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Report to the Ranking Member, Committee on the Budget, House of Representatives

October 2015

MEDICARE PART B

Expenditures for New Drugs Concentrated among a Few Drugs, and Most Were Costly for Beneficiaries

GAO-16-12

GAO Highlights

Highlights of GAO-16-12, a report to the Ranking Member, Committee on the Budget, House of Representatives

October 2015

MEDICARE PART B

Expenditures for New Drugs Concentrated among a Few Drugs, and Most Were Costly for Beneficiaries

Why GAO Did This Study

Questions have been raised about the effects of newly approved drugs on spending by the Medicare Part B program and its beneficiaries. Medicare Part B pays for drugs that are commonly physician-administered. In 2013, the Medicare program and its beneficiaries spent $20.9 billion on Part B drugs.

GAO was asked to review newly approved Part B drugs. This report (1) describes drugs newly approved by FDA and paid for by Medicare Part B and compares them to drugs newly approved and not paid for by Part B and (2) analyzes spending and utilization patterns for new Part B drugs.

To describe new Part B drugs, GAO obtained a list from FDA of the 250 drugs approved from 2006 through 2013, including chemically synthesized drugs and biologics. This list was limited to new drugs defined by FDA officials as innovative products that were significantly different from previously approved products. GAO cross-referenced the list with Medicare pricing files to identify 83 new Part B drugs. GAO analyzed the drugs' use of FDA's expedited programs and uses for which they were approved. GAO then compared these Part B drugs to new drugs not paid for under Part B. To analyze the spending and utilization patterns of new Part B drugs, GAO used claims files from CMS to calculate for each drug total Medicare Part B expenditures, spending per beneficiary, and number of unique beneficiaries who received it. GAO identified expenditures in 2013 for 75 of the 83 new Part B drugs.

View GAO-16-12. For more information, contact James Cosgrove at (202) 512-7114 or cosgrovej@gao.gov.

What GAO Found

New Medicare Part B drugs were more likely than new drugs not paid under Part B to be biologics, that is, products derived from living sources; be approved to treat a narrower range of conditions; and to have used a Food and Drug Administration (FDA) program to expedite their development and review. Sixty-one percent of the 83 new Part B drugs approved by FDA from 2006 through 2013 were biologics, compared to 16 percent of new non-Part B drugs. Biologics are more likely to be physician-administered and therefore paid for by Part B because they are usually injected or infused, their administration requires monitoring and individualized dosing, and they have unique storage requirements. Fifty-three percent of new Part B drugs were used to treat cancer or blood diseases, or were used in diagnostic imaging. New Part B drugs were more likely than new non-Part B drugs to have used an FDA expedited program or to have received an orphan designation, which applies to drugs that treat rare conditions and are received by a relatively small number of people.

Expenditures for new Part B drugs were concentrated among a small number of drugs and conditions, and most new Part B drugs were costly for beneficiaries. GAO identified expenditures in 2013 for 75 of the 83 new Part B drugs. Expenditures for these 75 drugs in 2013 were concentrated among 3 drugs—Lucentis, Eylea, and Prolia—which accounted for 53 percent of the $5.9 billion Medicare and its beneficiaries spent on new Part B drugs. The 20 highest expenditure drugs accounted for 92 percent of 2013 expenditures on new Part B drugs and for 26 percent of total expenditures for Part B drugs. Nearly two-thirds of new Part B drugs had expenditures per beneficiary in excess of $9,000 in 2013. Beneficiaries' share of the cost of these drugs ranged from $1,900 to $107,000 per drug in 2013, though many of these drugs received orphan designation and had low utilization. Total Part B drug expenditures grew at an average annual rate of 4.4 percent from 2007 through 2013, and this growth was driven primarily by new Part B drugs.

Five Highest Expenditure New Medicare Part B Drugs, 2013

Drug proprietary name	Approved use	Total expenditures (in millions)	Expenditures per beneficiary
Lucentis	Ophthalmologic	$1,369	$9,423
Eylea	Ophthalmologic	1,088	9,936
Prolia	Orthopedic	665	2,776
Treanda	Cancer	332	21,685
Lexiscan	Diagnostic Imaging	257	215

Source: GAO analysis of CMS and FDA data. I GAO-16-12

GAO received technical comments on a draft of this report from the Department of Health and Human Services, the agency that oversees FDA and the Centers for Medicare & Medicaid Services (CMS), and incorporated these comments as appropriate.

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Abbreviations

ASP	average sales price
AWP	average wholesale price
BLA	biologics license application
CMS	Centers for Medicare & Medicaid Services
DME	durable medical equipment
FDA	Food and Drug Administration
FDCA	Food, Drug, and Cosmetic Act
FFS	fee-for-service
HHS	Department of Health and Human Services
IND	investigational new drug
NDA	new drug application
NDC	national drug code
NME	new molecular entity
NOC	Not Otherwise Classified
WAC	wholesale acquisition cost

U.S. GOVERNMENT ACCOUNTABILITY OFFICE
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October 23, 2015

The Honorable Chris Van Hollen
Ranking Member
Committee on the Budget
House of Representatives

Dear Mr. Van Hollen:

Some of the drugs approved in recent years by the Food and Drug Administration (FDA)—the agency within the Department of Health and Human Services (HHS) responsible for ensuring the safety and effectiveness of drugs—have been associated with high spending. For example one drug, Provenge, which was approved in 2010 to treat prostate cancer, has been reported to cost about $90,000 for a course of treatment. In addition, many recently approved drugs, including Provenge, are complex drugs derived from living sources, known as biologics, and have used one or more expedited programs to shorten the time needed for development and review from FDA.[1] These programs are intended to ensure that therapies for serious conditions are approved and available as soon as it can be concluded that their benefits justify their risks.

Recent trends in drug development and spending have led policymakers and others to question the effect these drugs may have on prescription drug spending and the ability of patients to afford these drugs. These trends have also raised questions about the effect that recently approved drugs could have on spending by the Medicare Part B program and its beneficiaries. Medicare Part B covers prescription drugs not usually self-administered but that are instead commonly administered by a physician or under a physician's close supervision in physicians' offices and hospital outpatient departments.[2] We previously reported that in 2010, the 55 highest-expenditure Part B drugs represented 85 percent of all Medicare spending on Part B drugs and that most of the 55 drugs increased in total expenditures, expenditures per beneficiary, and prices

[1]Biologics—which include vaccines, blood products, and proteins—are derived from living sources such as humans, animals, and microorganisms.

[2]Part B drugs generally differ from those covered under Medicare Part D in that Part D drugs are usually self-administered.

from 2008 to 2010.[3] In 2013, the Medicare program and its beneficiaries spent $20.9 billion on Part B drugs.[4]

In general, the price Medicare Part B pays for drugs is based on the average sales price (ASP), plus an additional 6 percent.[5] ASP is the average price, after rebates and discounts, of sales of a specified drug in the United States; consequently, Medicare Part B's payment rates for drugs are based on prices set by the private market. Medicare Part B pays 80 percent of the expenditures for drugs, and the beneficiary is responsible for the remaining 20 percent, which may be covered by a Medicare supplemental health insurance policy, an employer-sponsored retiree health plan, or Medicaid.[6] In 2010, nearly 90 percent of Medicare beneficiaries had some form of supplemental coverage.[7]

You asked us to provide information on trends in the costs of newly approved Part B drugs and the potential implications of these costs for the Medicare program and its beneficiaries. This report (1) describes the drugs newly approved by FDA and paid for by Medicare Part B and compares them to drugs newly approved and not paid for by Part B; and (2) examines, for new Part B drugs, patterns of Medicare expenditures, including overall Part B expenditures and expenditures per beneficiary, and utilization.

[3]GAO, *Medicare: High-Expenditure Part B Drugs*, GAO-13-46R (Washington, D.C.: Oct. 12, 2012).

[4]For this report, we excluded vaccines for influenza and *haemophilus influenzae* as well as drugs that were billed using Not Otherwise Classified (NOC) billing codes, which are billing codes for drugs lacking specific billing codes.

[5]The ASP is determined by the total sales of a drug to all purchasers in the United States divided by the total number of units of the drug sold by the manufacturer in a calendar quarter, net of any price concessions. Manufacturers report ASPs and volume of sales by national drug code to CMS on a quarterly basis. CMS does not negotiate drug prices with manufacturers. The sequester, which took effect in April 2013, reduces payments for all Medicare services, including Part B drugs, by 2 percent.

[6]A Medicare supplemental health insurance policy is health insurance sold by private insurers that covers Medicare deductibles and copayments and may cover some services that Medicare fee-for-service (FFS) does not cover.

[7]The Henry J. Kaiser Family Foundation, *A Primer on Medicare: Key Facts About the Medicare Program and the People It Covers* (Menlo Park, Calif.: Mar. 20, 2015).

To describe drugs newly approved by FDA and paid for by Medicare Part B, we obtained a list from FDA of the 250 drugs, including chemically synthesized drugs and biologics, approved from 2006 through 2013.[8] This list was limited to new molecular entities (NME) and new biologics identified for us by FDA officials as innovative products that are significantly different than previously approved products and that previously had not been approved for marketing in the United States in any form.[9] We also obtained and analyzed information from FDA on the characteristics of these drugs, such as the condition the drug was approved to treat and the type of expedited program, if any, used in the drug's development and review. In order to compare drugs, we classified drugs into broad categories of conditions they were approved to treat.[10] For example, a drug approved to treat prostate cancer was classified as a cancer drug. We refer to these broader categories as conditions for the purposes of this report.

