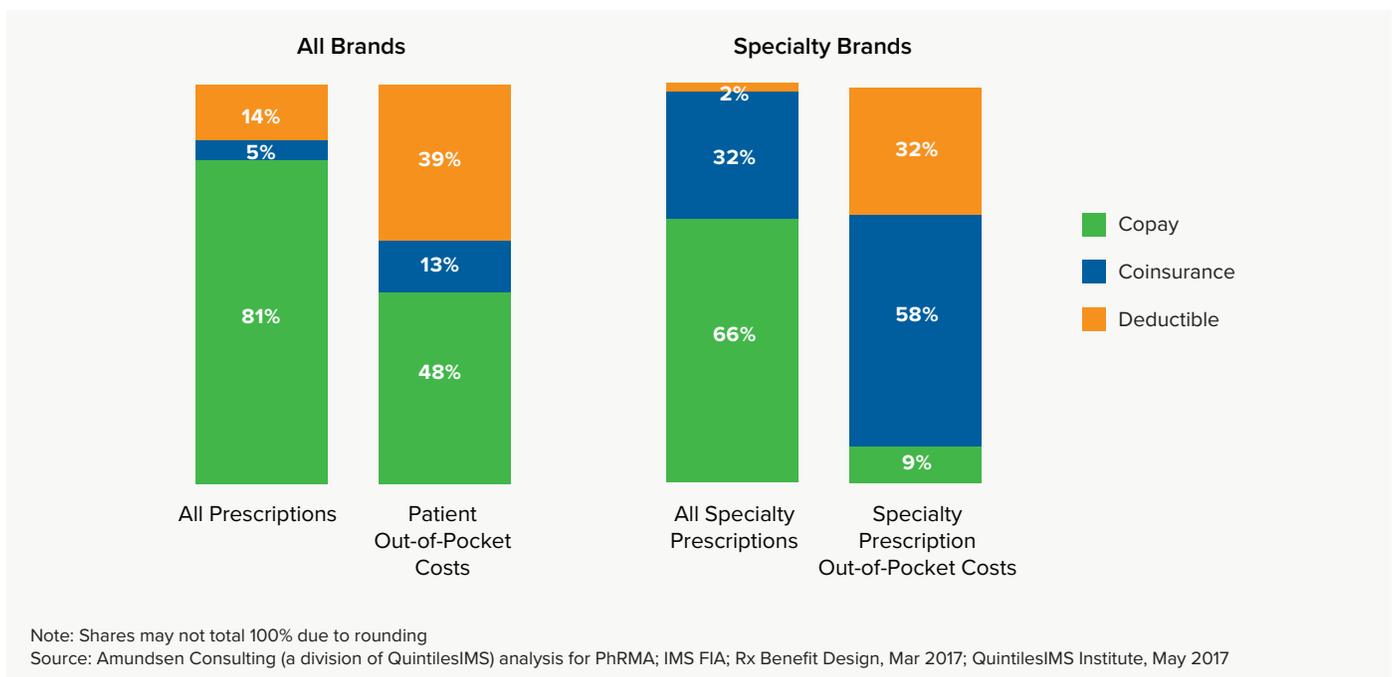
Scientific advances in the use of patient biomarkers to stratify individuals into subpopulations that differ in their susceptibility to a particular disease or their response to a specific treatment – also referred to as precision medicine – are also rebalancing the medicine portfolio toward drugs with lower volumes and higher patient outcomes value.³⁶

The evolution of drug spending has also been accompanied by mechanisms introduced by payers, intermediaries and other stakeholders in their efforts to better manage overall expenditure on medicines and encourage use of the most cost-effective treatments. These approaches and other dynamics affecting drug spending include the following:

- Introduction of specialty tiers in health plans
- Imposition of co-insurance payments by patients typically as a percentage of a drug’s pharmacy cost
- Greater use of health plans carrying pharmacy deductibles which require patients to pay the full price of their initial prescription costs until they reach their deductible threshold
- Use of specialty pharmacies and limited distribution networks by manufacturers and payers to exert greater control over pricing and use of drugs
- Strengthening negotiating power of pharmacy benefit managers, third-party administrators of prescription drug programs, due to consolidation at the same time manufacturers remain relatively fragmented
- Increased use of pre-authorization requirements and step therapy guidelines.

One of the consequences of these changes has been the greater financial burden on patients especially those prescribed specialty branded medicines (see Exhibit 6).

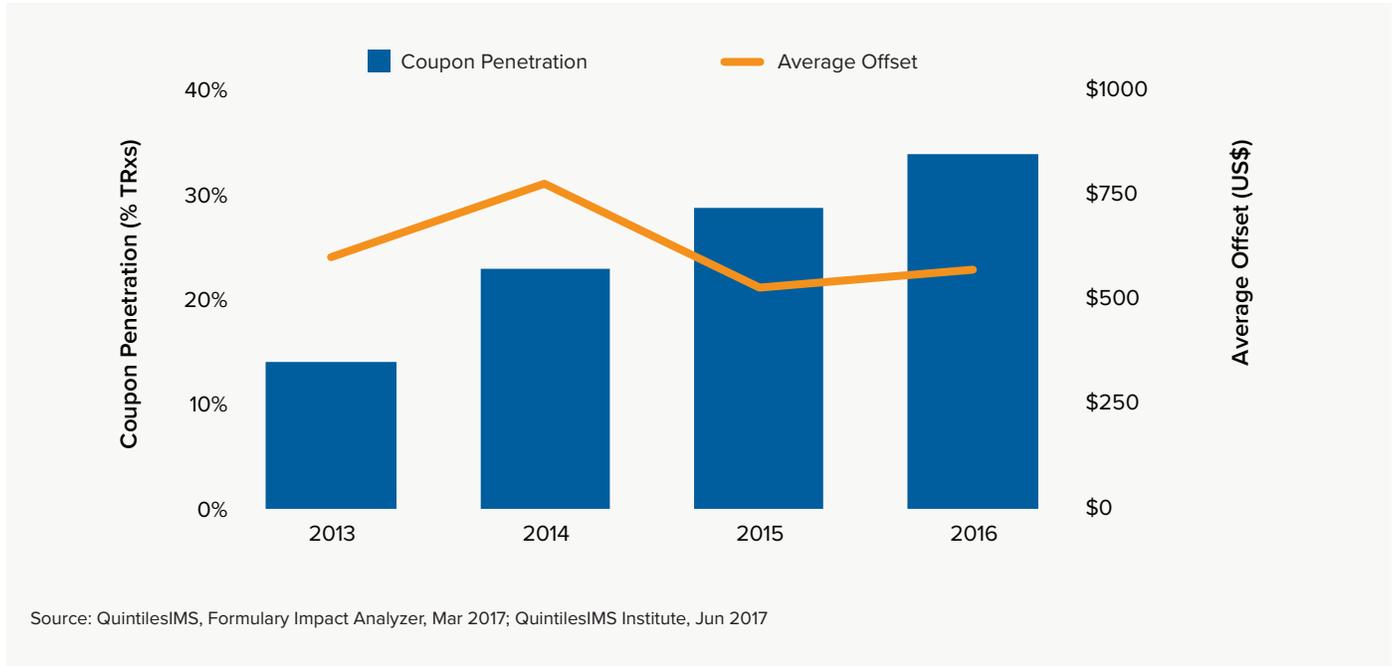
Exhibit 6: Distribution of Prescriptions by Out-of-Pocket Costs for Branded Medicines in Commercial Plans



EVOLUTION OF DRUG VOLUMES AND PRICES AND PAYER RESPONSES

Overall, these efforts have led to greater access barriers for patients.^{37,38} In response, patient assistance programs have expanded as a mechanism to offset patient out-of-pocket costs – even for those with insurance – and enable prescribed medicines to be used as recommended by healthcare professionals. For example, some type of coupon or patient cost offset was used in over 30% of retail prescriptions for cancer drugs filled by patients with commercial insurance in 2016 (see Exhibit 7).

