



Comments of the National Consumers League

**Docket No. FDA-2016-N-1895
Prescription Drug User Fee Act; Public Meeting
August 15, 2016**

Introduction

The National Consumers League (NCL) appreciates the opportunity to comment on the reauthorization of the Prescription Drug User Fee Act for fiscal years 2018 through 2022 (PDUFA VI). Founded in 1899, the National Consumers League has long been concerned with ensuring the safety of foods and drugs. Among NCL's top priorities are ensuring the safety, effectiveness, and appropriate use of both prescription and over-the-counter (OTC) drugs, and medication adherence, which we have helped to advance through our *Script Your Future* Campaign.

The Need for FDA to Remain Independent and Not Compromise its High Standards for Safety, Efficacy, and Quality of Prescription Drug Products

PDUFA resources have enabled the FDA to reduce the median approval time for new molecular entities (NMEs) from more than 30 months prior to the passage of PDUFA to less than 10 months today. PDUFA VI would maintain the current 8 months (priority) and 12 months (standard) FDA review performance goals for new drug applications (NDAs) and biologics license applications (BLAs). PDUFA VI also lays out an ambitious and admirable agenda for increased FDA attention and focus, demonstrating how the expansion of FDA's drug approval work is made possible by the PDUFA user fee program. That said, as PDUFA VI goes through the reauthorization process, NCL urges the FDA to remain mindful of the concerns expressed by some that because industry pays user fees, industry thereby controls the FDA's agenda and process. It is critical for the agency to act independently of industry influence and to uphold its high standards for safety, efficacy, and quality of prescription drug products.

NCL wants to be sure that in the quest to reduce barriers to new drug approvals, FDA does not lose sight of the importance of the agency's mission of protecting and promoting the health of consumers and patients. The FDA must balance the needs of consumers who are concerned about serious side effects with the concerns of patients who may be facing a life-threatening illness where time is of the essence. However, even patients in great need may be harmed rather than helped by drugs that have been

approved too quickly without adequate consideration of safety and effectiveness or toxic side effects.

More Attention Should be Given in PDUFA VI to Ensuring Drug Safety

While it is important to have an efficient and timely approval process, NCL believes that there is still too little emphasis in PDUFA VI on performance goals aimed at improving the safety and efficacy of drugs. Of the 46 pages of the PDUFA VI User Fee Agreement, only two are devoted to the enhancement and modernization of the FDA drug safety system.

We note that PDUFA VI does not call for an assessment of how well the agency's data systems and processes support the review, oversight, and communication of postmarketing safety issues until the end of FY 2022. NCL urges the agency to speed up the timeline for this important assessment.

Enhancing Competition

While NCL believes that drug makers should receive a fair return on their research and development investment, we also believe that drugs should be fairly priced. Access to medications is a key issue for consumers. To improve competition, FDA should prioritize the review of those drug and biologic products for which no competition exists in the marketplace. In addition, while not the subject of this proceeding on PDUFA VI, we believe what happens through PDUFA is connected to patient access to generic versions of approved drugs. In that vein, we want to stress that it is critical for the FDA to speed the approval of generic drugs. Currently, the FDA faces a backlog of nearly 4,000 generic drug applications. The FDA should be provided with the necessary resources to address this backlog and thus expand access to affordable treatments for patients.

In addition, the FDA should examine its Rare Disease Program to ensure that it is not being misused, especially in the case of older drugs that are being reclassified as orphan drugs. According to a report by the market research firm Evaluate, in 2014, the average annual cost per patient for the 100 top-selling orphan drugs in the United States was nearly \$112,000, compared with \$23,000 for non-orphans. NCL is concerned about the impact of these high orphan drug prices on patient access to healthcare and the medications they need to treat their rare diseases and conditions.