To identify the subset of the 250 new drugs that were paid for by Part B, we cross-referenced each drug against the Part B drug pricing files maintained by the Centers for Medicare & Medicaid Services (CMS), the

[8]We received a list of chemically synthesized drugs and biologics approved by the two FDA centers responsible for reviewing and approving drugs, the Center for Drug Evaluation and Research and the Center for Biologics Evaluation and Research.

[9]In identifying these drugs, FDA excluded medical devices, transfusion blood products, allergenic products, generics, biosimilars, and over-the-counter products.

[10]Drugs were classified based on the condition(s) they were approved to treat in their initial FDA approval. Conditions subsequently approved by FDA following these drugs' approval and off-label usage of the drugs were not considered. In some cases, a drug could have been classified into more than one category. In such cases, the classification was made based on the clinical judgment of a licensed physician, how a drug works, and the type of specialist usually treating the condition. Additionally, condition categories were defined broadly; for instance, "cancer" includes both drugs for cancer treatment and for the associated complications. We also categorized drugs used in diagnostic imaging as their own condition category. Each drug's categorization was consistent with information from the U.S. National Library of Medicine.

agency within HHS that oversees the Medicare program.[11] We identified 83 drugs that were approved by FDA in calendar years 2006 through 2013 and had either an associated Medicare billing code or price listed in CMS's pricing files. For the purposes of this report, we refer to these 83 chemically synthesized drugs and biologics as new Part B drugs. In order to put new Part B drugs in the broader context of drug approvals, we compared new Part B drugs to the remaining 167 new drugs approved during this time period and not paid for by Part B, which we refer to as new non-Part B drugs.[12] For simplicity, in this report the term "drugs" refers to both chemically synthesized and biologic products, while "synthetic drugs" refers specifically to chemically synthesized drugs and "biologics" refers to molecules derived from living sources.

To examine Medicare expenditure and utilization patterns for the Part B drugs in our analysis, including overall Part B expenditures and expenditures per beneficiary, we used the CMS National Claims History 100 percent file for physician and hospital outpatient services and for durable medical equipment (DME).[13] We analyzed these data for 2007—the first year for which data were available for the drugs in our analysis[14]—through 2013, which was the most recent full year of data

[11]For these drugs, we cross-referenced their national drug codes (NDC)—a numeric drug-specific identifier—against the NDCs listed in CMS's files that link NDCs to drug billing codes for the purpose of Part B payment as well CMS's drug pricing files. Some of the Part B drugs we identified did not have identifiable claims during the time period of our analysis but could have such claims in subsequent years. Our list of drugs includes any new drug that had a drug billing code and price established from 2006 through the first quarter of 2015. The list of 250 drugs provided by FDA excluded 6 influenza vaccines and 1 vaccine for *haemophilus influenzae*, which causes a bacterial infection. We excluded these drugs from our analysis as they may share billing codes with other drugs.

[12]New non-Part B drugs were likely covered by either Medicare Part A or Part D. Medicare Part A covers drugs associated with inpatient treatment provided during a covered stay in a hospital or skilled nursing facility. Medicare Part D covers prescribed drugs not covered under Parts A or B.

[13]The National Claims History file contains all claims for beneficiaries enrolled in the Medicare FFS program.

[14]We analyzed Medicare claims data beginning in 2007 to account for the period of time it takes for newly approved drugs to receive a permanent billing code. According to CMS officials, it may take up to 6 months for a specific billing code to be assigned for a given drug, and during this time period providers may bill Medicare for recently approved drugs using one or more NOC codes. Given that multiple drugs may simultaneously use NOC codes until a specific billing code has been assigned, we excluded Medicare claims billed using these codes from our analysis.

available at the time of our analysis. We identified 2013 Part B claims for 75 of the 83 part B drugs identified in the pricing files.[15] For each of these 75 drugs, we calculated the total amount spent by the Medicare fee-for-service (FFS) program and by or on behalf of its beneficiaries, the number of unique beneficiaries who received the drug, and the average amount spent per Medicare beneficiary (expenditures per beneficiary). We also calculated changes in the average sales price for the highest expenditure new Part B drugs. To safeguard confidential information, we excluded from tables the utilization and expenditures for drugs that were used by 50 or fewer Medicare beneficiaries.

We verified the reliability of FDA-provided information by cross-referencing it against other published FDA sources and by interviewing agency officials who were knowledgeable about the data.[16] We verified the reliability of the Medicare claims data used in this report by performing electronic data checks and by interviewing agency officials who were knowledgeable about the data. We also checked expenditures for the highest expenditure new Part B drugs in the claims data against the published total expenditures for these drugs in CMS's Part B National Summary Files for 2013. We determined that the data used in this report were sufficiently reliable for the purposes of this report.

We conducted this performance audit from July 2014 to October 2015 in accordance with generally accepted government auditing standards. Those standards require that we plan and perform the audit to obtain sufficient, appropriate evidence to provide a reasonable basis for our findings and conclusions based on our audit objectives. We believe that the evidence obtained provides a reasonable basis for our findings and conclusions based on our audit objectives.

[15]The remaining drugs were approved from 2011 through 2013 and did not have identifiable claims during the 2007 to 2013 time period but could have such claims in subsequent years.

[16]These sources included FDA's Drugs@FDA online database, FDA's Orange Book, and the annual NME Drug and New BLA Approval lists published by FDA, which each contain descriptive information about drugs approved by FDA.

Background

Synthetic and Biologic Drugs

While both synthetic drugs and biologics treat diseases and medical conditions, their structures and manufacturing processes differ. Synthetic drugs are produced from specific chemical ingredients and have small, well-defined chemical structures. Conversely, biologics are large compounds that are made in living systems using components from living entities. Biologics may replicate natural substances such as enzymes, antibodies, or hormones. The living systems used to produce biologics can be sensitive to very minor changes in the manufacturing process, which makes them more difficult to produce. Because of their complex structures and complicated manufacturing processes, biologics tend to be less stable, are often heat-sensitive, and are more susceptible to microbial contamination.

FDA's Role in Drug Development and Approval

FDA is responsible for overseeing the safety and effectiveness of drugs marketed in the United States, and the agency's approval is required before new drugs can be marketed for sale. FDA's role in the development of a new drug begins when a drug's sponsor—usually the manufacturer—submits an investigational new drug (IND) application to FDA, which signals the sponsor's intent to conduct clinical investigations to test the drug's diagnostic or therapeutic potential in humans. The IND is submitted following a period of development and animal testing and, when approved, begins a series of phases for a drug's development and approval.

- Phase I: In the first phase, drugs are tested on fewer than a hundred healthy volunteers to determine the drug's safety profile and potential side effects and to determine dosage.[17]
- Phase II: In the second phase, drugs are tested on several hundred patients with the goal of assessing a drug's effectiveness. At the end of Phase II, FDA officials meet with sponsors to discuss how Phase III studies will be conducted.

[17]According to FDA officials, the number of individuals studied varies greatly depending upon the indication, disease and other factors. In the case of a rare disease development program, the number of subjects for each of the phases is generally smaller and is determined on a case-by-case basis.

- Phase III: In the third phase, drugs are tested on several thousand patients to further assess the drug's effectiveness, to study different populations and different dosages, and to examine the uses of the drug in combination with other drugs. At the end of Phase III, sponsors submit a new drug application (NDA) for chemically synthesized drugs and a biologics license application (BLA) for biologics. The NDA or BLA contains data on the safety and effectiveness of the drug as determined through clinical trials and other research. Following receipt of the NDA or BLA, FDA evaluates the sponsor's research on the drug's safety and effectiveness, reviews the drug's labeling information, and inspects the drug's manufacturing facility. At the conclusion of this review, FDA determines whether the drug is approved to be marketed in the United States.

- Phase IV: Under certain circumstances, FDA can require or request sponsors to conduct one or more post-marketing studies as a condition of approval for marketing.[18]

FDA administers four programs to expedite the phases of development and review of new drugs that meet unmet medical need in the treatment of a serious or life-threatening condition: priority review designation, accelerated approval, fast track designation, and breakthrough therapy designation.[19] According to FDA officials, drugs are not limited to using a single expedited program, but may use one or more expedited programs in their development.

- Priority review designation: Under the Prescription Drug User Fee Act of 1992, FDA established the priority review designation to direct additional FDA attention and resources to drug applications and to shorten the goal for reviewing marketing applications from 10 months to 6 months.[20] Priority review designation is available for a drug that

[18]FDA may require or request a post-marketing study if it concludes that additional information, while not essential for approval, is important in improving the prescribing and use of the product; product quality; or consistency in product manufacturing. These studies may confirm existing data, raise or answer questions, or provide new data.

[19]FDA has established nonbinding guidance regarding these four expedited programs. See Food and Drug Administration, *Guidance for Industry: Expedited Programs for Serious Conditions – Drugs and Biologics* (Silver Spring, Md.: May 2014).