Exhibit 7: Coupon Penetration and Average Offset of Patient Savings Programs in Oral Oncology Drugs



Orphan drugs in context

Definitions

In order to fully understand the role that orphan drugs play in the U.S. health system, both from a volume and cost perspective, it is helpful to use a set of common definitions. For the purposes of this report, the following terms are used:

- **All medicines** include those prescription drugs approved by the FDA and distributed through retail and non-retail channels, including brands and generics, specialty and traditional drugs, and small molecules as well as biologics
- **Specialty medicines** can and are defined differently by various stakeholders. In this research, specialty medicines as defined by QuintilesIMS are those which treat chronic, complex or rare diseases and which have a minimum of four out of seven of the following additional characteristics:
 - Costly: list price in excess of \$6,000 per year
 - Initiated/maintained by a specialist physician
 - Requiring administration by another individual or healthcare professional (i.e., not self-administered)
 - Requiring special handling in the supply chain (e.g., refrigerated, frozen, chemo precautions, biohazard)
 - Requiring patient payment assistance
 - Distributed through non-traditional channels (e.g., “specialty pharmacy”)
 - Medication has significant side effects that require additional monitoring/counseling (including, but not limited to REMS programs) and/or disease requires additional monitoring or therapy (e.g., monitoring of blood/cell counts to assess effectiveness/side effects of therapy).

This definition of specialty drugs may differ from those used by other stakeholders. For example, CMS defines specialty drugs only in the context of Part D (i.e., not Part B) and uses a dollar threshold; pharmacy benefit managers typically define specialty also based on cost; pharmaceutical wholesalers consider specialty drugs to be those that they require special handling due to low volume, cold-chain or other storage requirements, or are managed through separate organizational or delivery channels.

- **Traditional medicines** are defined by QuintilesIMS as all drugs that do not meet the criteria to be classified as a specialty medicine.
- **Orphan drugs** are generally defined as those medicines with one or more indications approved under the Orphan Drug Act. In some cases, these medicines may also have other non-orphan indications approved by the FDA but not meeting the criteria for an orphan drug designation. Of the 449 distinct drugs approved with any orphan indications since the passage of the Orphan Drug Act, 351 drugs have only orphan designations and the remaining 98 drugs have both orphan and non-orphan indications (see Exhibit 8).

Exhibit 8: Orphan Drug Approval by Approval Indication

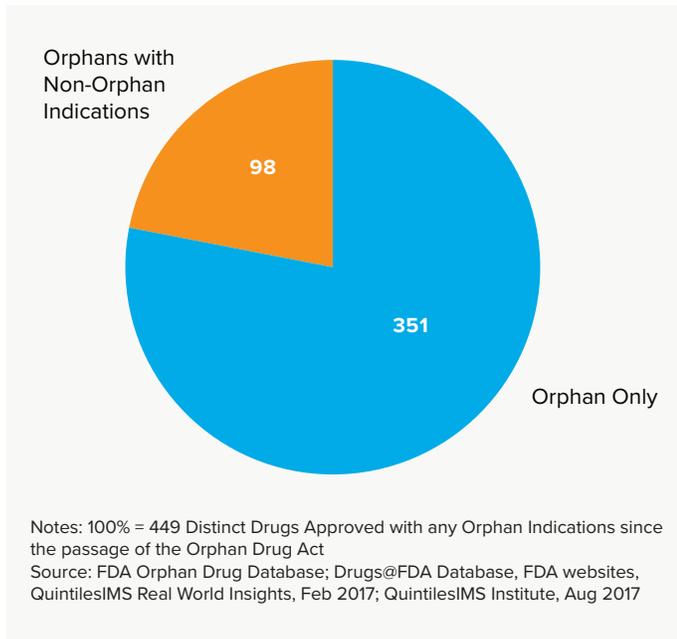
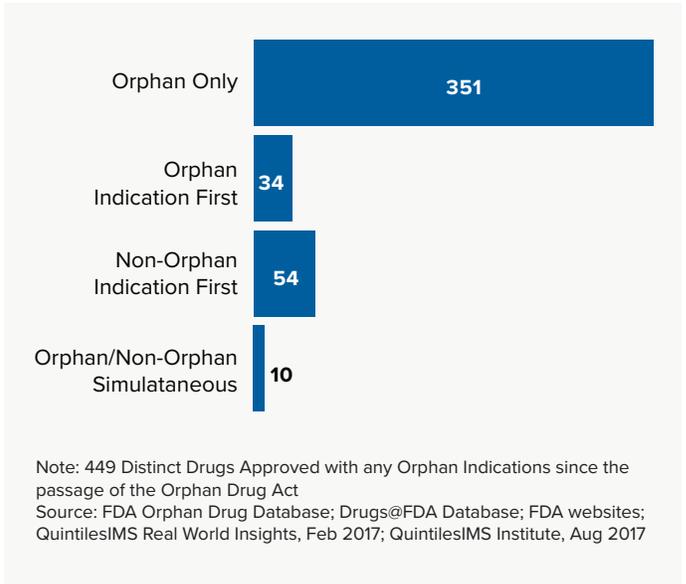


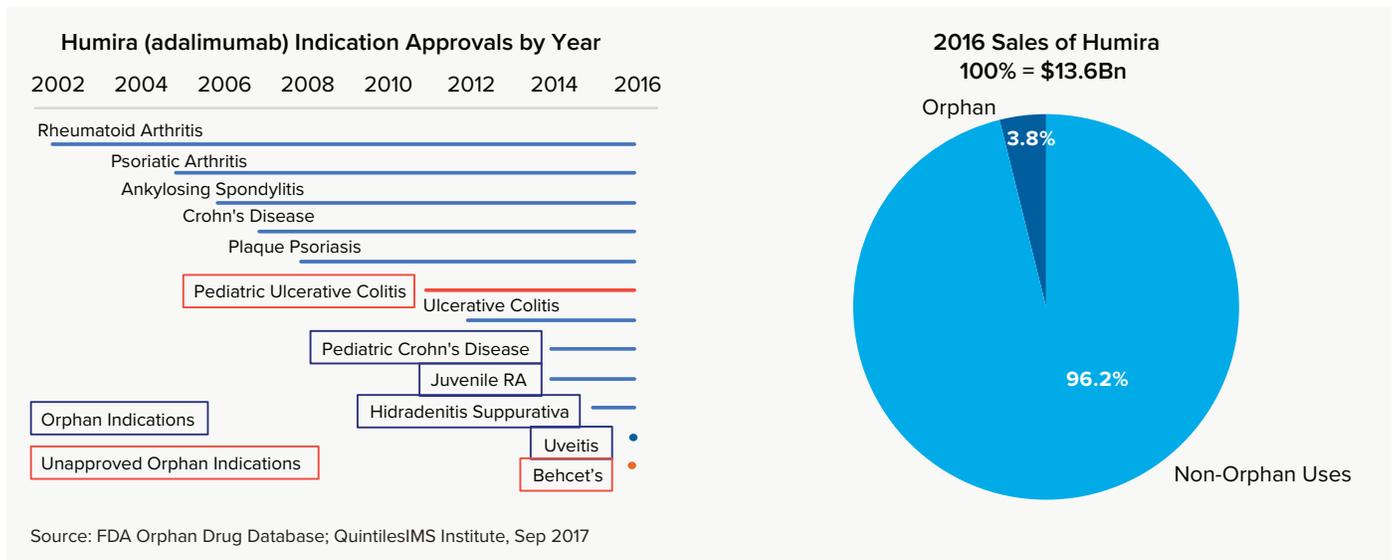
Exhibit 9: Number of Orphan Drugs Stratified by Approval Sequence



