Testimony of Mr. Geno Germano
President and General Manager
Specialty Care and Oncology
Pfizer Inc.
Before the House Energy & Commerce Subcommittee on Health
Reauthorization of the Prescription Drug User Fee Act (PDUFA)
February 1, 2012

Chairman Pitts, Chairman Upton, Ranking Member Pallone, Ranking Member Waxman and members of the Subcommittee, my name is Geno Germano. I am President and General Manager of the Specialty Care and Oncology businesses at Pfizer. Founded in 1849 in New York City, we have grown to become the world's largest biopharmaceutical company, providing treatments for myriad diseases that afflict people around the world.

I appreciate the opportunity to testify on behalf of Pfizer and our 40,000 U.S. based colleagues, to unequivocally support the reauthorization of the Prescription Drug User Fee Act (PDUFA).

Behind the acronym PDUFA is another acronym: R&D. Research and Development. R&D is the lifeblood of Pfizer. It is the lifeblood of our industry. And it is the lifeblood of great American innovation.

Today, it takes on average more than $1 billion and 12-15 years to research and develop a new medicine. Approximately 1-in-10,000 compounds that enter the drug discovery phase is ever approved by the Food and Drug Administration (FDA) and made available to patients.

Our R&D is ultimately codified in our patents. Patents represent our license to move forward and are a fundamental legal basis for our existence.

It is important to remember that we file our patents on compounds in the very early stages of development, often a decade or more before the review process begins at the FDA. Therefore, by the time we have submitted an application to the FDA, the patent life has already eroded to a meaningful extent, making an effective and efficient process with the FDA imperative for the firm investing in this innovation.

Biopharmaceutical companies like Pfizer typically have at most between 11-14 years to recoup our investment in a new compound before generic competition enters the market; however, the public health value of our investment continues for generations to come.

It is through this foundational work in R&D and manufacturing that the biopharmaceutical industry supports more than three million U.S. jobs, nearly $300 billion in total output to GDP. PDUFA will help keep R&D and new medicine introductions in the U.S.

The odds, financial commitment, and significant time and resources reflect the uncertainties inherent in our business. The scientific uncertainties are ultimately reduced to the core question:

Does the benefit of the drug outweigh the risk?

This is a question we and the FDA seek to answer and it will vary depending on the treatment and the intended patient population. Regulatory uncertainties can complicate this dynamic if the review process at the FDA is ambiguous and inefficient. This is why a strong partnership and communication with the FDA are essential.

R&D at Pfizer

Before discussing the provisions of PDUFA, it's important to provide additional background on the R&D process at Pfizer. My business is focused on developing and providing therapies of a specialized nature. This means treatments for rare diseases, for many forms of cancer, and vaccines that help prevent people from getting infectious diseases like pneumonia or meningitis.

As the head of a business, I am intimately engaged in the development of our medicines pipeline. The business and R&D share the goal of investing in the right therapeutic areas. That means making sure the compounds progressing have a reasonable chance of making it through the entire development process and gaining efficient and successful review by the FDA. We also look beyond that to ensure that payers recognize the value of the product.

One of our main focuses at Pfizer is to always improve the performance of our innovative core – the nexus where strong R&D leads to valuable products. Over the past year, there has been a steady cadence of progress in our late stage pipeline that includes positive clinical data presentations, submission of marketing applications, regulatory approvals, and new product launches, as well as the emergence of a promising mix of early to mid-stage assets.

We have a number of products that we're very excited about.

First is Prevnar 13, a vaccine for the prevention of pneumococcal disease which is approved for use in children 6 weeks through 5 years old, and in December of last year, received approval for adults 50 years of age and older. This new approval is very important given that the most common manifestation of the disease in adults is pneumococcal pneumonia, which occurs in about 440,000 Americans 50 and older every year, accounting for about 300,000 hospitalizations and significant related personal and societal costs.

FDA approved Prevnar 13 under the agency's accelerated approval pathway, which allows for earlier approval of certain drug products to treat serious or life-threatening disease which may not be adequately addressed by existing drug products. The approval of the vaccine was based on its effectiveness in relation to a surrogate endpoint that is likely to predict clinical benefit and was granted on the condition that a confirmatory clinical trial be conducted to verify the anticipated clinical benefit. While that confirmatory study is currently underway, today Americans 50 and older have access to an important new option for the prevention of a potentially life-threatening disease.