[20]See Pub. L. No. 102-571, §§ 102-105, 106 Stat. 4491-4498 (adding Food, Drug, and Cosmetic Act (FDCA) §§ 735, 736; codified as amended at 21 U.S.C. §§ 379g, 379g note, 379h).

treats a serious condition and, if approved, would provide significant improvement in safety or effectiveness. A drug's sponsor is not required to request a priority review designation, as FDA determines whether each drug qualifies for this designation.

- Accelerated approval: FDA implemented the accelerated approval program in 1992 to expedite a drug's approval by making decisions based on data from intermediate or surrogate endpoints, which predict rather than demonstrate clinical effectiveness, instead of final clinical endpoints.[21] For example, FDA may approve a cancer drug that has been shown to reduce tumor size, which is considered an indicator of extended survival, rather than require direct evidence of improved survival. If a drug is granted accelerated approval, the sponsor is required to conduct a Phase IV confirmatory study. FDA may remove a drug's approval if the Phase IV study does not verify clinical benefit. According to FDA officials, a drug's sponsor is not required to request accelerated approval, as FDA determines whether each drug qualifies for this program.

- Fast track designation: The Food and Drug Administration Modernization Act of 1997 required FDA to establish the fast track designation to facilitate the development and expedite the review of drugs that treat serious conditions if evidence suggests they fill unmet medical need.[22] Fast track designation provides the sponsor with additional opportunities to meet with FDA officials during the drug's development as well as the ability to obtain a rolling review, in which portions of a marketing application are reviewed prior to the complete application submission. Sponsors may request fast track designation at any time between submission of an IND and approval of a BLA or NDA.

- Breakthrough therapy designation: The Food and Drug Administrative Safety and Innovation Act, signed into law in 2012, required FDA to establish the breakthrough therapy program to expedite the development of drugs whose preliminary clinical evidence indicates

[21]See 57 Fed. Reg. 58958 (Dec. 11, 1192) (codified as amended at 21 C.F.R. §§ 314.500 et seq. (2014)).

[22]Pub. L. No. 105-115, § 112, 111 Stat. 2296, 2309 (adding FDCA § 506; codified as amended at 21 U.S.C. §356).

substantial improvements over existing therapies.[23] The breakthrough therapy designation expedites drug development and review by providing sponsors with additional FDA guidance on efficient drug development, a commitment from FDA to involve senior managers in the drug's development and review, and eligibility for a rolling review. Sponsors may request breakthrough therapy designation with or any time after the submission of an IND, but ideally a breakthrough therapy designation request should be received by the end of Phase II.

In addition to expedited programs, sponsors of drugs that have been designated to treat orphan diseases or conditions may be entitled to receive incentives under the Orphan Drug Act, including a 7-year market exclusivity period, tax credits for 50 percent of clinical trial costs, waiver of marketing application fees, and grants to public and private entities to defray the costs of development.[24] Sponsors must apply for orphan designation prior to a drug's approval. Such a designation is generally based on a determination that a drug would treat a rare disease or condition affecting fewer than 200,000 persons in the United States.[25] Often, drugs that have been granted orphan designation are the first available treatment for the diseases they treat.

[23]Pub. L. No. 112-144, § 902, 126 Stat. 993, 1086 (2012) (amending FDCA § 356; codified at 21 U.S.C. § 356.

[24]See Pub. L. No. 97-414, §§ 2-5, 96 stat. 2049-2056 (1983) (adding FDCA §§ 525 et seq., Internal Revenue Code § 44H; codified in pertinent part as amended at 21 U.S.C. §§ 360aa et seq., 26 U.S.C. 45C).

[25]Drugs may also receive orphan designation for a subset of persons with a particular disease or condition otherwise affecting 200,000 or more persons when, due to one or more properties of the drug, the remaining persons with such disease or condition would not be appropriate candidates for use of the drug. Drugs for which there is no reasonable expectation of recovery of development and marketing costs by U.S. sales may also be granted orphan designation, even if the disease they treat has a prevalence greater than 200,000 in the United States.

Medicare Payment for Part B Drugs

Medicare Part B generally covers drugs—including both synthetic drugs and biologics— that are not usually self-administered, and include those administered in physician offices and in hospital outpatient departments.[26] Drugs paid for by Medicare Part B are generally purchased by physicians, who are reimbursed by Medicare and its beneficiaries for these drugs' costs based on ASP.[27] In cases where the ASP of a new drug is unavailable, payment may be set at 106 percent of the wholesale acquisition cost (WAC), which is the manufacturer's list price to wholesalers. If the WAC is not available for the new drugs, payment is often based on the invoice price.[28]

New Part B Drugs Were More Likely to Be Biologics and to Treat Fewer Conditions Than New Non-Part B Drugs

The majority—61 percent—of the 83 Part B drugs approved by FDA from 2006 through 2013 were biologics, compared to 16 percent of new non-Part B drugs. Additionally, two-thirds of all biologics approved by FDA during this time were paid for by Part B. (For more information on the 83 Part B drugs approved by FDA from 2006 through 2013, see app. I.)

Certain qualities of biologics make them more likely to be physician-administered and, therefore, paid for by Part B. They are usually injected or infused directly into the bloodstream because they would be destroyed if taken orally and digested. Their administration may need to be closely monitored because they can cause immune reactions in patients and the

[26]Examples of drugs paid for by Part B include certain vaccines (influenza, pneumococcal, and hepatitis B), osteoporosis drugs, oral cancer drugs if the same drug is available in injectable form, antinausea drugs used as part of an anticancer chemotherapeutic regimen, erythropoiesis-stimulating agents, blood clotting factors for hemophilia patients, drugs infused through DME, and immunosuppressive drugs for transplant patients.

[27]Payment to physicians is set at 106 percent of ASP for most Part B drugs they administer; however, payment for some Part B drugs is set on a different basis. Vaccines, infusions, drugs furnished through DME, and blood products are paid at 95 percent of average wholesale price (AWP), which is the manufacturer's average price to wholesalers. Payment for Part B drugs administered in hospital outpatient departments is determined based on ASP, though the rate can vary from year to year; according to CMS, in 2015, the rate was ASP plus 6 percent. Part B drugs in the hospital outpatient setting are paid separately if the per day expenditure of the drug exceeds a certain threshold set by CMS each year. According to CMS, in 2015, this threshold was $95 per day.

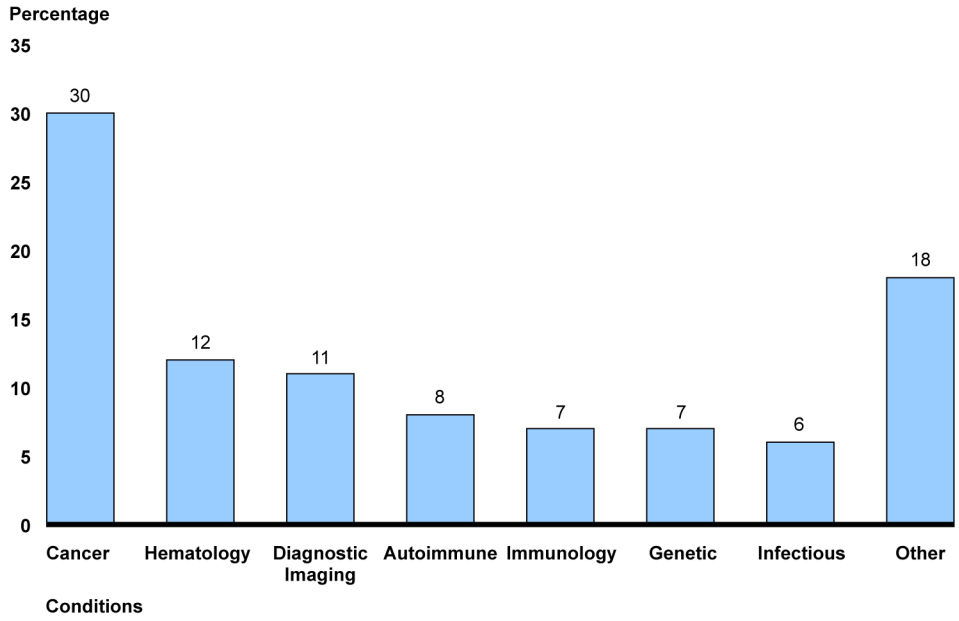
[28]For drugs provided in the hospital outpatient setting, however, payment is 95 percent of the published AWP. Medicare makes additional payments for certain drugs administered in the hospital outpatient setting in order to make drugs more accessible while a pricing history is developed. These are known as transitional pass-through payments, which can be paid for 2 to 3 years at 106 percent of ASP.

appropriate dose may need to be individually calculated for each patient. For example, one Part B drug—Wilate—is a biologic that treats a type of bleeding disorder by replacing deficient clotting substances, and its dosing is based on a patient's weight and severity of bleeding. Biologics are also more likely to be Part B drugs because they often require special handling and processing due to their sensitivity to physical conditions. For example, biologics may be degraded by light, heat, and movement.

New Part B drugs were approved to treat a narrower range of conditions than new non-Part B drugs. New Part B drugs were approved to treat 14 conditions, while new non-Part B drugs were approved to treat 20 conditions. Fifty-three percent of new Part B drugs were approved to treat cancer or blood diseases, or were used in diagnostic imaging (see fig. 1).[29] The three most common conditions treated by new non-Part B drugs—infectious, cancer, and cardiology—comprised 46 percent of new non-Part B drugs. More specifically, 30 percent of new Part B drugs were approved to treat cancers, compared to 14 percent of new non-Part B drugs. In addition, new non-Part B drugs were approved for several conditions for which there were no Part B drugs approved, which included psychiatric, pulmonary, gynecologic, urologic, and neonatal conditions.