Of those drugs with both orphan and non-orphan indications, 54 received a non-orphan indication first, while 34 received an orphan indication first, and 10 drugs received both orphan and non-orphan indications simultaneously (see Exhibit 9).

An example of a drug with both orphan and non-orphan indications is Humira (adalimumab). Since its initial approval in 2002 for rheumatoid arthritis, Humira has received approval for several new indications, including four under the Orphan Drug Act. These orphan indications account for an estimated 3.8% of the total use of Humira in 2016 (see Exhibit 10).

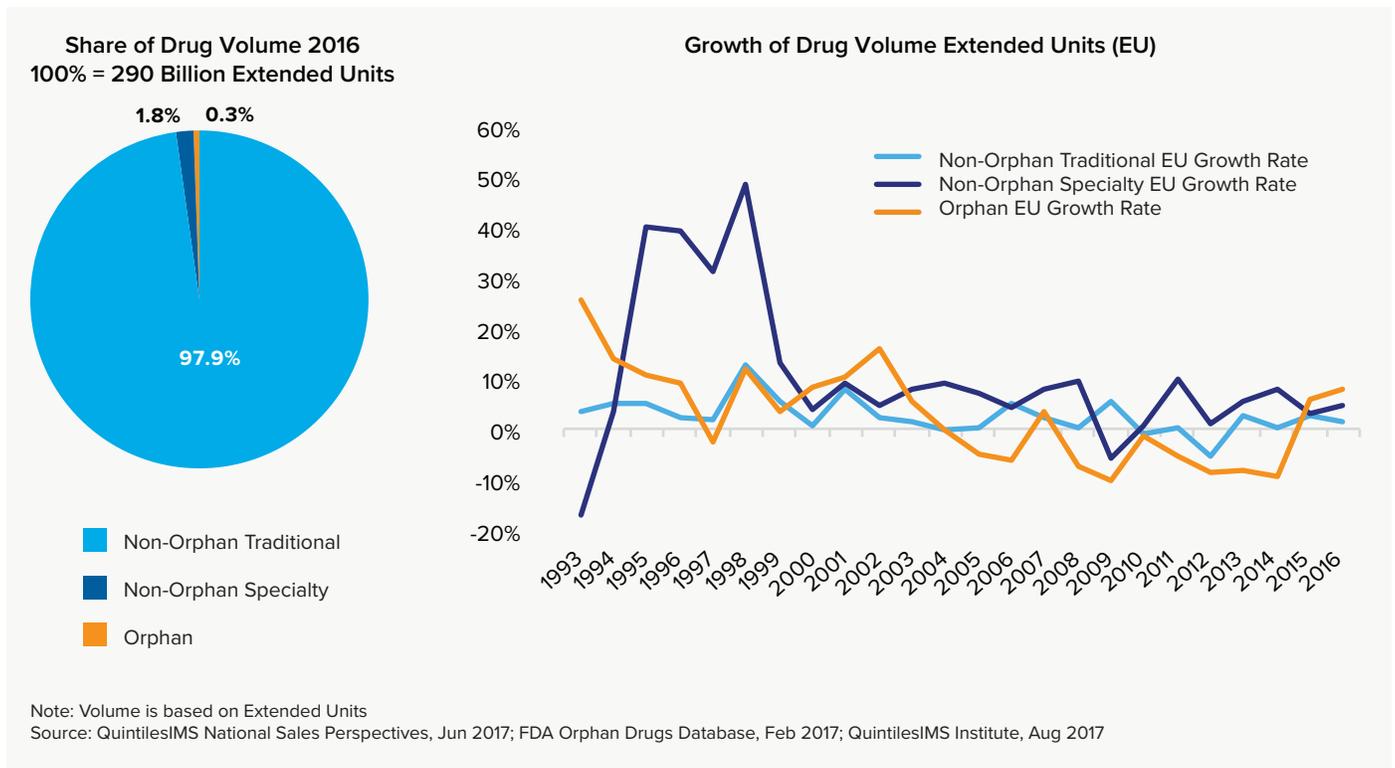
Exhibit 10: Orphan and Non-Orphan Approvals and Sales of Humira



Volumes

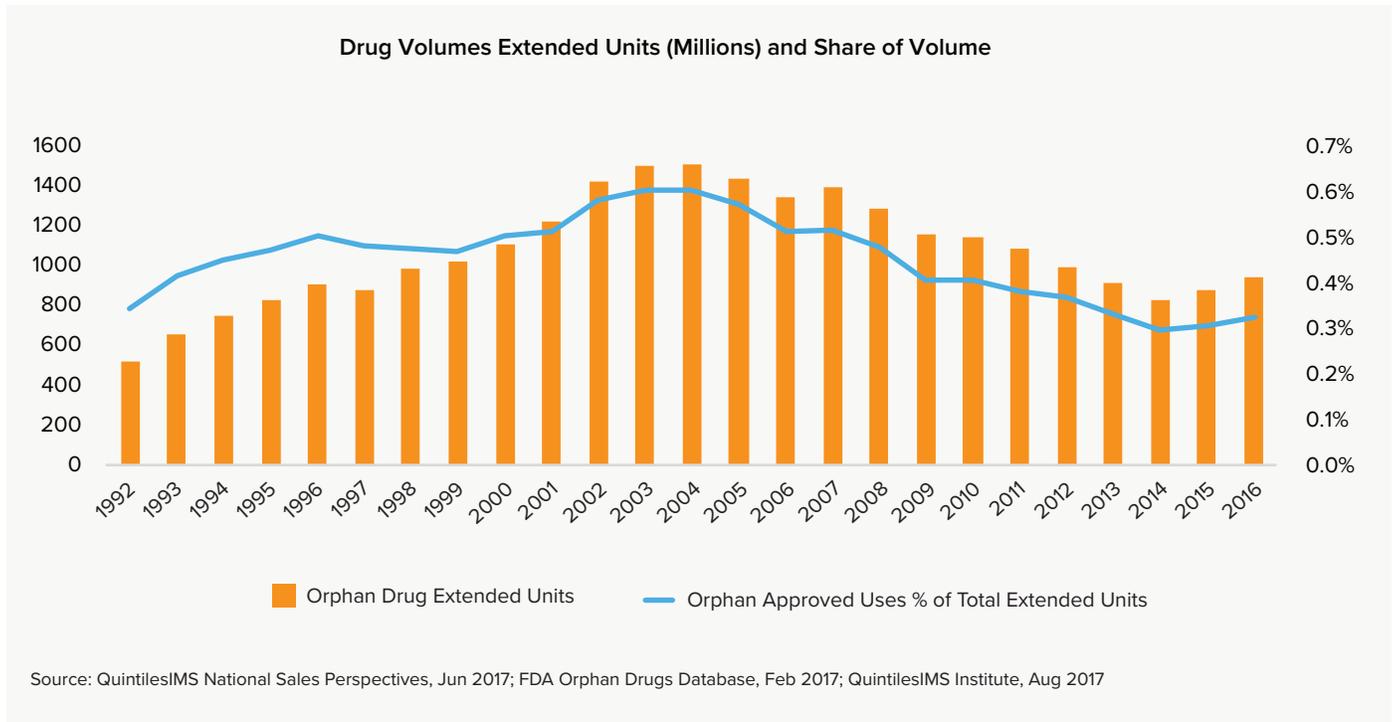
Medicine volume can be measured using extended units across all types of drugs. Those drugs with both orphan and non-orphan indications can have their volume split by indication based on claims data, diagnostics associated with the use of the medicine and epidemiological information (see Appendix for details). In 2016, non-orphan traditional drugs accounted for 97.9% of the total volume of drugs, with non-orphan specialty drugs accounting for an additional 1.8% of the volume. The remaining 0.3% of volume was the result of drugs used according to their orphan indications. Since 1993, the growth rates associated with each type of medicine has varied widely, with the volume growth of orphan drugs falling below non-orphan specialty and traditional medicines each year since 2004 (see Exhibit 11).