Second is Xalkori, a New Molecular Entity (NME), which is the first lung cancer drug approved by the FDA in more than six years. This scientific innovation is also one of the first personalized medicines, targeting a genetic abnormality shared by 3% to 5% of the 200,000 lung-cancer patients diagnosed in the U.S. each year.

Xalkori, which was approved last August, was a fast track product and was given priority review by the FDA. The goal for priority review is 6 months - FDA approved it in 4 months. Xalkori and other NME's are the highest priorities for Pfizer and FDA because as new treatments they target unmet medical needs. And while Xalkori's approval is an example of getting it right, the challenge we have is making sure that situations like Xalkori are the rule, not the exception.

Third, we recently received European approval for Vyndaqel (Tafamidis) to treat TTR-FAP, a rare and irreversible, progressive neurodegenerative disease that affects approximately 8,000 patients worldwide. Patients experience debilitating symptoms that usually prove fatal within 10 years, and until now there has been no treatment option other than liver transplant. This product has been submitted to the FDA with a decision expected later this year.

We are also pleased with the results we have seen with tofacitinib in the phase 3 rheumatoid arthritis program and have submitted applications for approval to regulators in both the U.S. and Europe. This represents just some of the near-term opportunities in our growing product pipeline. And while I'm encouraged, I know we have much more work to do.

In 2011, we made a strategic decision to narrow our therapeutic areas of focus, took steps to identify failures earlier in the development cycle, advanced the most promising compounds within our pipeline, and continued to invest in our R&D network and the capabilities needed to drive biomedical innovation.

We are allocating the majority of our R&D efforts to the areas that represent the intersection between unmet medical needs, our strength in biology and chemistry, and the willingness and ability of patients and payors to value our innovation. We are focusing on the areas where we believe we have the right elements for success -- Neuroscience, Cardiovascular, Oncology, Inflammation and Immunology, Vaccines, Pain and Sensory Disorders and Biosimilars.

In addition, in 2011, we established an enhanced focus on rare diseases; an area in which Pfizer has a strong legacy with more than 17 approved orphan indications. Rare diseases are among the most serious of all illnesses and impact greater than 50 million patients in the U.S. and the EU, yet fewer than 5% of the estimated 7,000 rare diseases have approved treatments. We believe that patients suffering from a rare disease deserve equal access to an approved treatment so we are actively expanding development in this space for conditions like sickle cell anemia, hemophilia, cystic fibrosis and muscular dystrophy.

We are prioritizing the R&D portfolio. We are focusing our internal capabilities in the areas where we offer unique value such as clinical trial design and overseeing the end-to-end strategy for our clinical assets. We have turned to external partners to manage the areas that don't drive competitive value for us such as clinical trial implementation. This makes it easier to scale activity up or down based on the needs of the portfolio.

We have made tough but necessary capital allocation decisions regarding our global R&D site network. We are laying the foundation for a new MIT-sponsored research site in Cambridge, MA, that will focus on cardiovascular, metabolic and neuroscience research. By locating in science and technology hubs we have better access to a highly skilled talent base that will enrich our capabilities in the biologic sciences and increase our opportunities for external partnerships.

We have invested in new technologies within our focus areas.

For example, in Oncology we are investing in Antibody-Drug Conjugates (ADC) which combine the best features of two proven cancer therapies – antibodies and cytotoxic drugs.

Through our Centers for Therapeutic Innovation we are partnering with 19 leading academic medical institutions located in Boston, New York, San Francisco and San Diego to tap into the research expertise of academics in diseases, targets and patient populations to help bridge the gap between early scientific discovery and the translation into new medicines.

We are adopting a Precision Medicine approach to research, integrating clinical and molecular information to understand the biological basis of disease. This leads to better selection of disease targets and the identification of patient populations that demonstrate better clinical outcomes.

We expect that in 5 years most of our Phase 3 clinical trial starts will reflect a Precision Medicine R&D approach.

Supporting all our actions is a more rigorous governance model across all of R&D and the Business Units that has clear metrics and a process for establishing that a compound will meet a clear medical need, be valued by payers and patients and have a strong rationale that the product would be approved by regulators prior to starting a proof of concept study. We revalidate the value needs and regulatory rationale at every step of the development path with increased rigor prior to starting our final pivotal Phase 3 trials needed for regulatory submissions and for an approval decision point.

I believe that through all the actions we are taking there is a greater sense of urgency, accountability and results focused across R&D.

All of the work that I described above means nothing without an efficient, well funded FDA that is able to keep pace with the evolving science needed to review drug applications efficiently and effectively.

Why PDUFA Matters to Pfizer

So why does PDUFA matter to Pfizer?