[29]Drugs used in diagnostic imaging were categorized as their own condition category in this report.

Figure 1: Percentage of New Part B Drugs Approved by the Food and Drug Administration, 2006-2013, by Condition Approved to Treat



Source: GAO analysis of CMS and FDA data. | GAO-16-12

Notes: We identified new Part B drugs using the list of new molecular entities and new biologics approved by FDA from 2006 through 2013 and comparing it with CMS's Part B pricing files.

We excluded vaccines for influenza and haemophilus influenzae as well as drugs billed using not otherwise classified drug codes from our identification of these drugs.

We categorized drugs' approved treatment conditions into 14 categories. Drugs used in diagnostic imaging were categorized as their own condition category. The "other" category includes the following conditions, each of which had fewer than three drugs approved from 2006 through 2013: neurology, ophthalmology, dermatology, orthopedic, cardiology, gastroenterology, endocrinology, and vaccines.

Characteristics of certain drugs may make them more likely to be paid for under Part B because they are generally administered by a physician or under a physician's direct supervision. Drugs that treat cancer and autoimmune conditions often provoke an immune response, which needs to be monitored by health care providers. For example, Yervoy is a biologic that treats melanoma by activating a patient's immune system to attack cancer cells. Additionally, drugs used in diagnostic imaging are usually injected directly into the blood stream to allow for better visualization of blood vessels. For example, Lexiscan, approved in 2008 for use in myocardial perfusion imaging, or imaging of the heart's blood supply, must be injected directly into the blood stream during a cardiac stress test to assess heart attack risk.

The use of expedited programs—which were granted for drugs that treat serious conditions and address unmet medical need—was greater among new Part B drugs than new non-Part B drugs. Sixty-one percent of new Part B drugs used one or more programs, compared to 38 percent of new non-Part B drugs. The most commonly used expedited program among new Part B drugs was priority review designation (see table 1). Fifty-three percent of new Part B drugs used priority review designation compared to 32 percent of new non-Part B drugs. Only one new Part B drug approved from 2006 through 2013, Gazyva,[30] was granted breakthrough designation, as this program did not go into effect until the middle of 2012. In addition, several new Part B drugs used multiple expedited programs in their development and review. The most commonly used combination of programs was priority review designation and fast track designation, with 22 percent of new Part B drugs using both of these programs.

New Part B drugs were more likely to have received orphan designation in their development and approval than new non-Part B drugs (see table 1). Nearly half, or 47 percent, of new Part B drugs received orphan designation, meaning they were approved to treat rare conditions, compared to 24 percent of new non-Part B drugs. For example, Kyprolis is an orphan Part B drug approved in 2012 to treat multiple myeloma, a cancer estimated to have affected fewer than 90,000 people in the United States in 2012.[31] Some new Part B drugs with orphan designation were the first available therapy for their approved treatment condition. For example, Elaprase was approved in 2006 as the first product to treat Hunter syndrome, a rare genetic disorder that leads to premature death.

[30]Gazyva was approved to treat a type of blood cancer.

[31]National Cancer Institute. *SEER Cancer Statistics Factsheets: Myeloma*, accessed July 28, 2015, http://seer.cancer.gov/statfacts/html/mulmy.html.

Table 1: Descriptive Characteristics of New Part B Drugs Approved by FDA 2006-2013, by Number and Percentage of Drugs

Approval year	Number of drugs approved	Biologic (%)	Type of expedited program			Orphan drug (%)
			Priority review designation (%)	Accelerated approval (%)	Fast track designation (%)	
2006	8	75	63	13	50	38
2007	7	43	57	0	14	57
2008	14	36	36	0	14	43
2009	12	67	42	17	50	75
2010	14	71	57	0	43	29
2011	10	80	80	20	60	60
2012	10	60	50	20	40	40
2013	8	63	50	0	13	38
All years	83	61	53	8	36	47

Source: GAO analysis of FDA and CMS data. I GAO-16-12

Notes: We identified new Part B drugs using the list of new molecular entities and new biologics approved by FDA from 2006 through 2013 and comparing it with CMS's Part B pricing files.

We excluded vaccines for influenza and haemophilus influenzae as well as drugs billed using not otherwise classified drug codes from our identification of these drugs.

Breakthrough therapy designation was not included in this table because that program was first implemented in 2012 and therefore only available to drugs approved in 2012 or later. One Part B drug approved during the period 2006 through 2013 received breakthrough designation.

Expenditures Were Concentrated among a Few Drugs, and Most New Drugs Were Costly for Beneficiaries

Expenditures for New Part B Drugs in 2013 Were Concentrated among a Few Drugs and Conditions

Expenditures for the 75 new Part B drugs for which we identified claims in 2013 were concentrated among a small number of drugs. The 20 highest expenditure drugs accounted for 92 percent of 2013 expenditures on new Part B drugs and 26 percent of total Part B drug expenditures. Biologics accounted for 13 of the top 20 highest expenditure new Part B drugs and 82 percent of expenditures for these 20 drugs (see table 2). Three new Part B drugs—Lucentis, Eylea, and Prolia—accounted for 53 percent of the $5.9 billion Medicare and its beneficiaries spent on new Part B drugs in 2013. These three drugs accounted for 15 percent of the $20.9 billion Medicare and its beneficiaries spent on all Part B drugs in 2013. These

three drugs were all biologics and were approved to treat chronic conditions, for which treatment is required on an ongoing basis.

Table 2: 20 Highest Expenditure New Part B Drugs, 2013

Rank	Drug proprietary name (biologics bolded)	Condition	Total expenditures (in millions)	Percentage of new Part B drug expenditures	(cumulative %)
1	**Lucentis**	Ophthalmologic	$1,369	23%	(23%)
2	**Eylea**	Ophthalmologic	1,088	19	(42)
3	**Prolia**	Orthopedic	665	11	(53)
4	Treanda	Cancer	332	6	(59)
5	Lexiscan	Diagnostic imaging	257	4	(63)
6	**Yervoy**	Cancer	224	4	(67)
7	**Privigen**	Immunologic	184	3	(70
8	**Provenge**	Cancer	183	3	(73)
9	**Soliris**	Hematologic	150	3	(76)
10	Dacogen	Hematologic	147	3	(78)
11	**Actemra**	Autoimmune	130	2	(80)
12	**Hizentra**	Immunologic	128	2	(83)
13	**Nplate**	Hematologic	118	2	(85)
14	**Cimzia**	Gastroenterological	71	1	(86)
15	Jevtana	Cancer	68	1	(87)
16	Emend	Cancer	65	1	(88)
17	Kyprolis	Cancer	62	1	(89)
18	**Vectibix**	Cancer	56	1	(90)
19	**Lumizyme**	Genetic	52	1	(91)
20	Halaven	Cancer	51	1	(92)
Total			**$5,400**	**92%**	

Source: GAO analysis of FDA and CMS data. I GAO-16-12

Notes: Expenditures were calculated for the 75 new Part B drugs for which we identified claims in 2013. We identified new Part B drugs using the list of new molecular entities and new biologics approved by FDA from 2006 through 2013 and comparing it with CMS's Part B pricing files.

We excluded vaccines for influenza and haemophilus influenzae as well as drugs billed using not otherwise classified drug codes from our identification of these drugs.

We categorized drugs' approved treatment conditions into 14 categories. Drugs used in diagnostic imaging were categorized as their own condition category.

The 20 highest expenditure new Part B drugs in 2013 generally had high expenditures because of their high utilization. Of the top 20 drugs, 13 were also in the top 20 in utilization, whereas 4 were in the top 20 in expenditures per beneficiary (see table 3). For example, Lexiscan and

Lucentis had very high expenditures primarily because of their high utilization—ranking 1st and 3rd, respectively, for utilization but 69th and 49th, respectively, in per beneficiary expenditures.[32] However, two drugs that were among the 20 highest expenditure drugs, Soliris and Lumizyme, had low utilization and high per beneficiary expenditures. For example, Soliris, which was approved to treat a hematologic condition, had the 3rd highest per beneficiary expenditures ($341,000) in 2013, but ranked 40th in utilization. (For more information on each drug's expenditures and utilization, see app. II.)