Exhibit 11: Volume Trends of Traditional, Specialty and Orphan Drugs



Despite an increase in the number of orphan drugs available on the market, the total volume of orphan drugs (factored for their orphan indications, only) has declined since 2003, though the last two years has shown growth. This volume trend reflects lower use of some of the older orphan drugs, such as metronidazole, balsalazide and midodrine. The orphan drug share of total volume has similarly declined from a peak of 0.6% in 2003 to just over 0.3% in 2016 (see Exhibit 12).

Exhibit 12: Drug Volume, United States 1992–2016

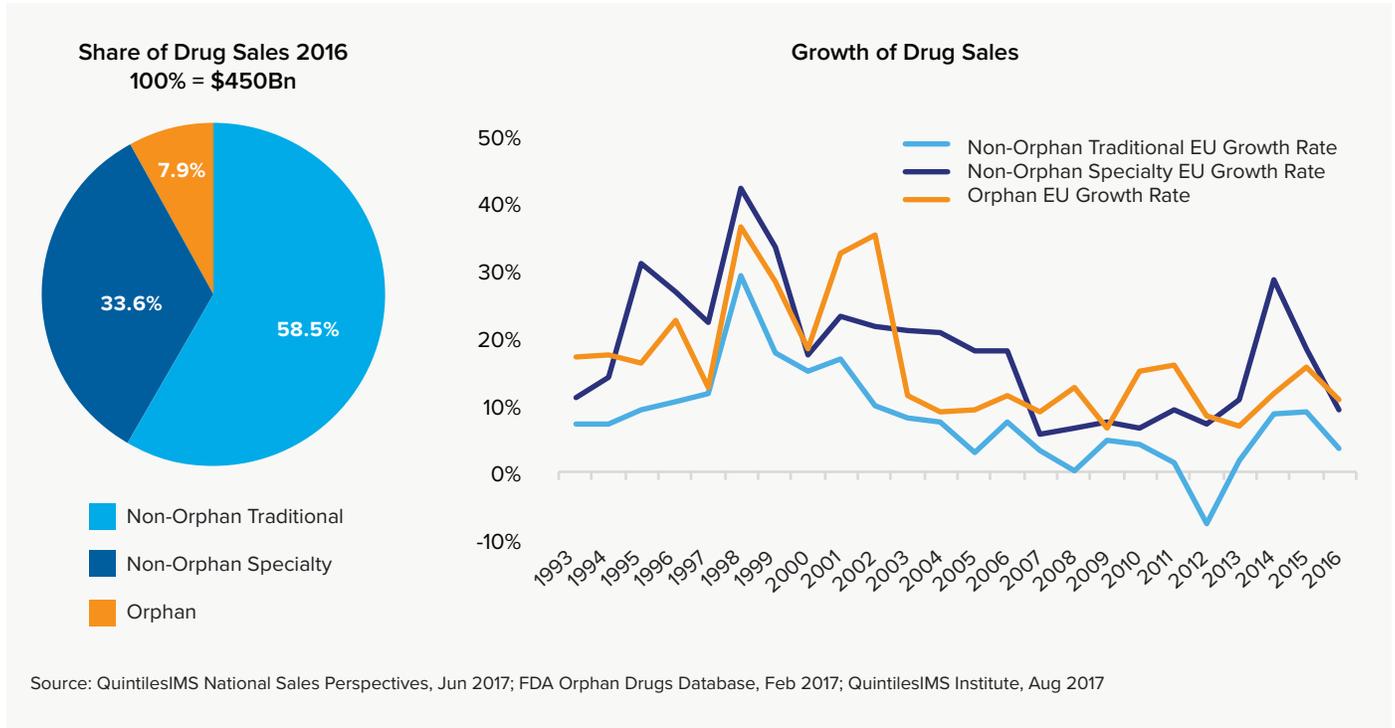


Sales

Only 7.9% of drug spending in the United States is attributed to orphan indications.

