

# **“Examining FDA’s Prescription Drug User Fee Program”**

**Testimony of  
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**Before the  
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Committee on Energy and Commerce  
Subcommittee on Health**

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U.S. Department of Health and Human Services  
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[www.fda.gov](http://www.fda.gov)



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## **INTRODUCTION**

Mr. Chairman and Members of the Subcommittee, thank you for the opportunity to testify today on the sixth authorization of the Prescription Drug User Fee Act (PDUFA), also referred to as “PDUFA VI,” and the Food and Drug Administration’s (FDA or the Agency) efforts to deliver timely access to safe and effective new medications for all Americans.

The PDUFA reauthorization proposal described below was submitted to Congress in December under the previous Administration, and reflects a different approach to the Federal Budget. The Blueprint Budget supports many of the goals of the reauthorization proposal but proposes a different way of financing these goals. The Administration looks forward to working with Congress, with industry input, to develop a reauthorization proposal that speeds the development and approval of vital drugs and biologics that are safe and effective.

### **PDUFA**

The timely review of the safety and effectiveness of new drug applications (NDAs) and biologics license applications (BLAs) is central to FDA’s mission to protect and promote the public health – and PDUFA is essential to these efforts.

Before PDUFA’s enactment in 1992, Americans’ access to innovative, new medicines lagged behind other countries. FDA’s premarket review process was understaffed, unpredictable, and slow. The Agency lacked sufficient staff to perform timely reviews or develop procedures and standards to assure a more rigorous, consistent, and predictable process.

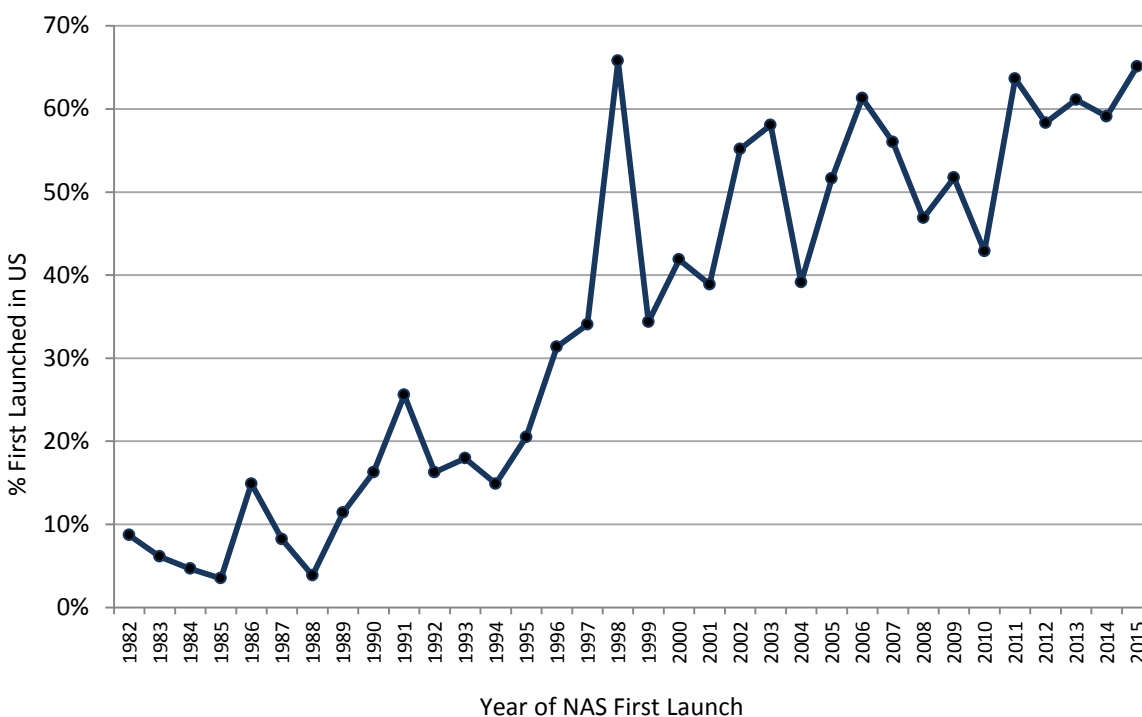
To tackle these challenges, Congress passed PDUFA, which authorized FDA to collect industry user fees to hire additional staff and upgrade its information technology systems. In return, it committed the Agency to speed the application review process for new drugs without compromising its high standards for new drug safety, efficacy, and quality.

*Speeding Americans' Access to Safe and Effective New Therapies*

PDUFA has revolutionized the United States' drug approval process. It reversed the lag in drug approvals that prompted its creation, providing Americans with more rapid access to safe and effective new drugs and biologics.

As shown in Figure 1, today, almost two-thirds of new active substances approved in the world market are launched first in the United States. To put this figure in perspective, that is more than triple the rate approved first in the United States in the first year of PDUFA.