Table 3: 20 Highest Expenditure New Part B Drugs in 2013 Ranked by Utilization and Expenditures per Beneficiary

20 highest expenditure new Part B drugs that:		
Ranked in top 20 for utilization (13 drugs)	**Ranked in top 20 for expenditures per beneficiary (4 drugs)**	**Ranked below top 20 for utilization and expenditures per beneficiary (3 drugs)**
Lucentis	Yervoy	Jevtana
Eylea	Provenge	Kyprolis
Prolia	Soliris	Vectibix
Treanda	Lumizyme	
Lexiscan		
Privigen		
Dacogen		
Actemra		
Hizentra		
Nplate		
Cimzia		
Emend		
Halaven		

Source: GAO analysis of FDA and CMS data. I GAO-16-12

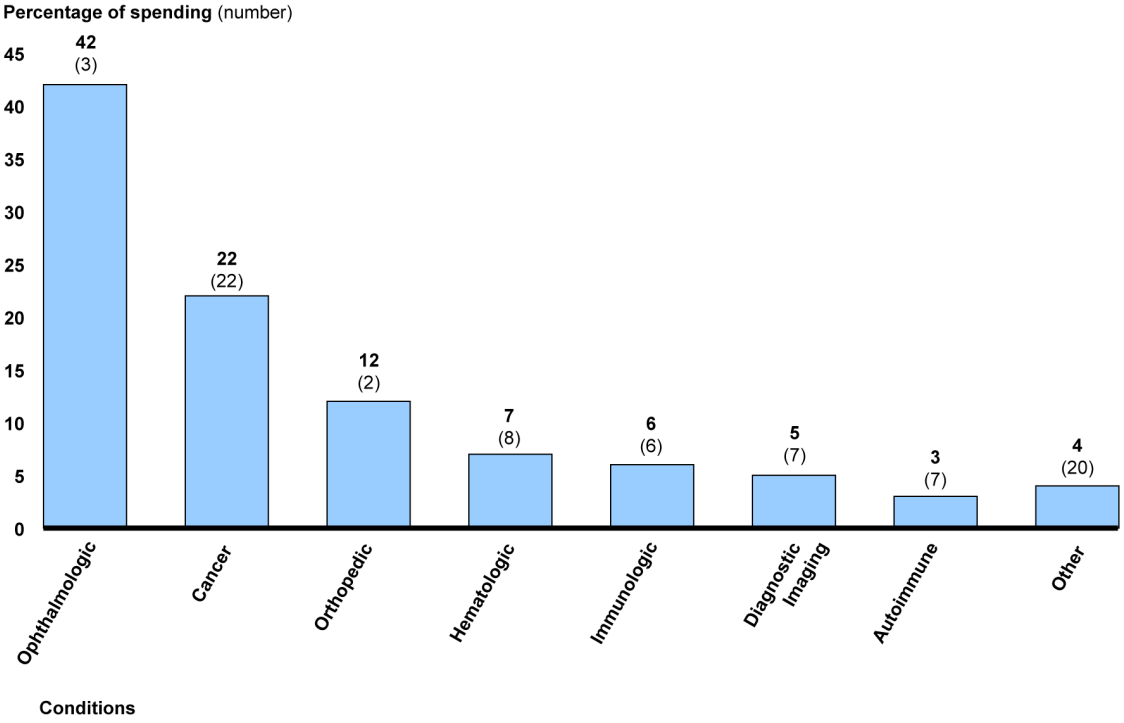
Notes: Utilization and per beneficiary expenditures were calculated for the 75 new Part B drugs for which we identified claims in 2013. We identified new Part B drugs using the list of new molecular entities and new biologics approved by FDA from 2006 through 2013 and comparing it with CMS's Part B pricing files.

We excluded vaccines for influenza and haemophilus influenzae as well as drugs billed using not otherwise classified drug codes from our identification of these drugs.

[32]Lexiscan is a chemical stress agent used to test heart function in patients who cannot take a stress test on a treadmill or a stationary bike.

Expenditures for the 75 new Part B drugs that had claims in 2013 were concentrated among a few of the 14 conditions that new Part B drugs were approved to treat. For example, 3 ophthalmologic drugs accounted for 42 percent of new Part B drug expenditures and 22 cancer drugs accounted for 22 percent of expenditures (see fig. 2). Furthermore, 96 percent of the 2013 expenditures for new Part B drugs were concentrated among 7 conditions: ophthalmologic, cancer, orthopedic, hematologic, immunologic, diagnostic imaging, and autoimmune. The remaining 7 conditions accounted for 20 drugs and 4 percent of expenditures.[33]

Figure 2: New Part B Drug Treatment Conditions, by Percentage of Expenditures, 2013



Source: GAO analysis of CMS and FDA data. | GAO-16-12

Notes: We identified new Part B drugs using the list of new molecular entities and new biologics approved by FDA from 2006 through 2013 and comparing it with CMS's Part B pricing files.

We excluded vaccines for influenza and haemophilus influenzae as well as drugs billed using not otherwise classified drug codes from our identification of these drugs.

[33]These conditions included genetic, gastroenterological, infectious, dermatologic, neurologic, endocrine, and cardiac conditions.

We categorized drugs' approved treatment conditions into 14 categories. Drugs used in diagnostic imaging were categorized as their own condition category. The "other" category includes the following conditions: genetic, gastroenterological, infectious, dermatologic, neurologic, endocrine, and cardiac conditions.

Most New Part B Drugs Were Costly for Beneficiaries

Nearly two-thirds of new Part B drugs (49 drugs) had annual per beneficiary expenditures in excess of $9,000 in 2013, and nearly 50 percent (37 drugs) had per beneficiary expenditures greater than $20,000. The 49 drugs with per beneficiary expenditures over $9,000 were used by approximately 332,000 beneficiaries.[34] These beneficiaries were accountable to pay the associated 20 percent of cost sharing, which ranged from $1,900 to $107,000 per year for each of these drugs. Most beneficiaries have some form of supplemental insurance that may help cover the cost of Part B drugs.[35] However, especially for beneficiaries without such coverage, these high cost-sharing amounts could result in a substantial financial burden relative to the average Medicare beneficiary's annual income of $23,500 in 2013.[36] Beneficiaries likely were responsible for additional cost sharing for prescription drugs as they tend to take more than one.[37]

The 20 new Part B drugs with the highest per beneficiary expenditures were particularly costly, with yearly per beneficiary expenditures ranging from $51,000 to $536,000 in 2013. Among the 20 highest per beneficiary expenditure drugs that were used by more than 50 beneficiaries, per beneficiary expenditures ranged from $51,000 to $457,000 (see table 4 for these drugs). The associated cost sharing for these drugs ranged from $10,000 per beneficiary for Arzerra, a drug approved to treat leukemia, to $91,000 per beneficiary for Lumizyme. Most of these 20 drugs were orphan drugs that treated rare diseases, and therefore utilization of these drugs was generally low (see table 4). Because of the low utilization of these drugs, only 4 were among the 20 new Part B drugs with highest

[34]Cost-sharing calculations were based on the number of beneficiaries receiving each drug, and beneficiaries receiving multiple drugs would be counted more than once.

[35]The Henry J. Kaiser Family Foundation, *A Primer on Medicare*.

[36]According to analysis conducted by the Urban Institute and Kaiser Family Foundation, the median annual income for Medicare beneficiaries in 2013 was $23,500.

[37]According to the National Center for Health Statistics, 67 percent of individuals aged 65 years and over took three or more prescription drugs within a 30-day period from 2007 through 2010. See National Center for Health Statistics, *Health, United States, 2012: With Special Feature on Emergency Care* (Hyattsville, Md: 2013).

expenditures in 2013. (For more detailed information on the expenditures and utilization for each drug, see app. II.)

Table 4: Highest Per Beneficiary Expenditures for New Part B Drugs Used by More Than 50 Beneficiaries, 2013

Drug proprietary name (orphan drugs bolded)	Condition	Annual expenditures Per beneficiary (20% cost sharing)		Number of beneficiaries
Lumizyme	Genetic	$457,000	($91,000)	114
Soliris	Hematologic	341,000	(68,000)	441
Vpriv	Genetic	292,000	(58,000)	111
Xyntha	Hematologic	196,000	(39,000)	72
Yervoy	Cancer	93,000	(19,000)	2,419
Provenge	Cancer	86,000	(17,000)	2,123
Folotyn	Cancer	84,000	(17,000)	211
Adcetris	Cancer	62,000	(12,000)	671
Istodax	Cancer	58,000	(12,000)	355
Glassia	Genetic	56,000	(11,000)	84
Arzerra	Cancer	51,000	(10,000)	699

Source: GAO analysis of CMS and FDA data. I GAO-16-12

Notes: Results are for the 11 new Part B drugs that were in the top 20 in per beneficiary expenditures in 2013 and were used by more than 50 beneficiaries. We excluded from this table drugs for which claims were submitted for fewer than 50 beneficiaries. If not for this exclusion, the following drugs would have been included in this table: Elaprase, Myozyme, Elelyso, Corifact, Erwinaze, Voraxaze, Wilate, Cinryze, and Ceprotin.

We identified new Part B drugs using the list of new molecular entities and new biologics approved by FDA from 2006 through 2013 and comparing it with CMS's Part B pricing files. We excluded vaccines for influenza and haemophilus influenzae as well as drugs billed using not otherwise classified drug codes from our identification of these drugs.