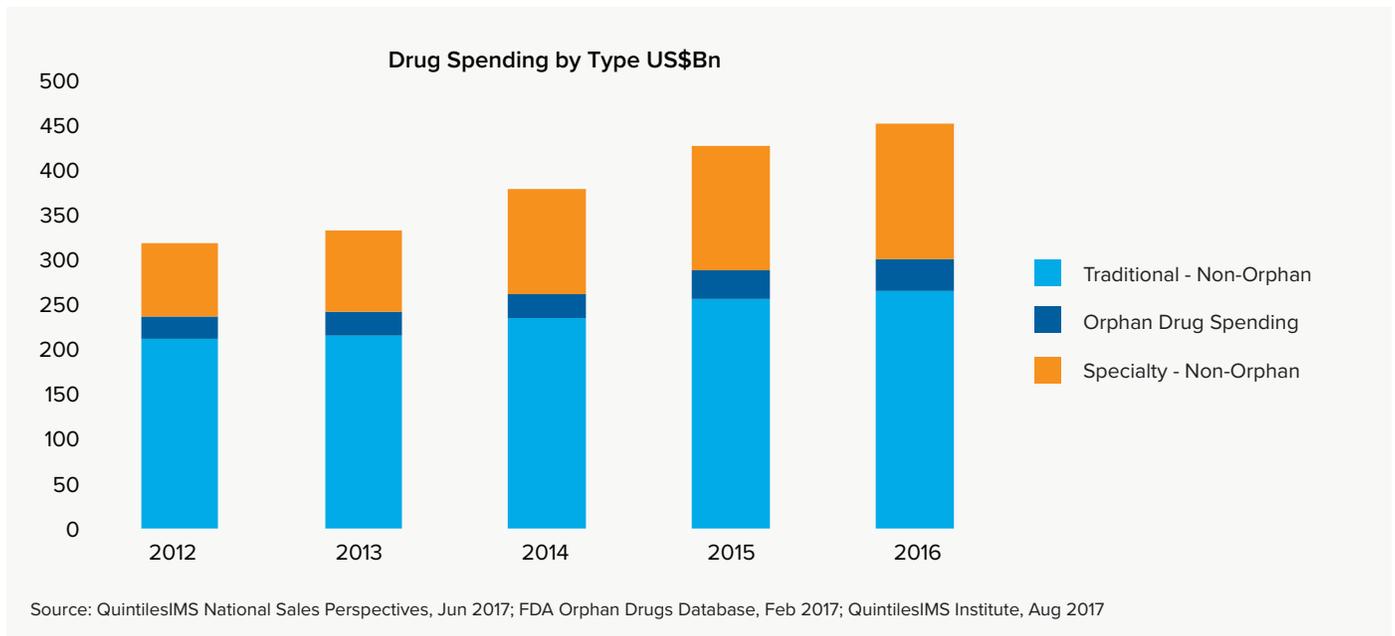
The total sales of drugs can also be looked at by segment, disaggregating the market into non-orphan traditional drugs, non-orphan specialty drugs and orphan drugs. In 2016, of the total drug sales in the United States of \$450 billion, almost 60% was from non-orphan traditional drugs, while one-third was spent on non-orphan specialty drugs. The remaining 7.9% of spending – about \$36 billion – is attributed to the orphan indications of approved drugs. Growth rates over time have generally slowed since the late 1990s, with orphan drugs growing faster than other segments during the period 2008–2012. In 2014 and 2015, non-orphan specialty drug sales rose rapidly due primarily to the introduction of new hepatitis C treatments bringing dramatic advances to those patients suffering from these diseases (see Exhibit 13).

Exhibit 13: Sales Trends of Traditional, Specialty and Orphan Drugs



During the past five years, while the number of orphan drugs approved has increased from 315 to 449, the share of spending has increased more moderately and these drugs represent a small part of the overall medicine budget (see Exhibit 14).

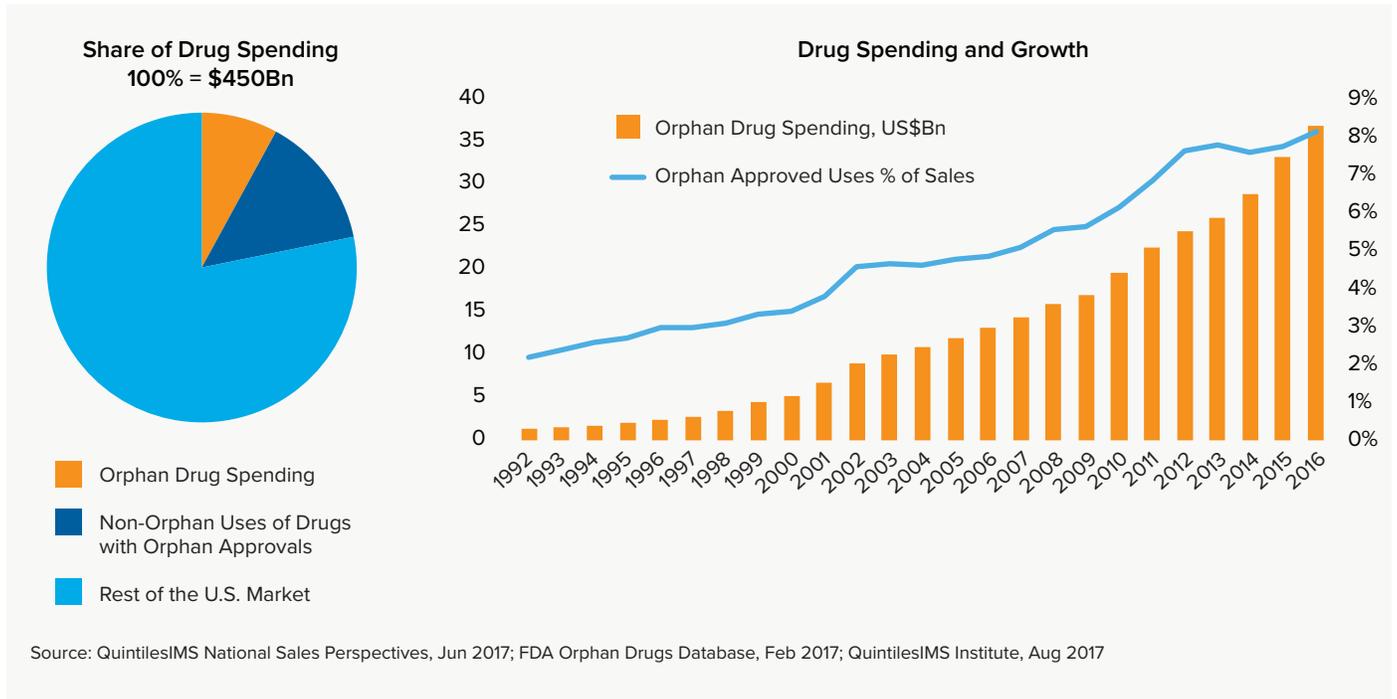
Exhibit 14: Total Drug Spending by Orphan, Traditional and Specialty, United States



ORPHAN DRUGS IN CONTEXT

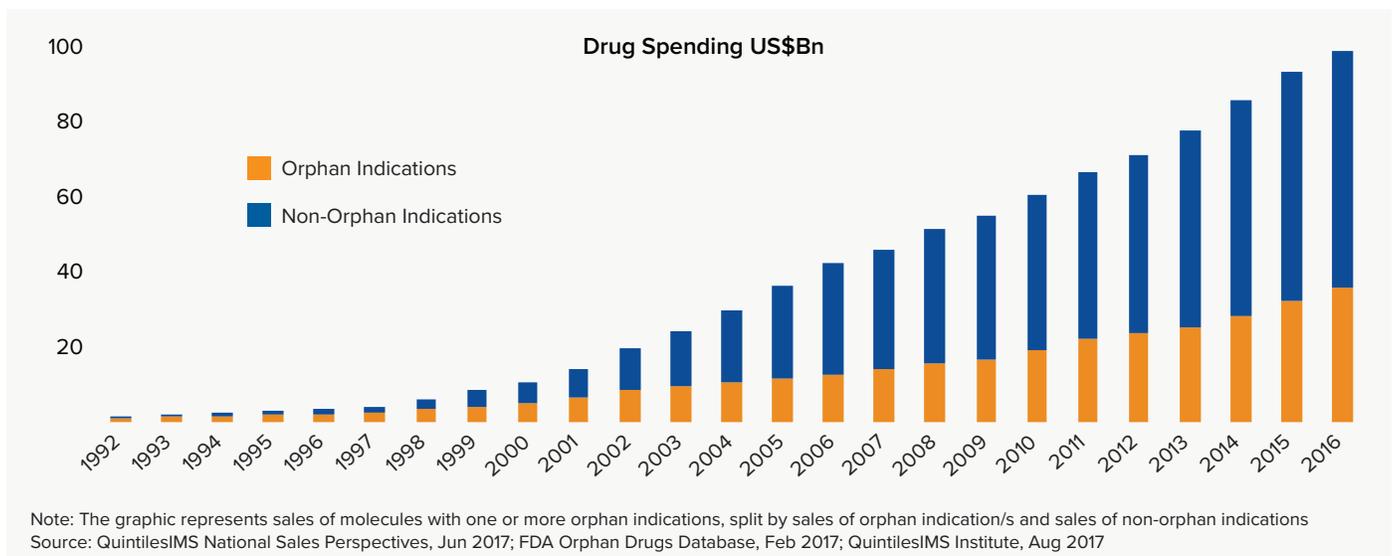
Since 1993, the share of total drug spending attributed to orphan drugs has increased from about 3% to 8% in 2016 (see Exhibit 15).