**Figure 1. US Share of New Active Substances (NAS)  
Launched on the World Market**



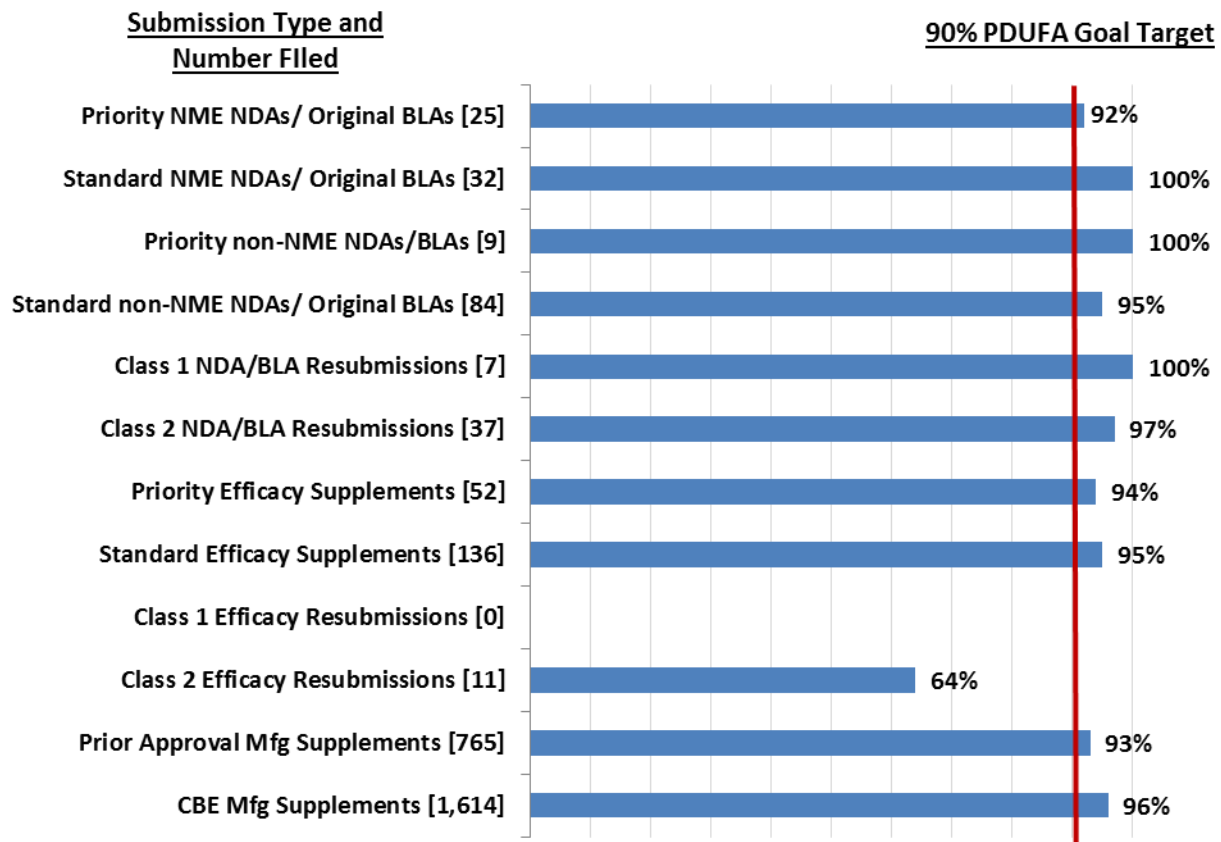
Source: *Scrip Magazine* (1982 - 2006), *Pharmaprojects/Citeline Pharma R&D Annual Review* (2007 - 2016)

The five-year reauthorization cycles for PDUFA have supported continuous program innovation, evaluation, and improvement. The enhancements to the process of human drug review originally focused on the FDA pre-market review of NDAs and BLAs. Through successive PDUFA reauthorizations, program enhancements have evolved and expanded to include extensive communication and consultation between drug sponsors and FDA throughout drug development. This has enabled better incorporation of advances in regulatory science applied to drug development and regulatory oversight. The continued modernization of drug review under PDUFA has also strengthened and enabled innovation in approaches to post-market safety. Most recently, the program has been enhanced by increasing patient focus and modernizing supporting informatics.

These enhancements have contributed to the United States becoming a global leader in drug innovation and Americans are typically the first to benefit from safe and effective new medicines. PDUFA, with its reauthorization cycles, has resulted in a scientifically and financially strong program with transparent stakeholder engagement as a routine way of doing business.

Throughout this program evolution, FDA has continued to review large volumes of sponsor submissions and deliver predictably high levels of performance against PDUFA goal commitments for timely regulatory review and development phase consultation, as shown in Figure 2, below.

**Figure 2. FDA Review Performance - FY 2015: Percent of Submissions Acted on by Goal Date<sup>1</sup>**



Data as of 9/30/2016

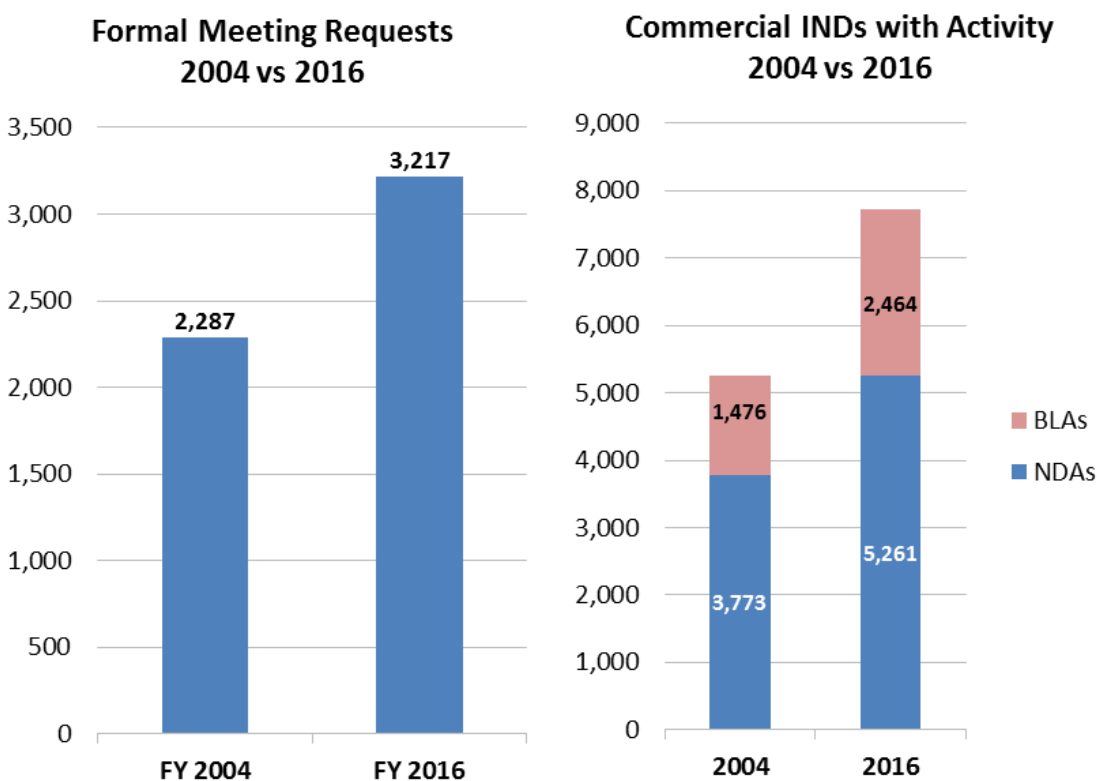
*Increasing the Timeliness and Efficiency of Premarket Review*