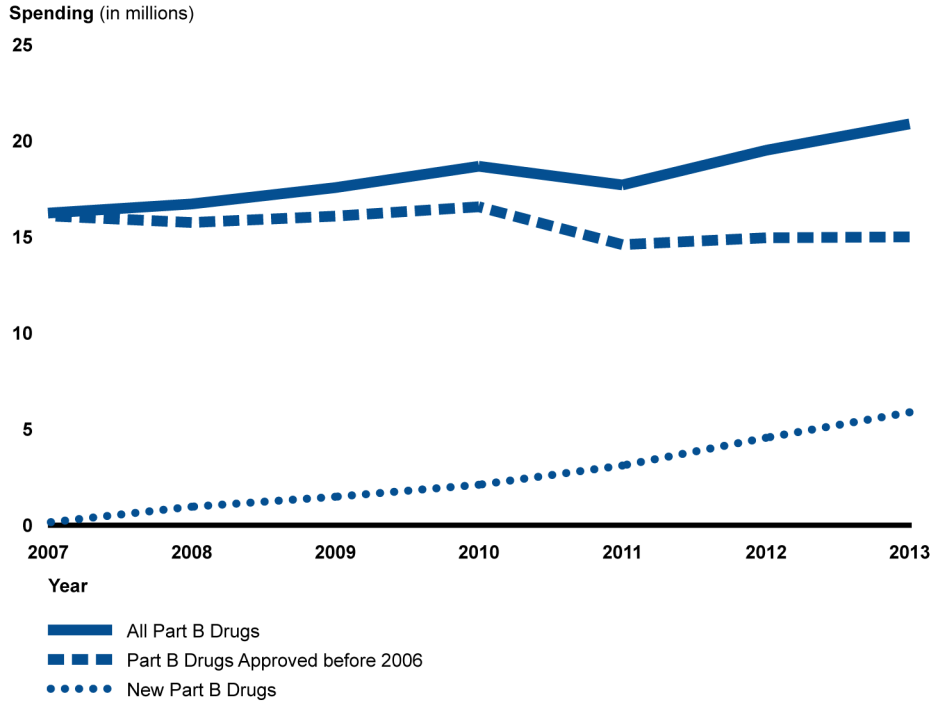
We categorized drugs' approved treatment conditions into 14 categories. Drugs used in diagnostic imaging were categorized as their own condition category.

The Increase in Total Part B Drug Expenditures Was Driven by New Part B Drugs

Growth in total Part B drug expenditures from 2007 through 2013 was driven primarily by new Part B drugs. Total Part B drug expenditures increased at an average annual rate of 4.4 percent from 2007 to 2013, from $16.2 billion to $20.9 billion. Total expenditures for new Part B drugs increased steadily from 2007 to 2013, from $0.1 to $5.9 billion, as the number of new drugs and the number of beneficiaries receiving them increased. In contrast, expenditures for Part B drugs approved before 2006 decreased at an average annual rate of 1 percent from 2007

through 2013, from $16.1 billion to $15 billion (see fig. 3).[38] Because many new Part B drugs were orphan drugs and used expedited programs, it is unlikely that new Part B drugs coming on to the market from 2006 through 2013 were substitutes for Part B drugs approved prior to 2006.[39] Drugs that are granted orphan designation are often the first available treatment for the rare diseases they treat. Drugs qualifying for expedited programs generally demonstrate the potential to fill an unmet medical need.

Figure 3: 2007-2013 Expenditures for All Part B Drugs, Part B Drugs Approved 2006-2013, and Part B Drugs Approved Prior to 2006



Source: GAO analysis of CMS and FDA data. | GAO-16-12

Notes: We identified new Part B drugs using the list of new molecular entities and new biologics approved by FDA from 2006 through 2013 and comparing it with CMS's Part B pricing files.

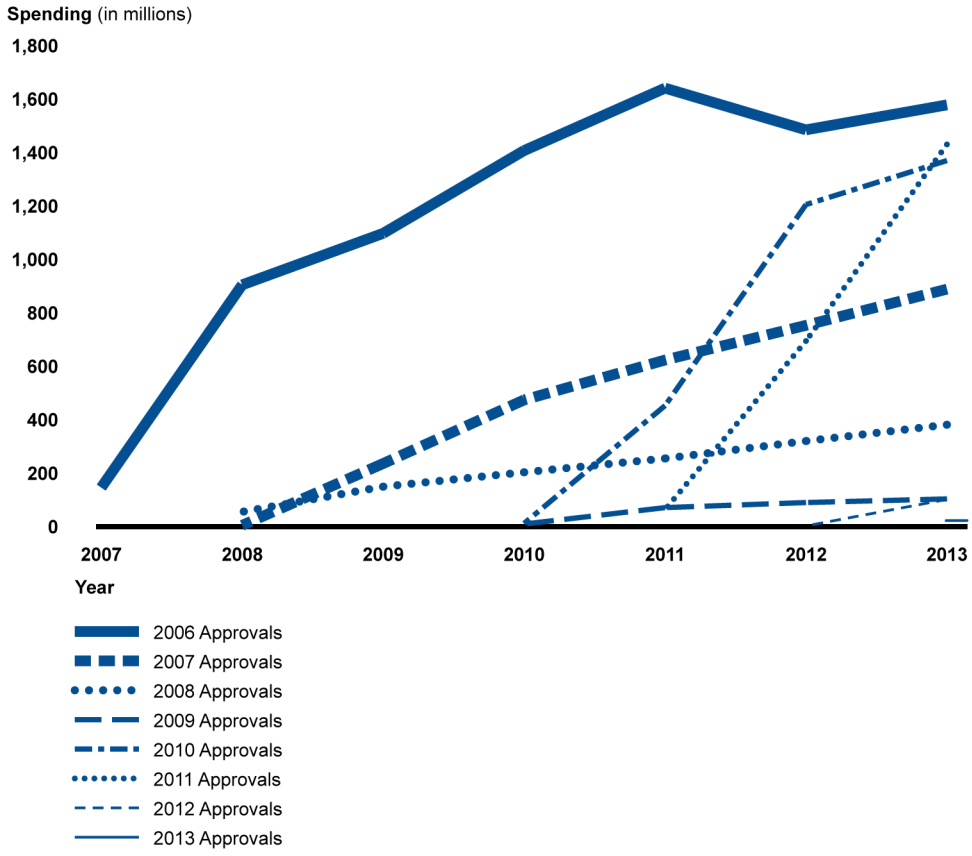
[38]After adjusting for inflation using the gross domestic product index, the annual percentage change in expenditures from 2007 through 2013 was 2.8 percent for all Part B drugs and was -2.5 percent for drugs approved before 2006.

[39]Utilization and expenditures for Part B drugs approved by FDA before 2006 were not analyzed.

We excluded vaccines for influenza and haemophilus influenzae as well as drugs billed using not otherwise classified drug codes from our identification of these drugs.

There was generally a rapid increase in expenditures for new Part B drugs in the years immediately following their approval as more beneficiaries used these drugs (see fig. 4). Drugs approved in 2006, 2010, and 2011 had the highest increases in total expenditures, and these increases were driven by a few drugs, including Lucentis (approved in 2006), Prolia (approved in 2010), and Eylea (approved in 2011). For example, expenditures for drugs approved in 2011 increased 106 percent from 2012 to 2013, from $695 million to $1.4 billion; Eylea alone accounted for 91 percent of that increase. The increase in total expenditures was lower for drugs approved in 2009, 2012, and 2013. Expenditures for drugs approved in 2012 and 2013 were likely low in both years because of the time it takes between a drug's approval and the appearance of identifiable Medicare claims.

Figure 4: Change in Expenditures from Date of Approval to 2013 for New Part B Drugs, by Year Approved



Source: GAO analysis of CMS and FDA data. | GAO-16-12

Notes: We identified new Part B drugs using the list of new molecular entities and new biologics approved by FDA from 2006 through 2013 and comparing it with CMS's Part B pricing files.

We excluded vaccines for influenza and haemophilus influenzae as well as drugs billed using not otherwise classified drug codes from our identification of these drugs.

Expenditures for the 20 highest expenditure new Part B drugs in 2013 generally increased from 2011 through 2013, and this increase was primarily due to higher utilization during this time. The change in expenditures for these 20 drugs ranged from a 5 percent decrease to a 992 percent increase. The corresponding changes in utilization were of a similar magnitude, from a 6 percent decrease to a 1,274 percent increase

(see table 5).[40] In contrast, the changes in expenditures per beneficiary were much smaller in magnitude, ranging from -21 to 30 percent. For example, expenditures for Prolia increased 992 percent from 2011 to 2013, because of a 1,274 percent increase in utilization. The increase in expenditures for Prolia occurred despite a concurrent decrease in the drug's per beneficiary expenditures of 21 percent. The changes in ASP, which is a component of per beneficiary expenditures, were modest and therefore had a minimal effect on the change in drugs' total expenditures.[41] (For more detailed information on changes in expenditures, utilization, and ASP, see app. III.)

Table 5: Percentage Change in Expenditures and Utilization, 2011-2013, for the 20 Highest Expenditure Part B Drugs in 2013

Drug Proprietary Name	Percentage change in		
	Expenditures	Utilization	Expenditures per beneficiary
Lucentis	-5	9	-13
Vectibix	0	-6	6
Dacogen	15	5	10
Lexiscan	19	15	3
Hizentra	25	20	4
Privigen	43	29	11
Treanda	43	30	10
Nplate	48	36	9
Emend	62	55	4
Provenge	64	60	3
Lumizyme	73	36	28
Jevtana	100	73	15
Soliris	114	87	15
Actemra	142	86	30
Cimzia	219	145	30
Halaven	309	225	26
Yervoy	330	271	16

[40]We did not identify any Medicare claims in 2011 for two drugs—Eylea and Kyprolis—that were approved in 2011 and 2012, respectively.

[41]Per beneficiary expenditures is a function of both the unit price of a drug (a drug's ASP) and the number of units of a drug each beneficiary receives.

Drug Proprietary Name	Percentage change in Expenditures	Utilization	Expenditures per beneficiary
Prolia	992	1,274	-21
Eylea	[a]	[a]	[a]
Kyprolis	[a]	[a]	[a]

Source: GAO analysis of CMS and FDA data. I GAO-16-12

Notes: Expenditures are for 20 of the 75 new Part B drugs for which we identified claims in 2013.