As I mentioned, to develop a single new medicine for patients, Pfizer will invest more than $1 billion and more than ten years identifying new molecules, establishing tolerability, and confirming safety and effectiveness in large numbers of patients. PDUFA is part of the gateway between such R&D and the many patients needing these medicines. The performance metrics and process requirements within PDUFA help ensure that the FDA is efficient, transparent and predictable.

Each of the PDUFA three main focus areas helps enable Pfizer and FDA to improve transparency in the drug approval process. Here is how.

1. Enhancements to the Drug Review Process

New Molecular Entity (NME) Review Program: PDUFA V will increase predictability, transparency, and scientific communication during FDA's review of NME's (drugs containing no active molecules previously approved by the FDA in any other application) for new applications.

Under PDUFA V, the NME review program will help identify and resolve issues earlier in the review process and thereby shorten the time to a review decision – potentially providing patients with earlier access to needed treatment.

Communication during drug development: Interaction between the FDA and drug sponsors during drug development is critical, and this program will help sponsors and the FDA engage more efficiently and productively.

At times, such interactions require a formal meeting; at other times, a response can be provided without a formal meeting. However, obtaining FDA responses to questions outside the formal meeting process has been challenging due to FDA staff workload and competing demands.

The PDUFA V performance goals propose funding an agency communication and training staff that will focus on improving communication between the FDA and drug sponsors during development.

Benefit/risk assessment: When safety issues arise, the confidence of patients and the public may be shaken. Benefit/Risk assessment measures a drug's benefits and risks and then assesses whether the balance of these factors is favorable. This analysis is critical to ensure patient confidence in their medicine.

PDUFA V would facilitate continued development and implementation of a structured benefit-risk framework in the drug evaluation process to increase transparency and objectivity.

E-submission and data standards: Pfizer alone sends about 10,000 submissions a year to the FDA and has been a leader as the industry moves toward digitization, which allows FDA reviewers to have an entire product submission in electronic form. This new proposal will require that all applications be submitted to the FDA in standardized electronic format – bringing still greater efficiency and predictability to the review process. Additionally, the FDA would begin a public process to standardize clinical data terminology for certain therapeutic indications.

2. Modernizing Regulatory Science

Advances in regulatory science will help make the evaluation and approval process more efficient, helping deliver safe and effective new products to patients faster and strengthening the ability to monitor product use and improve performance, thus enhancing patient outcomes.

PDUFA V's enhancements will affect Pfizer in each of the regulatory science provisions:

Meta-analysis: Meta-analysis is the technique of pooling data from different clinical trials on a particular drug. FDA needs the ability to review and respond to meta-analyses, conducted by third parties to determine if they were in fact conducted appropriately. There a number of these analyses conducted on Pfizer products and it is crucial that we and the FDA have an accurate way to interpret the results of them to better inform patients.

PDUFA V addresses this issue by requiring FDA to develop a scientific method to determine how to best use the information from a meta-analysis. Doing so will help give the FDA the tools it needs to provide appropriate guidance on the results of meta-analysis.

Biomarkers & pharmacogenomics: A biomarker is anything that can be measured as an indicator of biological activity, such as a blood pressure count or DNA sequence. Pharmacogenomics is the study of how genes affect a person's response to drugs. Biomarkers and Pharmacogenomics can be predictors of many things, such as the natural course of a tumor, and enable doctors to decide which patients are likely to respond to a given drug and at what dose.

PDUFA V will enhance FDA's ability to address the increasing workload of applications that involve biomarkers and thereby allow innovative new treatments to get to patients sooner.

Patient-reported outcome tools: Many quality-of-life problems go unnoticed as patients don't always tell their doctors how they are feeling. The only real way to get the patient's perspective is to ask the patient directly. However, FDA staff is already overloaded and timely patient-reported outcome reviews are difficult to ensure – sometimes taking months.

PDUFA V would increase FDA's review capacity, ensuring that agency reviewers have access to every tool needed to support claims within a specific drug context.

Rare disease drug development: Pfizer has a strong legacy in rare diseases, with more than 17 approved orphan indications, and is actively expanding its activity in the space.

PDUFA V will continue to ensure that regulatory evaluations of orphan drugs are conducted flexibly to take account of the specific issues facing that particular rare disease. The small number of patients suffering from a given rare disease often makes it difficult to enroll a sufficient number of patients in a clinical trial. The rarity of the condition results in smaller set of data on efficacy and safety compared to more common diseases, and often the evidence will include case histories, registry data and studies from distant countries.