A key element of the success of the PDUFA program is ongoing development-phase consultation provided to drug sponsors by FDA, helping to minimize unnecessary or suboptimal development steps, and getting important new drugs to patients more rapidly and efficiently. FDA’s capacity to provide sponsors, including small first-time innovators, with timely advice enabled by PDUFA funding, has contributed to the strong drug development pipeline in the United States today. This is reflected in the increased numbers of drug development programs underway in

<sup>1</sup> NME = New Molecular Entity; NDA = New Drug Application; BLA = Biologic Licensing Application; CBE = Changes-Being-Effectuated

companies, and the corresponding growth in company requests for development phase meetings with FDA, as shown in Figure 3.

**Figure 3. FDA Commercial Investigational New Drug (INDs) with Activity and Formal Meeting Requests 2004 vs. 2016**



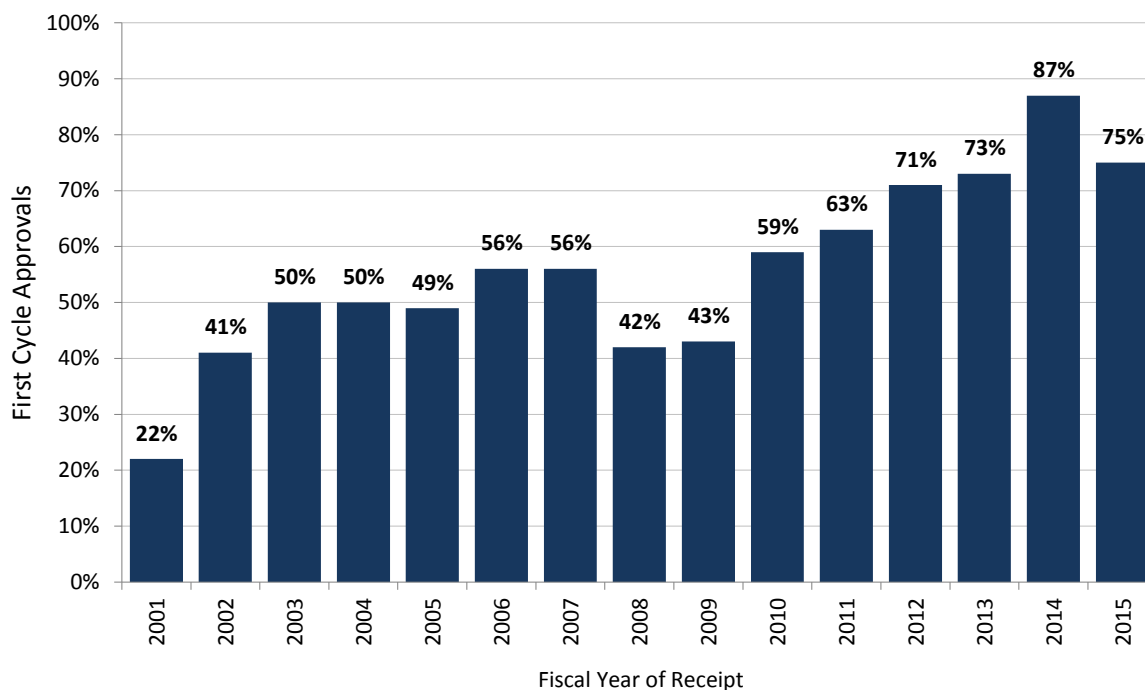
- IND Data represents a 12 month Academic period of July 1<sup>st</sup>-June 30<sup>th</sup>
- Activity is defined as any incoming submissions received within the reporting period on/after the date of the new IND application.
- Meeting Data as of 9/30/2016.
- Data includes activity for both FDA's Center for Drug Evaluation and Research and Center for Biologics Evaluation and Research.

The volume of formal meetings requested by drug sponsors has steadily grown over the course of PDUFA. Early and frequent communication between sponsors and FDA serves to improve the efficiency of drug development. Indeed, it is one of the cornerstones of the Breakthrough Therapy program. FDA-sponsor meetings help sponsors navigate key milestones during drug development, support the assembly and submission of sponsors' marketing applications, and

enable sponsors to clarify requirements for complete application submissions and potentially avoid the need for an additional review cycle.

The improvement in the quality of drug development programs and the submitted applications, supported by these PDUFA-enabled consultations between FDA and drug sponsors, is but one explanation for the observed trend toward higher first cycle approvals of applications for novel drugs (referred to as new molecular entity (NME) NDAs and BLAs), as shown in Figure 4.

**Figure 4. FDA NME NDA/BLA  
First Action Approval Rate**



- Multiple applications pertaining to single new molecular/biologic entity (e.g. single ingredient and combinations) are only counted once. Therefore, the numbers represented here for filings are not indicate of workload in the PDUFA V Program.
- Original BLAs that do not contain a new active ingredient are excluded. Percentages exclude pending applications from the denominator.
- Data includes activity for both FDA's Center for Drug Evaluation and Research and Center for Biologics Evaluation and Research.

Development-phase consultations can be particularly helpful in support of the most innovative drug programs. Of the NME NDAs and BLAs that FDA approved in calendar year 2016, over



one-third were indicated for rare diseases. In addition, over one-third (36 percent) of the drugs approved by the Center for Drug Evaluation and Research were first in their drug class and over eighty percent (86 percent) were approved first in the United States.