Exhibit 15: Spending on Orphan Drugs in the United States 1992–2016



For those molecules with both orphan and non-orphan indications, the non-orphan indications typically represent the majority of their use and sales. Over time, the sales of these molecules has risen to almost \$100 billion in 2016. However, the amount of those sales attributed to the orphan indications is approximately a third of the total (see Exhibit 16).

Exhibit 16: Spending on Orphan Drugs by Orphan and Non-Orphan Indications, United States 1992–2016

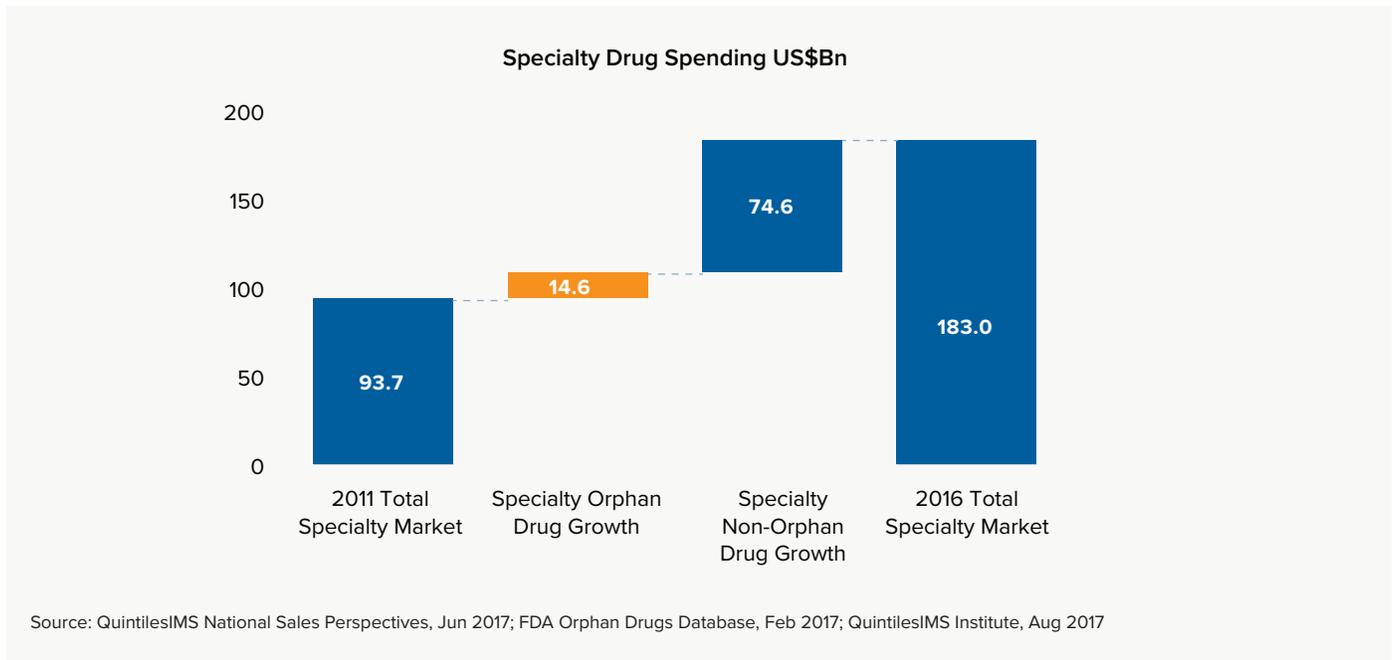


Orphan Drug Contribution to Spending Growth

Of the \$89 billion in spending increase over the last five years, specialty orphan drugs contributed less than 20% – about \$15 billion of growth – with non-orphan specialty drugs contributing the remaining \$74 billion of growth.

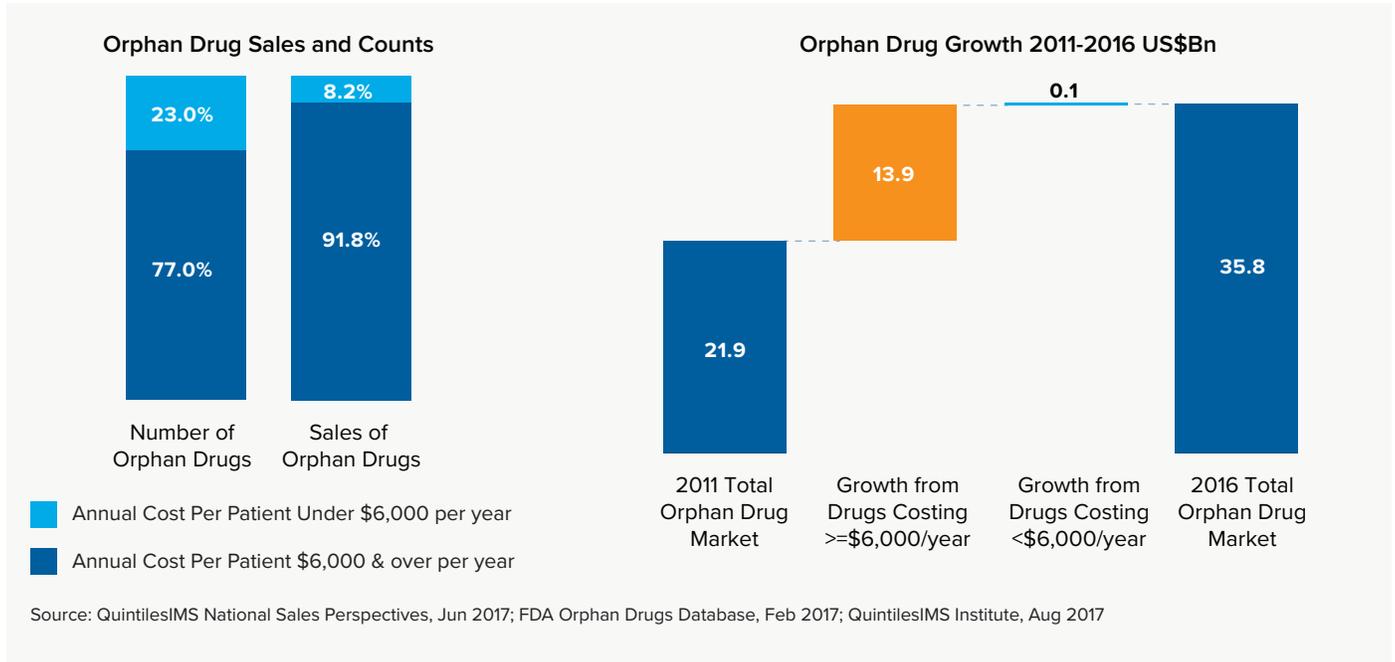
Over the past five years, significant attention has been placed on the growth in spending on specialty drugs. These include drugs for the treatment of cancer, autoimmune disorders and HIV/AIDS, multiple sclerosis and viral hepatitis. Growth in spending of these drugs is primarily due to the approval and availability of new treatments, and an increased number of patients receiving these medicines. Over the five-year period, total spending has almost doubled, from \$94 billion to \$183 billion. Of this \$89 billion increase over the five-year period, specialty orphan drugs contributed less than 20% – about \$15 billion of growth – with non-orphan specialty drugs contributing the remaining \$75 billion of growth (see Exhibit 17).