We identified new Part B drugs using the list of new molecular entities and new biologics approved by FDA from 2006 through 2013 and comparing it with CMS's Part B pricing files. We excluded influenza and haemophilus influenzae as well as drugs billed using not otherwise classified drug codes from our identification of these drugs.

[a]We did not identify any Medicare claims in 2011 for two drugs—Eylea and Kyprolis—that were approved in 2011 and 2012, respectively.

Agency Comments

We received technical comments on a draft of this report from HHS and incorporated these comments as appropriate.

As agreed with your office, unless you publicly announce the contents of this report earlier, we plan no further distribution until 30 days from the report date. At that time, we will send copies of this report to the appropriate congressional committees, and the Secretary of Health and Human Services. In addition, the report will be available at no charge on the GAO website at http://www.gao.gov.

If you or your staff have any questions about this report, please contact me at (202) 512-7114 or cosgrovej@gao.gov. Contact points for our Offices of Congressional Relations and Public Affairs may be found on the last page of this report. GAO staff who made key contributions to this report are listed in appendix IV.

Sincerely yours,

James Cosgrove
Director, Health Care

Appendix I: Descriptive Information for Part B Drugs Approved by FDA from 2006 through 2013

Proprietary name (nonproprietary name)	Condition (type of condition)	Drug type	Orphan	Priority review	Accelerated approval	Fast track	Expenditures, 2013 (thousands)	Utilization, 2013	Expenditures per beneficiary, 2013
Drugs approved in 2006									
Vivaglobin (immunoglobulin G)	primary immunodeficiency (immunologic)	B		✓			N/R	N/R	N/R
HepaGam B (hepatitis B immune globulin)	post-exposure hepatitis B prophylaxis (infectious)	B					$2,602	348	$7,476
Eraxis (anidulafungin)	esophageal candidiasis (infectious)	S					$79	52	$1,510
Myozyme (alglucosidase alfa)	Pompe disease (genetic)	B	✓	✓		✓	N/R	N/R	N/R
Dacogen (decitabine)	myelodysplastic syndrome (hematologic)	S	✓			✓	$147,066	4,747	$30,981
Lucentis (ranibizumab)	age-related macular degeneration (ophthalmologic)	B		✓			$1,369,374	145,325	$9,423
Elaprase (idursulfase)	Hunter syndrome (genetic)	B	✓	✓		✓	N/R	N/R	N/R
Vectibix (panitumumab)	colorectal cancer (cancer)	B		✓	✓	✓	$55,654	2,081	$26,744
Drugs approved in 2007									
Soliris (eculizumab)	paroxysmal nocturnal hemoglobinuria (hematologic)	B	✓	✓			$150,161	441	$340,500
Ceprotin (protein C concentrate)	protein C deficiency (hematologic)	B	✓	✓			N/R	N/R	N/R
Torisel (temsirolimus)	renal cell carcinoma (cancer)	S	✓	✓		✓	$29,933	1,761	$16,998
Privigen (immunoglobulin G)	primary immunodeficiency; chronic immune thrombocytopenia (immunologic)	B					$183,561	9,019	$20,353
Somatuline (lanreotide)	acromegaly (endocrine)	S	✓				$1,752	67	$26,151

Proprietary name (nonproprietary name)	Condition (type of condition)	Drug type	Orphan	Priority review	Accelerated approval	Fast track	Expenditures, 2013 (thousands)	Utilization, 2013	Expenditures per beneficiary, 2013
Doribax (doripenem)	complicated urinary tract infections (infectious)	S					$34	66	$511
Ixempra (ixabepilone)	breast cancer (cancer)	S		✓			$15,937	1,000	$15,937
Drugs approved in 2008									
Emend (fosaprepitant)	chemotherapy-induced nausea and vomiting (cancer)	S					$65,391	60,532	$1,080
Xyntha (Factor VIII)	hemophilia A (hematologic)	B	✓				$14,092	72	$195,721
Artiss (fibrin sealant)	sealant for skin graft (dermatologic)	B					$98	584	$167
Treanda (bendamustine)	chronic lymphocytic leukemia (cancer)	S	✓	✓			$331,513	15,288	$21,685
Lexiscan (regadenoson)	myocardial perfusion imaging (diagnostic imaging)	S					$257,277	1,198,585	$215
Cimzia (certolizumab pegol)	Crohn's disease (gastroenterological)	B					$70,852	5,744	$12,335
Eovist (gadoxetate disodium)	magnetic resonance imaging of liver (diagnostic imaging)	S					$768	4,337	$177
Cleviprex (clevidipine)	hypertension (cardiac)	S					$6	52	$109
Nplate (romiplostim)	chronic immune thrombocytopenia (hematologic)	B	✓	✓		✓	$118,120	3,250	$36,344
AdreView (Iobenguane I-123)	scintigraphy in the detection of pheochromocytoma and neuroblastoma (diagnostic imaging)	S	✓	✓			N/R	N/R	N/R
Cinryze (C1 esterase inhibitor)	hereditary angioedema (autoimmune)	B	✓	✓		✓	N/R	N/R	N/R
Mozobil (plerixafor)	stem cell mobilization in multiple myeloma and non-Hodgkin's lymphoma (cancer)	S	✓	✓			$15,407	1,188	$12,968

Proprietary name (nonproprietary name)	Condition (type of condition)	Drug type	Orphan	Priority review	Accelerated approval	Fast track	Expenditures, 2013 (thousands)	Utilization, 2013	Expenditures per beneficiary, 2013
Ablavar (gadofosveset)	magnetic resonance angiography (diagnostic imaging)	S					$43	385	$112
Firmagon (degarelix)	prostate cancer (cancer)	S					$12,790	10,733	$1,192
Drugs approved in 2009									
Dysport (botulinum toxin, type A)	cervical dystonia (neurologic)	B	✓				$4,722	2,242	$2,106
Ilaris (canakinumab)	cryopyrin-associated periodic syndromes (autoimmune)	B	✓	✓		✓	N/R	N/R	N/R
Vibativ (telavancin)	skin and subcutaneous infections (infectious)	S				✓	N/R	N/R	N/R
Gammaplex (immunoglobulin G)	primary humoral immunodeficiency (immunologic)	B					$4,585	301	$15,231
Folotyn (pralatrexate)	peripheral T-cell lymphoma (cancer)	S	✓	✓	✓	✓	$17,687	211	$83,826
Stelara (ustekinumab)	psoriasis (dermatologic)	B					$17,119	801	$21,373
Berinert (C1 esterase inhibitor)	hereditary angioedema (autoimmune)	B	✓	✓			N/R	N/R	N/R
Arzerra (ofatumumab)	chronic lymphocytic leukemia (cancer)	B	✓	✓	✓	✓	$35,807	699	$51,225
Istodax (romidepsin)	cutaneous T-cell lymphoma (cancer)	S	✓			✓	$20,572	355	$57,950
Qutenza (capsaicin)	neuropathic pain (neurologic)	S	✓				$301	228	$1,319
Kalbitor (ecallantide)	hereditary angioedema (autoimmune)	B	✓	✓		✓	N/R	N/R	N/R
Wilate (von Willebrand factor and factor VIII)	von Willebrand disease (hematologic)	B	✓				N/R	N/R	N/R

Proprietary name (nonproprietary name)	Condition (type of condition)	Drug type	Orphan	Priority review	Accelerated approval	Fast track	Expenditures, 2013 (thousands)	Utilization, 2013	Expenditures per beneficiary, 2013
Drugs approved in 2010									
Actemra (tocilizumab)	rheumatoid arthritis (autoimmune)	B					$129,669	9,759	$13,287
Xiaflex (collagenase clostridium histolyticum)	Dupuytren's contracture (orthopedic)	B	✓	✓			$22,448	5,834	$3,848
Prevnar 13 (pneumococcal conjugate vaccine 13)	immunization against streptococcus pneumonia (infectious)	B		✓		✓	$19,368	139,867	$139
Vpriv (velaglucerase alfa)	Type I Gaucher disease (genetic)	S	✓	✓		✓	$32,416	111	$292,035
Hizentra (immunoglobulin G)	primary immunodeficiency (immunologic)	B					$128,262	2,625	$48,862
Provenge (sipuelucel-T)	prostate cancer (cancer)	B		✓		✓	$183,012	2,123	$86,205
Lumizyme (alglucosidase alfa)	Pompe disease (genetic)	B	✓	✓		✓	$52,137	114	$457,346
Prolia (denosumab)	osteoporosis (orthopedic)	B					$664,523	239,393	$2,776
Jevtana (cabazitaxel)	prostate cancer (cancer)	S		✓		✓	$68,380	2,021	$33,835
Glassia (alpha-1-proteinase inhibitor)	alpha-1-antitrypsin deficiency (genetic)	B					$4,697	84	$55,916
Xeomin (incobotulinumtoxin A)	blepharospasm; cervical dystonia (neurologic)	B					$5,136	3,969	$1,294
Krystexxa (pegloticase)	gout (autoimmune)	B	✓	✓			$8,500	313	$27,157
Teflaro (ceftaroline)	pneumonia and skin infections (infectious)	S					$602	2,494	$242
Halaven (eribulin mesylate)	breast cancer (cancer)	S		✓		✓	$50,977	2,849	$17,893
Drugs approved in 2011									
DaTscan (ioflupane I-123 and iodine)	SPECT imaging for Parkinson's disease (diagnostic imaging)	S		✓			$14,172	6,960	$2,036