While a standard review is typically completed in ten months, FDA expedites review for priority drugs to be completed within six months. Priority drugs are generally targeted at severe illnesses with few or no available therapeutic options. They typically receive greater assistance from FDA reviewers throughout the development process, including providing advice in the design and implementation of the clinical trials necessary to demonstrate product safety and effectiveness.

In 2016, over 60 percent of NME NDAs and new BLAs approved by FDA benefited from one or more of FDA's expedited programs.

#### Expanded Access to Investigational Products

While the best means of providing access to useful medical treatments for all Americans is to approve drugs demonstrated to be safe and effective as quickly as possible, FDA also recognizes circumstances in which there may be value to patients and physicians in having access to products prior to marketing approval. In some cases where an individual has a serious or life-threatening disease and lacks a satisfactory therapy, that individual may believe that a promising but not yet fully evaluated treatment is his or her best choice.

FDA allows for access to investigational products through clinical trials and the Agency's Expanded Access program. Clinical trials collect the necessary clinical information needed for

FDA review and, if appropriate, approval, of investigational drugs, thereby making the drug widely available to patients. However, there are times when an individual cannot enroll in a clinical trial. In these cases, the patient may be able to gain access to an investigational therapy through the Expanded Access program.

In order for an individual patient to qualify for the Expanded Access program, several criteria must be met, including that the patient must have a serious or life-threatening disease or condition and no comparable or satisfactory alternative therapy. The patient's physician then approaches the pharmaceutical company to ask for its agreement that it will provide the drug being sought. The company has the right to approve or disapprove the physician's request. If the company agrees to the physician's request, the physician can then apply to FDA for authorization to proceed. Should they do so, they are highly likely to be allowed to proceed with the expanded access use. FDA has authorized more than 99 percent of the requests received in Fiscal Years 2010-2015. Emergency requests are usually granted immediately over the phone and non-emergencies are processed in a median of four days.

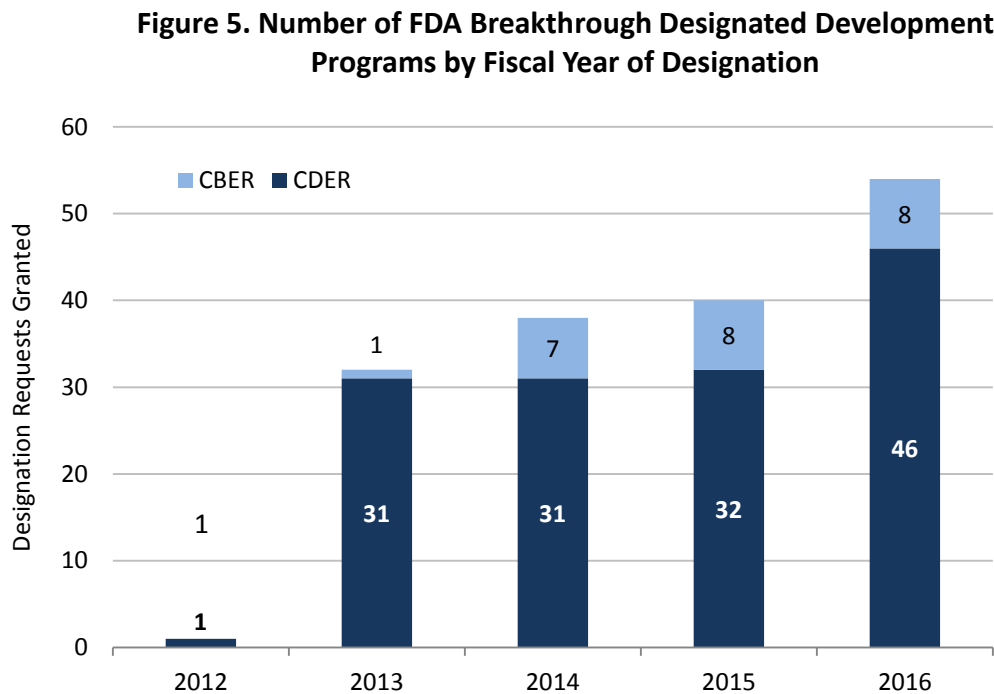
Access to investigational products requires the active cooperation of the treating physician, industry and FDA in order to be successful. In particular, the company developing the investigational product must be willing to provide it – FDA cannot force a company to manufacture a product or to make a product available. Companies might have their own reasons to turn down requests for their investigational products, including their desire to maintain their clinical development program or simply because they have not produced enough of the product.

For over 20 years, FDA has been committed to ensuring that this program works well for patients and has recently made significant improvements to its functioning and efficiency.

## Breakthrough Therapy Designation

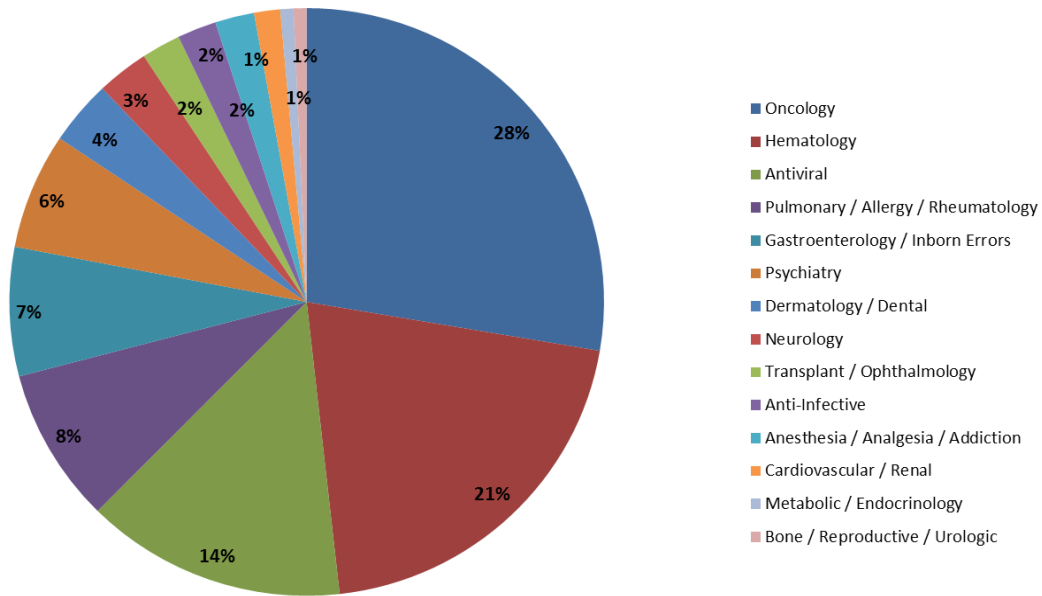
The Breakthrough Therapy (BT) program, authorized by the FDA Safety and Innovation Act (FDASIA), has further enhanced the engagement of FDA and sponsors during drug development. This program, which is for new drugs to treat serious and life-threatening diseases with unmet medical need, calls for intensive FDA-sponsor consultation during development, when preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over available therapies on one or more clinically significant endpoints.