Exhibit 17: Orphan Drug Contribution to Specialty Market Growth, United States 2011–2016



Within the orphan drug segment, most of the drugs, spending, and growth in spending comes from medicines with annual costs in excess of \$6,000. In 2016, these drugs represented almost 80% of the drugs and over 90% of total sales, as well as the vast majority of growth in orphan drug spending (see Exhibit 18).

Exhibit 18: Orphan Drug Distribution and Contribution to Growth by Annual Cost

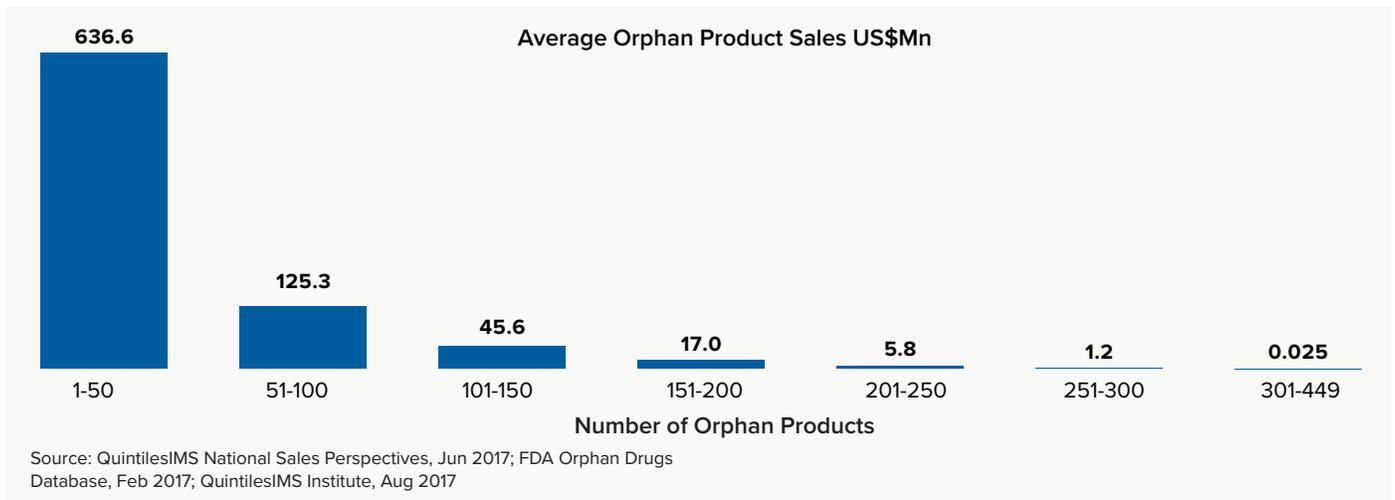


Average Sales and Price of Orphan Drugs

Among the cohort of drugs with one or more orphan designations, there is wide variation in pricing and annual sales of individual products. In some cases, these drugs are sold to very small numbers of patients (less than 1,000) while in others, the number of patients receiving the drug can reach several hundred thousand (across multiple indications).

In 2016, the 50 highest-selling orphan products had average sales for the year of \$637 million. The next 50 highest-selling products averaged \$125 million, and the next 50 averaged \$45 million. The remaining 300 orphan drugs averaged much lower average sales (see Exhibit 19), and include products such as Neupogen and Evista.

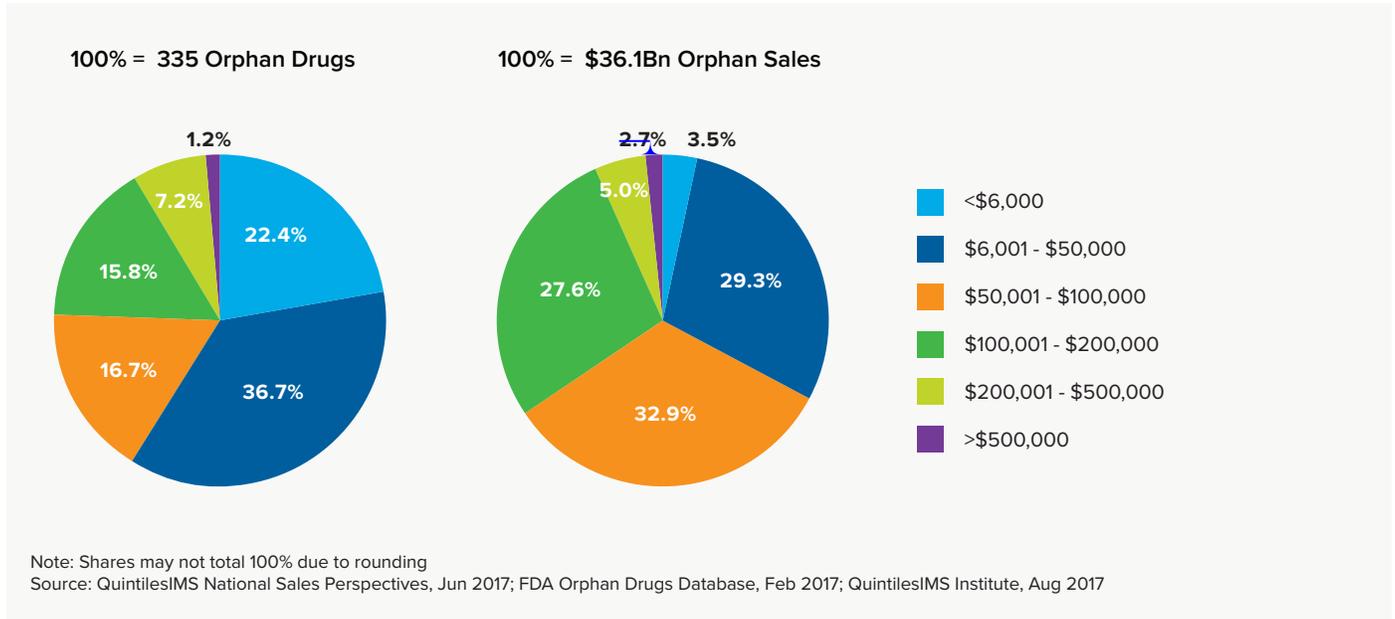
Exhibit 19: Average Orphan Product Sales, United States 2016



ORPHAN DRUGS IN CONTEXT

The annual cost per patient for orphan drugs also varies widely. About 20% of the drugs are priced at less than \$6,000 per year, and they contribute 3.5% of total spending on orphan drugs. About 1% of orphan drugs are priced in excess of \$500,000 per year, but they only account for 1.6% of orphan drug spending due to relatively few patients being treated with these medicines (see Exhibit 20); examples include Actimmune, Folutyn and Soliris.