But the standards for safety and efficacy should not be lower, so regulatory agencies need to have evidence and flexible approaches to assessment that take into account the rarity of the condition, the degree of fundamental understanding of the natural history of the disease, the limitations in identification and diagnosis of patients, and the urgency of patient need.

PDUFA V will enhance development of new drugs for rare diseases though FDA policy development and training of review staff on scientific issues unique to rare diseases, and will support outreach to industry, patients, and the scientific investigator communities.

3. Strengthening Post-Market Safety Surveillance

As part of its mission to protect and promote the public health, the FDA has always kept a keen focus on the safety of drugs and other medical products. Once a drug is approved and reaches the marketplace, FDA and drug sponsors maintain a system of post-marketing surveillance and risk assessment programs to identify adverse events that may not have been detected during the drug approval process.

Under PDUFA V, two safety enhancements are being proposed, both of which would affect Pfizer.

The Sentinel Initiative: FDA's Sentinel Initiative would access national electronic data systems to actively monitor medical product safety in real time – a development Pfizer supports as a powerful public health resource that may greatly improve drug safety reporting. FDA's phased approach to Sentinel implementation, emphasizing success over speed, is appropriate.

As Sentinel advances, FDA must ensure it has the financial resources and the IT infrastructure to build a distributed system on such a large scale. Also, Sentinel governance and operating frameworks must ensure timely and effective company access to product safety data and to signal detection and analysis methods – both of which will be critical to maintaining company involvement over product safety and risk management. It is extremely important that Sentinel not be used as a method for comparative effectiveness.

Standardizing risk evaluation and mitigation strategies (REMS): FDA has the authority to require REMS from manufacturers to ensure that the benefits of a drug or biological product outweigh its risks over the longer-term. However, industry has expressed concerns regarding the ability of REMS programs to mitigate risk and the burden REMS places on providers, patients, pharmacists, and the healthcare delivery system.

It is vital that FDA consider REMS' impact on their ability to meet PDUFA review performance metrics and expedite patients' access to safe and effective new medicines. Pfizer supports PDUFA V's goal of improving REMS approval times by providing better clarity during development meetings and holding public meetings on how to reduce the burden of implementing REMS.

Fees

The PDFUA V agreement includes an additional 129 full time equivalents (FTE) to support additional activities at FDA. The additional FTE is dedicated to support the provisions on modernizing regulatory science. We believe the current level of FTE dedicated to the review of applications and to perform post marketing safety activities can be accomplished with existing resources.

User fees to fund drug review activities at FDA are approaching 70% of the total budget. While Pfizer understands the current economic situation, this level of support nonetheless concerns us. It is important Congress and the Administration devote additional government resources to supplement the fees paid by our industry.

Reauthorization of the Best Pharmaceuticals for Children Act (BPCA) and the Pediatric Research and Equity Act (PREA)

Pfizer strongly supports the reauthorization of BPCA and PREA. They work together by providing biopharmaceutical companies like Pfizer an incentive, six months of additional exclusivity, to study our products in the pediatric populations and ensuring that drugs are appropriately studied and labeled for pediatric populations. While BPCA and PREA are working, Pfizer supports making them both permanent in order to provide us with the certainty that is important in the R&D and regulatory process.

Supply Chain Safety

Pfizer has comprehensive systems and processes in place to manage our global supply chain, starting with the raw materials we use to manufacture our products and ending when consumers receive our products. Our processes help us to prevent, detect, and respond to threats so that we protect the quality and safety of our products and ensure that our patients are receiving the life-saving and sustaining drugs they expect.

Pfizer recognizes that the Committee may be considering supply chain security provisions for inclusion in the PDUFA legislation. A number of proposals addressing upstream and downstream drug supply safety as well as a national track and trace system are being discussed. As we have put in place strong measures to ensure the integrity of our materials and the quality of our suppliers and distributors, we look forward to working with members of Congress who have put forward proposals to address these issues.

Conclusion

PDUFA represents the best kind of collaborative leadership: where government and business come together with rigor and excellence to ensure that patients have timely access to the critical medicines they and their families deserve. The ability of Pfizer to do its job depends on the ability of FDA to do its job, and PDUFA provides a framework and resources for that to happen. PDUFA is must pass legislation. It is must pass for Pfizer and the biopharmaceutical industry. Must pass for FDA. And most importantly it is must pass for patients and society as a whole.

Again, thank you for the opportunity to testify. I look forward to answering any questions you may have and hearing your views.