Given the known benefits of development-phase consultation with FDA, the BT designation has been much sought after by sponsors. As of November 30, 2016, FDA had received 492 requests for BT designation and had granted 165 requests. Figure 5 shows the trend of increasing numbers of development programs.

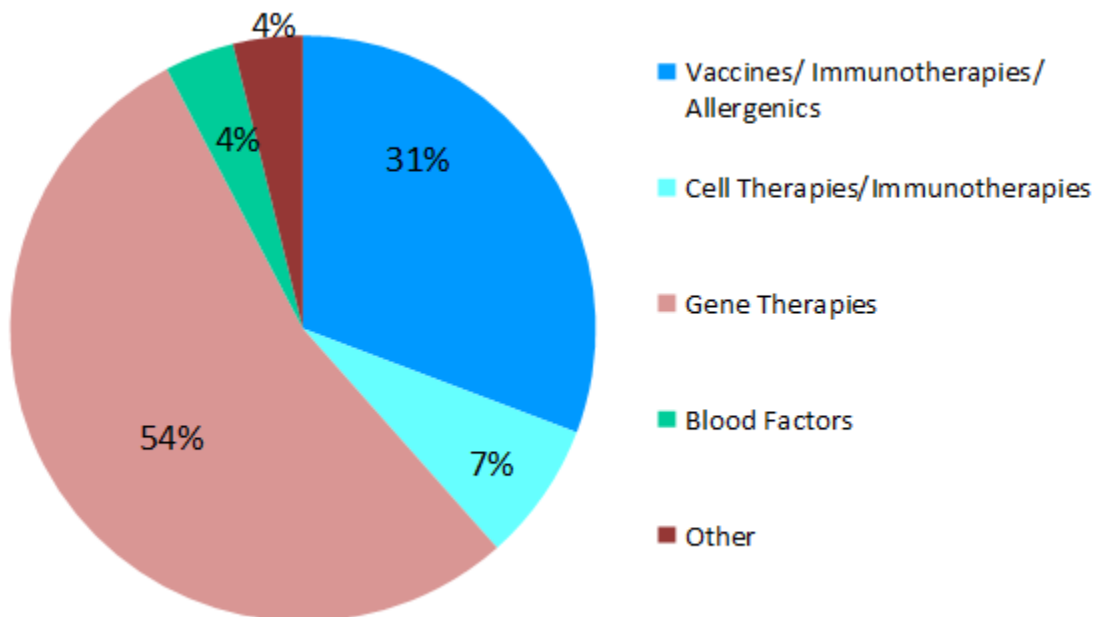


Although oncology, hematology and antiviral products account for the largest share of BT designation requests in CDER, it should be noted that BT requests and the granted designations and ongoing programs span the entire spectrum of disease areas as shown in Figure 6a, reflecting granted designations as of November 30, 2016. In CBER, most of the BT designation requests and granted designations are for gene therapies, vaccines and immunotherapies as shown in Figure 6b.

**Figure 6a. CDER Breakthrough Therapy Requests Granted by Product Type**



**Figure 6b. CBER Breakthrough Therapy Requests Granted by Product Type**



Data as of 12/1/16. Figures include total # of granted breakthrough designations for drug/indications under active IND development but have not yet received marketing approval or rescission decision.

## PDUFA V

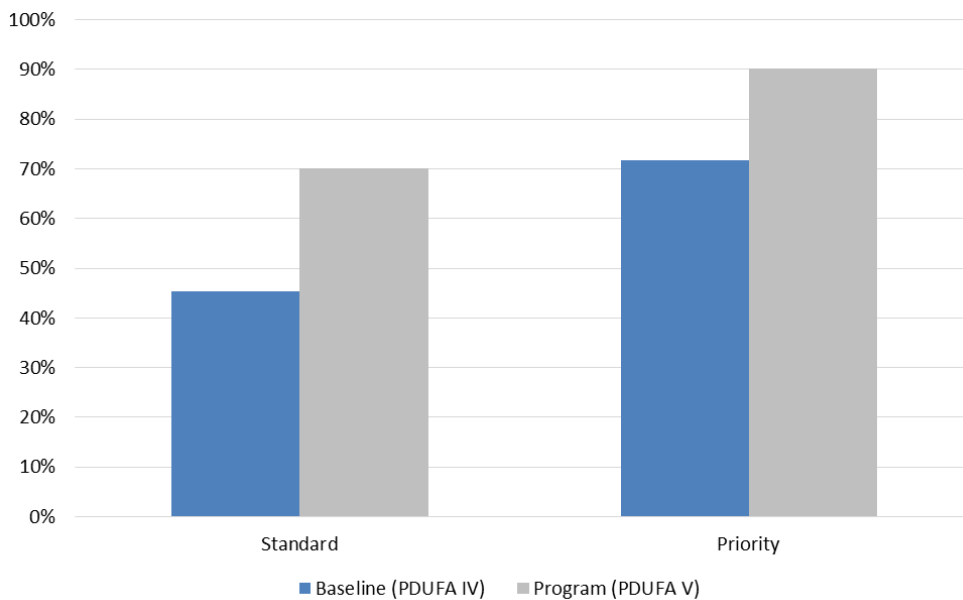
We are currently in the final year of the PDUFA V program. Over the years since the start of PDUFA I in 1992, the complexity of scientific and clinical issues in the study of new drugs has grown, including use of genetic targeting, biomarkers, novel trial designs, plans and programs to ensure effective post-market risk management. These approaches and issues were less common or nonexistent at the start of PDUFA. In addition, predictability and increased communication with FDA during drug development and application review emerged as a top priority for drug sponsors.