Exhibit 20: Orphan Drug Distribution by Annual Cost, United States 2016



About 6% of orphan drugs are priced in excess of \$500,000 per year, but they only account for 2.7% of orphan drug spending due to relatively few patients being treated with these medicines.

Notes on sources

This report is based on the QuintilesIMS services detailed below.

National Prescription Audit (NPA)[™] is a suite of services that provides the industry standard source of national prescription activity for all products and markets.

National Sales Perspectives (NSP)[™] measures spending within the U.S. pharmaceutical market by pharmacies, clinics, hospitals and other healthcare providers. NSP reports 100% coverage of the retail and non-retail channels for national pharmaceutical sales at actual transaction prices. The prices do not reflect off-invoice price concessions that reduce the net amount received by manufacturers.

“SMART – Launch Edition” is a service that allows users to study the market uptake and launch criteria, both of the marketplace and product, for branded and generic launches from 1992 to present-day.

QuintilesIMS MIDAS[™] is a unique data platform for assessing worldwide healthcare markets. It integrates QuintilesIMS national audits into a globally consistent view of the pharmaceutical market, tracking virtually every product in hundreds of therapeutic classes and providing estimated product volumes, trends and market share through retail and non-retail channels. MIDAS data is updated monthly and retains 12 years of history.

QuintilesIMS Formulary Impact Analyzer (FIA) provides insight into what impact utilization-control measures enforced by managed care organizations have had on prescription volumes including the dynamics that affect patient behavior in filling and/or refilling prescriptions. Formulary measures include tiered copay benefit designs, prior authorization restrictions, and often result in non-preferred prescriptions being rejected or switched at the pharmacy. FIA offers visibility to claims rejected for other reasons such as contraindications as well as those attempted to be refilled too soon. FIA sources include national and regional chains, independent pharmacies and a claims coordination switch company providing a comprehensive view of retailers and across geographies.

Appendix

Study Data and Methods

The FDA's Orphan Drug Product designation database was used to identify a comprehensive list of drugs that were approved by the FDA and given orphan status between passage of the Orphan Drug Act in 1983 and the end of 2016.³⁹ We used the QuintilesIMS SMART US Launch edition database to assess U.S. invoice-price revenues on all drugs and on orphan drugs in the period 1992–2016 (at the time of the analysis, 2016 was the last year for which full data were available). The 449 unique brand-name orphan drugs that had been approved in the United States from 1983 to 2016 were identified from the FDA's Orphan Drug Product designation database. Where a medicine had multiple orphan designations, this was noted for analysis of orphan and non-orphan designation approval time sequence analysis, and for the application of factors to estimate orphan uses of the drugs. Each orphan drug with multiple dosage formulations is counted only once, aggregating the formulations. According to the FDA, orphan drug designation is conferred on the active moiety, or principal molecular structure, instead of on the formulation.⁴⁰ The Drugs@FDA database, which includes information on approved drug products and approval history, was used to identify all approved indications for orphan drugs and approval dates.

The QuintilesIMS SMART US Launch Edition database was used to identify sales of the orphan drugs, and to include data from 1992-2016 as the most complete archive available. The database accurately summarizes estimated product volumes and sales by product and therapy class through retail and non-retail channels. All volume data and associated drug sales for products included in this study were validated by QuintilesIMS's data integrity team.

Partial Orphan Drugs

Since the FDA may approve a drug for multiple indications, we undertook a further evaluation to identify “partial orphan drugs,” products with both orphan and non-orphan indications. Of the 449 orphan drugs designated by the FDA, 98 had both orphan and non-orphan approved indications. Because the QuintilesIMS sales data are not segmented by indication, an in-depth analysis was conducted of the partial orphan drugs to determine a “disease” factor to apply to the expenditures of each drug, with the goal of isolating and segmenting orphan uses from non-orphan uses of the drugs. To conduct this analysis, we consulted several U.S. sources to determine the sizes of the different disease populations and factor the sales and volume data. These sources included including the March 2017 Cowen and Company Therapeutic Categories Outlook report, manufacturers' audited financial reports, published epidemiology estimates (incidence or prevalence rates) for the United States, medical claims data, or office-based physician diagnosis surveys collected in QuintilesIMS National Disease and Therapeutic Index (NDTI).⁴¹ The disease factor from the most robust source (taking into account sample size — that is, number of claims; setting of care; and so on) out of all available sources was applied to the total product expenditures of a given drug to measure spending associated with orphan indications only. Approval dates for orphan and non-orphan indications were considered as applicable (the disease factors used have varied across years — for example, an orphan drug might have been approved for an orphan indication only in 2008 and then for a non-orphan indication in 2011, so the disease factor would be 100% orphan from 2008-2010 and then factored to reflect the mix of orphan and non-orphan uses from 2011).

Limitations

QuintilesIMS database coverage may be subject to limitations where volumes are low or distributed through limited wholesaler or pharmacy networks and some product sales may be understated for lower volume products, which could include orphan drugs. Nevertheless, sales data are estimated to represent 98 percent of overall sales in the United States. Factoring sales data by epidemiology, claims or reported diagnosis data are all less robust methods than recording exact sales values. Products with only orphan indications represent a more reliable measure of orphan spending as they are not adjusted by these factors.

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About the QuintilesIMS Institute

The QuintilesIMS Institute leverages collaborative relationships in the public and private sectors to strengthen the vital role of information in advancing healthcare globally. Its mission is to provide key policy setters and decision-makers in the global health sector with unique and transformational insights into healthcare dynamics derived from granular analysis of information.

Fulfilling an essential need within healthcare, the Institute delivers objective, relevant insights and research that accelerate understanding and innovation critical to sound decision-making and improved patient care. With access to QuintilesIMS's extensive global data assets and analytics, the Institute works in tandem with a broad set of healthcare stakeholders, including government agencies, academic institutions, the life sciences industry and payers, to drive a research agenda dedicated to addressing today's healthcare challenges.

By collaborating on research of common interest, it builds on a long-standing and extensive tradition of using QuintilesIMS information and expertise to support the advancement of evidence-based healthcare around the world.

Research Agenda

The research agenda for the Institute centers on five areas considered vital to the advancement of healthcare globally:

The effective use of information by healthcare stakeholders globally to improve health outcomes, reduce costs and increase access to available treatments.

Optimizing the performance of medical care through better understanding of disease causes, treatment consequences and measures to improve quality and cost of healthcare delivered to patients.

Understanding the future global role for biopharmaceuticals, the dynamics that shape the market and implications for manufacturers, public and private payers, providers, patients, pharmacists and distributors.

Researching the role of innovation in health system products, processes and delivery systems, and the business and policy systems that drive innovation.

Informing and advancing the healthcare agendas in developing nations through information and analysis.

Guiding Principles

The Institute operates from a set of Guiding Principles:

The advancement of healthcare globally is a vital, continuous process.

Timely, high-quality and relevant information is critical to sound healthcare decision-making.

Insights gained from information and analysis should be made widely available to healthcare stakeholders.

Effective use of information is often complex, requiring unique knowledge and expertise.

The ongoing innovation and reform in all aspects of healthcare require a dynamic approach to understanding the entire healthcare system.

Personal health information is confidential and patient privacy must be protected.

The private sector has a valuable role to play in collaborating with the public sector related to the use of healthcare data.

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