Proprietary name (nonproprietary name)	Condition (type of condition)	Drug type	Orphan	Priority review	Accelerated approval	Fast track	Expenditures, 2013 (thousands)	Utilization, 2013	Expenditures per beneficiary, 2013
Corifact (Factor XIII concentrate)	factor XIII deficiency (hematologic)	B	✓	✓	✓	✓	N/R	N/R	N/R
Benlysta (belimumab)	lupus (autoimmune)	B		✓		✓	$47,740	2,135	$22,360
Gadavist (gadobutrol)	magnetic resonance imaging of CNS vasculature (diagnostic imaging)	S					$2,511	72,394	$35
Yervoy (ipilimumab)	melanoma (cancer)	B	✓	✓		✓	$224,038	2,419	$92,616
Nulojix (belatacept)	organ rejection prophylaxis (immunologic)	B	✓			✓	$9,881	663	$14,903
Anascorp (scorpion antibody)	scorpion envenomation (other)	B	✓	✓			N/A	N/A	N/A
Adcetris (brentuximab vedotin)	Hodgkin's lymphoma (cancer)	B	✓	✓	✓	✓	$41,482	671	$61,822
Erwinaze (asparaginase)	acute lymphoblastic leukemia (cancer)	B	✓	✓		✓	N/R	N/R	N/R
Eylea (aflibercept)	age-related macular degeneration (ophthalmologic)	B		✓			$1,088,267	109,527	$9,936
Drugs approved in 2012									
Voraxaze (glucarpidase)	methotrexate toxicity (cancer)	B	✓	✓		✓	N/R	N/R	N/R
Amyvid (florbetapir F-18)	PET scanning (diagnostic imaging)	S		✓			N/A	N/A	N/A
Elelyso (taliglucerase alfa)	Type I Gaucher disease (genetic)	S	✓			✓	N/R	N/R	N/R
Perjeta (pertuzumab)	breast cancer (cancer)	B		✓			$27,711	1,029	$26,930
Kyprolis (carfilzomib)	multiple myeloma (cancer)	S	✓		✓	✓	$61,855	1,954	$31,656
Zaltrap (ziv-aflibercept)	colorectal cancer (cancer)	B		✓			$10,078	491	$20,525
Granix (tbo-filgrastim)	chemotherapy-induced neutropenia (cancer)	B					N/A	N/A	N/A

Proprietary name (nonproprietary name)	Condition (type of condition)	Drug type	Orphan	Priority review	Accelerated approval	Fast track	Expenditures, 2013 (thousands)	Utilization, 2013	Expenditures per beneficiary, 2013
Jetrea (ocriplasmin)	vitreomacular adhesion (ophthalmologic)	B		✓			$655	129	$5,076
Synribo (omacetaxine mepesuccinate)	chronic myeloid leukemia (cancer)	S	✓		✓	✓	N/R	N/R	N/R
Bivigam (immunoglobulin G)	primary immunodeficiency (immunologic)	B					N/R	N/R	N/R
Drugs approved in 2013									
Kadcyla (ado-trastuzumab emtansine)	breast cancer (cancer)	B		✓			$21,650	710	$30,493
Lymphoseek (tilmanocept)	lymphatic imaging (diagnostic imaging)	S					$981	276	$3,554
Dotarem (gadoterate meglumine)	MRI (diagnostic imaging)	S		✓			N/A	N/A	N/A
Kcentra (prothrombin, factor VII, factor IX, and factor X)	coagulation factor deficiency due to Vitamin K anticoagulation (hematologic)	B	✓				$214	58	$3,690
Xofigo (radium 223)	prostate cancer (cancer)	S		✓		✓	N/A	N/A	N/A
Rixubis (factor IX)	hemophilia B (hematologic)	B					N/A	N/A	N/A
Gazyva[a] (obinutuzumab)	chronic lymphocytic leukemia (cancer)	B	✓	✓			N/A	N/A	N/A
Tretten (factor XIII)	factor XIII subunit A deficiency (hematologic)	B	✓				N/A	N/A	N/A

Legend: B = biologic; S = synthetic drug; N/A = instances in which claims were unavailable; N/R = instances in which claims were submitted for fewer than 50 beneficiaries.

Source: GAO analysis of CMS and FDA data. I GAO-16-12

Notes: New Part B drugs were identified using the list of new molecular entities and new biologics approved by FDA from 2006 through 2013 and comparing them against CMS's Part B pricing files. Expenditures for Part B drugs approved from 2006 to 2013 exclude some vaccines as well as drugs billed using not otherwise classified drug codes.

[a]Gazyva was the only Part B drug approved from 2006 through 2013 by FDA which received breakthrough therapy designation.

Appendix II: 2013 Expenditure and Utilization Information for the 20 Highest Expenditure New Part B Drugs Approved by FDA, 2006-2013

				Utilization		Expenditures per beneficiary	
Rank	Brand name (Biologics in bold)	Condition	Total expenditures (in millions)	Number of beneficiaries	Rank	Dollars	Rank
1	**Lucentis**	Ophthalmologic	$1,369	145,325	3	$9,423	49
2	**Eylea**	Ophthalmologic	1,088	109,527	5	9,936	48
3	**Prolia**	Orthopedic	665	239,393	2	2,776	58
4	Treanda[a]	Cancer	332	15,288	8	21,685	34
5	Lexiscan	Diagnostic Imaging	257	1,198,585	1	215	69
6	**Yervoy**[a]	Cancer	224	2,419	22	92,616	10
7	**Privigen**	Immunologic	184	9,019	11	20,353	37
8	**Provenge**	Cancer	183	2,123	25	86,205	12
9	**Soliris**[a]	Hematologic	150	441	40	340,500	3
10	Dacogen[a]	Hematologic	147	4,747	15	30,981	25
11	**Actemra**	Autoimmune	130	9,759	10	13,287	44
12	**Hizentra**	Immunologic	128	2,625	20	48,862	21
13	**Nplate**[a]	Hematologic	118	3,250	18	36,344	22
14	**Cimzia**	Gastroenterological	71	5,744	14	12,335	46
15	Jevtana	Cancer	68	2,021	27	33,835	23
16	Emend	Cancer	65	60,532	7	1,080	65
17	Kyprolis[a]	Cancer	62	1,954	28	31,656	24
18	**Vectibix**	Cancer	56	2,081	26	26,744	30
19	**Lumizyme**[a]	Genetic	52	114	50	457,346	2
20	Halaven	Cancer	51	2,849	19	17,893	38

Source: GAO analysis of CMS and FDA data. I GAO-16-12

Notes: We identified new Part B drugs using the list of new molecular entities and new biologics approved by FDA from 2006 through 2013 and comparing it with CMS's Part B pricing files. We categorized drugs' approved treatment conditions into 14 categories. Drugs used in diagnostic imaging were categorized as their own condition category.

We excluded vaccines for influenza and haemophilus influenzae as well as drugs billed using not otherwise classified drug codes from our identification of these drugs.

[a]Drugs granted orphan designation by FDA

Appendix III: Percent Changes in Expenditures, Utilization, and Average Sales Price for the 20 Highest Expenditure New Part B Drugs, 2011-2013

Drug proprietary name (Biologics in bold)	Year approved	Percentage change in expenditures	Percentage change in utilization	Percentage change in expenditures per beneficiary	Percentage change in ASP
Lucentis	2006	-5	9	-13	-2
Vectibix	2006	0	-6	6	3
Dacogen[a]	2006	15	5	10	8
Lexiscan	2008	19	15	3	3
Hizentra	2010	25	20	4	1
Privigen	2007	43	29	11	3
Treanda[a]	2008	43	30	10	12
Nplate[a]	2008	48	36	9	10
Emend	2008	62	55	4	-1
Provenge	2010	64	60	3	0
Lumizyme[a]	2010	73	36	28	N/A
Jevtana	2010	100	73	15	N/A
Soliris[a]	2007	114	87	15	7
Actemra	2010	142	86	30	6
Cimzia	2008	219	145	30	26
Halaven	2010	309	225	26	N/A
Yervoy[a]	2011	330	271	16	N/A
Prolia	2010	992	1,274	-21	N/A
Eylea	2011	N/A	N/A	N/A	N/A
Kyprolis[a]	2012	N/A	N/A	N/A	N/A

Legend: N/A = instances in which data were unavailable.

Source: GAO analysis of CMS and FDA data. I GAO-16-12

Notes: We identified new Part B drugs using the list of new molecular entities and new biologics approved by FDA from 2006 through 2013 and comparing it with CMS's Part B pricing files.

We excluded vaccines for influenza and haemophilus influenzae as well as drugs billed using not otherwise classified drug codes from our identification of these drugs.

[a]Drugs granted orphan designation by FDA.

Appendix IV: GAO Contact and Staff Acknowledgments

GAO Contact

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Staff Acknowledgments

In addition to the contact named above, Will Black (Assistant Director), George Bogart, Zhi Boon, William A. Crafton, Maria Maguire, and Beth Morrison made key contributions to this report.

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