PDUFA V sought to achieve a better balance between the desire for increased communication with sponsors and the need to ensure adequate review time for the most complex and innovative products reviewed by FDA. This resulted in a cornerstone of the PDUFA V agreement, a new program for NME NDAs and BLA reviews that is designed to promote greater transparency and improve communication between the FDA review team and the applicant. We anticipated that the increased communications before application submissions and at key points within the first review cycle would ensure that FDA had access to all of the information that might inform and enable a first-cycle approval for those applications that could be approved, avoiding unnecessary additional cycles of review. This would enable faster access to new drugs for the patients who need them and would help reduce avoidable costs for drug sponsors.

A key measure of program success is the percentage of applications approved in a single, first review cycle. Figure 7 illustrates the success of the PDUFA V NME Program in achieving its first cycle review goals for both standard and priority reviews. The figure presents the share of

first-cycle approvals for priority and standard NDAs and BLAs filed. First cycle approvals for NME NDAs and new BLAs have been significantly higher under the new PDUFA V review program.

**Figure 7. Findings of the Final Assessment of the PDUFA V NME Review Program**



Data includes activity for both FDA’s Center for Drug Evaluation and Research and Center for Biologics Evaluation and Research.

Other PDUFA V enhancements include improved communications during drug development, strengthening the rare disease program, exploring new methods for regulatory science, and implementation of structured benefit-risk assessment. PDUFA V also provided for additional drug safety enhancements focused on standardizing the design of Risk Evaluation and Management Systems (REMS) and using the Sentinel Initiative, FDA’s active surveillance

systems for post-market safety (see PDUFA IV), to evaluate drug safety issues. This has prepared the way for expanded reliance on the data from Sentinel.

### Patient-Focused Drug Development

As part of the PDUFA V benefit-risk assessment initiative, FDA and industry recognized that patients are uniquely positioned to inform aspects of FDA's benefit-risk assessment, particularly the understanding of the disease and its severity and the adequacy of existing treatment options. Therefore, FDA committed to hold at least 20 public meetings over the five year period, with each meeting focused on obtaining direct patient input in a specific disease area. This initiative, referred to as "patient-focused drug development," has since been described as potentially transformational in advancing the role of the patient in drug development and decision-making. Although initially committing to conduct 20 meetings, FDA is on track to conduct 24 meetings each in different disease areas. The goal of the meeting is to hear from patients and their caregivers about the impact of their disease on their lives, and for FDA to hear more about what treatment benefits would be most meaningful to patients, and what treatment burdens are most important to consider. Following each meeting FDA develops a *Voice of the Patient* report to capture what was heard in the meetings (and comments from patients received in the docket); these documents serve as a valuable reference for FDA reviewers in subsequent drug reviews and related decision making.

Patient-focused drug development has provided key learnings for FDA that are being carried forward and integrated into our methods and approaches to development and decision making. We recognize that patients with chronic serious disease are experts on what it is like to live with their condition, and we have learned that they want to be as active as possible in the work to



develop and evaluate new treatments. In the past, patients’ “chief complaints” were often not factored explicitly into drug development plans (as endpoints and measures of drug benefit planned in trials), and this is an area of needed attention going forward. Although the PDUFA V patient-focused drug development initiative was intended as a pilot to elicit broader patient input, a key question for the agency was how to best build upon this pilot to advance the science and processes for effective incorporation of the patient’s voice in drug development and decision making.

In preparing for PDUFA VI reauthorization discussions, FDA has worked to build on the successes and learnings of PDUFA V and pursue new areas of opportunity for innovation in the enhancement of regulatory decision tools and new potential sources of evidence to inform drug development and review.

#### *PDUFA VI Reauthorization Process*

Congress directed the Agency to reach out to all stakeholders to solicit thoughts and insights on PDUFA reauthorization and changes to PDUFA performance goals. FDA held an initial public meeting on July 15, 2015, which included presentations by FDA and representatives of different stakeholder groups, including patient advocates, consumer groups, regulated industry, health professionals, and academic researchers. A transcript and Webcast recording are available on FDA’s website at <https://www.fda.gov/ForIndustry/UserFees/PrescriptionDrugUserFee/ucm446608.htm>.

Based on comments to a public docket, and the Agency’s own internal analyses of program challenge areas, FDA developed a set of potential proposed enhancements for PDUFA VI and

began negotiations with industry. Parallel discussions with public stakeholders were held monthly from September 2015—February 2016 to update participants on ongoing negotiations and solicit thoughts. Meeting minutes were posted on FDA’s website and are available at <https://www.fda.gov/ForIndustry/UserFees/PrescriptionDrugUserFee/ucm446608.htm>.

A final public meeting was held on August 15, 2016, to discuss the PDUFA VI agreement and engage with interested parties on proposed recommendations. A summary of the agreement and a draft of the Commitment Letter were provided a month in advance of this discussion. Final PDUFA VI recommendations were transmitted to Congress in December 2016.

### *PDUFA VI Overview*

Building on the success of previous agreements, PDUFA VI continues to support early and meaningful communication between FDA and drug sponsors to deliver safe and effective medications to Americans more quickly, and, expands on such communications by providing resources for the popular, highly successful, and resource-intensive Breakthrough Therapy program and streamlining review of products combining a drug or biologic with a device. It enhances drug development tools including biomarker qualification and provides resources to increase our understanding of how “real-world evidence” can be generated and used appropriately in regulatory decision making. The agreement also enables us to leverage the use of real-world health data by enhancing the capabilities of FDA’s Sentinel System.

Many of these core provisions are explained in greater detail below.

## *Capturing the Patient Voice in Drug Development*

Central to PDUFA VI, and its largest single investment component, are plans to elevate patient voices in developing new drugs to treat their diseases. The agreement shares the Committee's goals reflected in the 21<sup>st</sup> Century Cures Act ("Cures") – and the highest priority of our stakeholders – to leverage essential patient input and insights to fight disease.

We are building on the success of PDUFA V which established the Patient-Focused Drug Development (PFDD) program to obtain valuable patient perspectives. Areas of focus were carefully chosen based on a public process soliciting patient and stakeholder input. Under PDUFA VI, we look forward to engaging in a transparent, multi-stakeholder approach that will lead to development of the methods and approaches to ensure patients not only become active participants but informants to industry drug development and agency review. The performance commitments and matching resources to sufficiently staff this critical new work are intended to bridge from patient-focused drug development meetings to fit-for-purpose tools to collect meaningful patient input, including capturing information on the natural progression of disease.

To help identify sound and rigorous methods to capture science-based, disease-specific patient input, FDA has committed to enhance its staff capacity, hold a series of four public workshops, and develop four key guidance documents on needed methods and approaches. The Agency will also publish on its website a repository of publicly available tools as a resource for stakeholders and ongoing efforts.

We are gratified by the enthusiastic response within the patient community to PFDD, and look forward to working with the broader community to advance the science of patient input – and deliver new and better treatment options.

*Building a Solid Foundation for Breakthrough Therapies*

The Breakthrough Therapy program, authorized by FDASIA, has become one of the most popular components of the human drug review program with requests and designations far exceeding expectations.

The increase in promising new drugs to treat serious and life-threatening diseases with unmet medical need is, of course, a very good thing for both patients and public health. But the growth of the BT program has strained limited available review staff resources. A hallmark of the BT program is intensive Agency interaction with sponsors during the development process on complex products with transformative potential. This “all hands on deck” approach provides a sponsor of a designated breakthrough product with guidance from the Agency on efficient drug development beginning as early as the Phase I period, an organizational commitment to involve senior managers, and eligibility for rolling review. Many of the BT designations granted so far have been for rare disease indications.

The PDUFA VI agreement provides dedicated funding to ensure the sustained success of the BT program. Additional resources will enable FDA to increase review staff and to supplement resources for clinical pharmacology, statistics, and product quality. This renewed commitment will enable the Agency to continue to work closely with sponsors to ensure expedited development and review of breakthrough therapies for serious or life-threatening diseases.

### Advancing Biomarker Development

FDA and industry share the goals of Cures to accelerate development of reliable biomarkers to advance important new therapies. Biomarkers are currently used throughout the drug development process, including as surrogate endpoints to support earlier evidence for regulatory decision-making when evidence from a clinical endpoint could take much longer or require many more patients to be studied.

FDA commonly uses surrogate endpoints in accelerated approvals where confirmatory evidence is required to verify the expected clinical benefit after marketing begins. Surrogate endpoints have been the basis for 60 percent of rare-disease approvals. Once a surrogate endpoint is well established to predict clinical benefit, surrogate endpoints can be used to support traditional approvals as well. For example, FDA regularly relies on a surrogate endpoint for approval of new therapies for diabetes (the HbA1C test, a measurement of hemoglobin with attached sugar in the blood that reflects the extent and persistence of elevated blood sugar) greatly expanding patient treatment options.

The PDUFA VI proposed enhancements include some of the same activities specified in Cures. PDUFA VI addressed the opportunity for application of biomarkers in two different areas, one involving proprietary use of a biomarker as a surrogate endpoint in a specific drug development program, and the other involving the more public process of biomarker qualification as a drug development tool.

FDA recognizes that early consultation can be important to an efficient development program when a sponsor intends to use a biomarker as a new surrogate endpoint that has never been used as the primary basis for product approval in the proposed context of use. The PDUFA VI

commitments therefore provide for early consultation with the sponsor to enable the FDA review team to consult with senior management to evaluate the sponsor's proposal before providing advice to the sponsor on a critical aspect of their development program. This will enable FDA to discuss the feasibility of the surrogate as a primary endpoint, any knowledge gaps, and how these gaps should be addressed before the surrogate endpoint could be used as the primary basis for approval.

PDUFA VI also provides enhancements for the more public drug development tool qualification pathway for biomarkers. The biomarker qualification program was established to support FDA's work with external partners to develop biomarkers that aid in the drug development process. To facilitate the enhancement of the drug development tools qualification pathway for biomarkers in PDUFA VI, FDA proposes to convene a public meeting to discuss taxonomy and a framework with standards for biomarkers used in drug development, to develop guidance on biomarker taxonomy, contexts of uses, and general evidentiary standards for biomarker qualification, and to maintain a public website to communicate a list of biomarker qualification submissions in the qualification process.

Meaningful progress in developing additional biomarkers for public qualification requires collaboration among a wide range of stakeholders. FDA will continue to work with the National Institutes of Health, the Biomarkers Consortium, the Critical Path Institute and others to advance biomarker development.

### Streamlining Combination Product Review

More streamlined oversight of combination products is another FDA and industry priority reflected in PDUFA VI. Under the proposed enhancements FDA will pursue improvements in inter-center and intra-center combination product review coordination and transparency for PDUFA products that are combination products regulated by CDER and CBER (PDUFA combination products). FDA proposes to enhance staff capacity and capability across the relevant medical product centers and the Office of Combination Products to more efficiently, effectively, and consistently review combination products. FDA also proposes to streamline the process for combination product review and to improve the Agency's ability to track combination product review workload, including a third party assessment of current practices for combination drug product review.

Our goal, consistent with Cures, is to enhance the overall efficiency, consistency, and predictability of combination product review without imposing new administrative burdens.

Under PDUFA VI enhancements FDA will also establish new performance goals and submission procedures for the review of human factors protocols for PDUFA combination products. These goals will be to provide the sponsor with written comments on these protocols within 60 days of receipt. The goals to provide written comments within 60 days will begin at the 50 percent level in FY 2019, and increase to 90 percent by FY 2021.

### Advancing the Use of Complex Innovative Trial Designs and Model Informed Drug Development

FDA routinely works closely with industry to facilitate innovative approaches to drug development that maintain our high standards for drug safety and efficacy. PDUFA VI promises

to encourage future efforts by advancing Model-Informed Drug Development (MIDD) and the use of complex innovative and adaptive clinical trial designs.

The development and application of exposure-based, biological, and statistical models derived from preclinical and clinical data sources can be used to inform regulatory decision making, for example, in determining patient selection in clinical trials, individualized dosing for specific populations, or the need for post-marketing studies. To facilitate the development and application of these approaches during PDUFA VI, FDA proposes to convene a series of workshops to identify best practices for MIDD, to conduct a pilot program, to develop guidance on MIDD, and to update policies and procedures, as appropriate, to incorporate guidelines for the evaluation of MIDD approaches.

To facilitate the advancement and use of complex adaptive, Bayesian, and other novel clinical trial designs during PDUFA VI, FDA proposes to convene a public workshop on complex innovative trial designs, publish guidance on complex innovative trial designs, to conduct a pilot program, and to update policies and procedures as appropriate to incorporate guidelines on evaluating complex innovative trial designs.

#### *Utilizing Real-World Observational Data*

It has been said that medical care and biomedical research are in the midst of a data revolution, and networked systems, electronic health records, electronic insurance claims databases, social media, patient registries, and other new sources may comprise an immense new set of sources for data about health and healthcare. In addition, these “real-world” sources can provide data about patients in the setting of their environments—whether at home or at work—and in the social context of their lives. There is little doubt that the new sources of data now becoming



increasingly available to researchers, clinicians, and patients hold enormous potential for improving the quality, safety, and efficiency of medical care. More work is needed to understand both the promise and pitfalls of far-reaching technological changes, including the multiple dimensions of quality and fitness for purpose for appropriate use of such data in regulatory decision making.

FDA recognizes the potential value of utilizing "real-world" evidence in evaluating not only the safety of medications but also their effectiveness. To better understand how real-world evidence can be generated and used appropriately in product evaluation, FDA proposes to conduct one or more public workshops, as well as other appropriate activities (e.g. pilot studies or methodology development projects). Considering the available input, FDA will then publish draft guidance on how real-world evidence can contribute to the assessment of safety and effectiveness in regulatory submissions.

Under PDUFA VI, FDA also proposes to pursue a more well-established use of real-world evidence to support post market drug safety surveillance utilizing Sentinel. FDA's Sentinel Initiative is a long-term program designed to build and implement a national electronic system for monitoring the safety of FDA-approved medical products. FDA recently transitioned from the Mini-Sentinel pilot to the Sentinel System, but full utilization of the Sentinel System remains a work in progress. Continued development and integration of the Sentinel System is needed to realize the system's full value to the postmarketing safety review process.

To help realize the full value of the Sentinel System during PDUFA VI, FDA proposes to continue to expand the systems' data sources and core capabilities, to systematically integrate Sentinel into postmarketing review activities, to enhance communication practices with sponsors

and the public regarding general methodologies for Sentinel queries, and to conduct an analysis of the impact of Sentinel expansion and integration for regulatory purposes.

#### *Hiring and Retaining Highly Qualified Experts*

To speed and improve development of safe and effective new therapies for patients requires that FDA hire and retain sufficient numbers and types of technical and scientific experts to efficiently conduct reviews of human drug applications. In order to strengthen this core function during PDUFA VI, FDA proposes to commit to completing implementation of: a modernized position management system, corporate recruiting practices, augmenting capacity with contractor support, a dedicated scientific recruiting function, metric goals for human drug review staff hiring, and a comprehensive independent assessment of hiring and retention system performance. We want to thank you again for providing vital new hiring authority in Cures. Cures will greatly improve FDA's ability to hire and retain scientific experts in more complex and specialized areas and meet our growing responsibilities.

The hiring commitments proposed in PDUFA VI will complement Cures by supplementing the expertise and resources the Agency needs to perform its vital prescription drug mission.

#### *Enhancing Management of User Fee Resources*

FDA is committed to enhancing management of PDUFA resources and ensuring PDUFA user fee resources are administered, allocated, and reported in an efficient and transparent manner. In PDUFA VI, the Agency proposes to establish a resource capacity planning function to improve its ability to analyze current resource needs and project future resource needs, modernize its time reporting approach, conduct an evaluation of PDUFA program resource management, and publish a five-year PDUFA financial plan with annual updates.

In addition, under PDUFA VI, FDA proposes to enhance the program fee structure and related mechanisms, to achieve increased predictability, stability, and efficiency. The current overall PDUFA fee structure and the fee setting process were established in 1992. Both FDA and industry recognize that updating some elements of the fee structure and the fee setting process will enhance administrative efficiency and the predictability and stability of fee amounts and revenues and improve FDA's ability to engage in long-term financial planning. FDA proposes to shift a greater proportion of the target revenue allocation to more predictable fee-paying types (20 percent to applications; 80 percent to Program fees), and make other modifications to improve efficiency and stability including discontinuation of the establishment and supplement fees, modifying the annual fee billing date to minimize the need for multiple billing cycles, and other technical changes.

## **CONCLUSION**

PDUFA has revolutionized the drug review process in the United States, allowing FDA to speed the application review process without compromising the Agency's high standards. User fees offer a strong example of what can be achieved when FDA, industry, and other stakeholders work together towards the same goal. User fees provide a critical way to ensure that FDA has the resources needed to conduct reviews in a timely fashion. While we have made demonstrable progress in partnering to bring drug and biologics to market in as timely a manner as possible, we know that more work remains to be done to further enhance and optimize our processes. The reauthorization of PDUFA will allow FDA to build upon the program's demonstrated success, and in so doing, further benefit patients and affirm our nation's standing as a global leader in biomedical innovation.