Positive Features of PDUFA VI

In reviewing the proposed PDUFA VI User Fee Agreement, NCL notes that it has many good features, including the following:

Enhanced Communication and Meetings between the FDA and Product Sponsors

NCL supports PDUFA VI's emphasis on improving communication between FDA and product sponsors, with the goal of promoting the efficiency and effectiveness of the first cycle review process and minimizing the number of review cycles necessary for approval. As FDA implements Formal Communication and meeting plans, NCL urges the agency to ensure that required meetings and communications with sponsors do not become too burdensome and time consuming for FDA review staff.

Mechanisms to Incorporate the Patient Perspective into the Drug Development and Review Processes

NCL supports the provisions in PDUFA VI that will enhance incorporation of the patient and caregiver perspective in drug development and decision-making. Gathering the patient and caregiver perspective helps both in the development of new drugs and biologics and in FDA's evaluation of the risks and benefits of these products.

Along these lines, we want to suggest that perhaps FDA create a fund for patients wishing to attend and speak at an FDA workshop but who cannot afford the expense involved to get to Washington. There has been criticism of the fact that patients who travel to Washington may only find support from the drug manufacturers to make the trip. We don't necessarily see that as the enormous conflict of interest that others have suggested, because we believe the patients and their conditions speak for themselves and deserve to be heard. However, to avoid this criticism, perhaps allowing those patients to apply for support from a Patient Workshop Travel Fund or something akin to that to cover the cost of travel is worth exploring.

Dedicated Resources for the Review of Breakthrough Therapies for Serious or Life Threatening Diseases or Conditions

NCL supports the provision of additional resources for the Breakthrough Therapy Program to expedite the development and review of innovative therapies for serious or life-threatening diseases or conditions when preliminary clinical evidence indicates that the drug may demonstrate a substantial improvement over existing therapies.

Increased Investment in the Sentinel Postmarket Surveillance System

NCL was pleased to see the agency's intent to continue development and expansion of the Sentinel System to realize its full value for the postmarketing safety review process and integrate Sentinel into postmarketing review activities and to conduct an analysis of how well Sentinel expansion is working – and whether it is helping to better predict adverse effects. NCL applauds the PDUFA VI further investment of \$50 million in the Sentinel System, and believes that it is critically important for independent researchers to have access to Sentinel and similar surveillance databases such as the IMEDS program.

Enhancement of the FDA's Ability to Hire and Retain a Highly Qualified Drug Review Staff

Enhancing FDA's ability to hire and retain a highly qualified drug review staff is one of the most important components of PDUFA VI. In order to carry out its public health mission to protect and advance the public health, FDA must be able to hire and retain highly trained and qualified technical and scientific experts to efficiently conduct reviews of drugs and biologics. Yes, many could perhaps find better salaries in the private sector, but many physicians and scientists are committed public servants, and FDA has a long history of attracting these talented and highly educated professionals. We agree with the FDA that the agency must find the means to continue to bring and retain such talent.

Processes to Gather Stakeholder Input

In addition to including the perspective of patient organizations, we appreciate FDA's commitment to including consumer organizations such as NCL and our colleagues in the consumer advocacy community in stakeholder meetings and discussions. As we have previously noted, the consumer viewpoint is separate and distinct from the patient voice and both need to be heard.

NCL Recommendations for Additional PDUFA VI Activities

Evaluation of the Safety of Off-Label Prescribing

NCL is disappointed that the PDUFA VI Agreement does not include the recommendations we made on off-label prescribing and direct-to-consumer advertising in our July 15, 2015 testimony. As we have said in our comments to the agency over the last five years, NCL believes that a portion of PDUFA funding should be directed towards examining the safety of off-label prescribing to address consumers' lack of awareness and understanding of the practice. Specifically, NCL suggests tracking the use of off-label medications by the public, which would contribute to our understanding of the use and health and safety implications of off-label prescribing.

Pre-Approval of Direct-to-Consumer Advertising

NCL thinks it is imperative that the FDA have the staff and resources to ensure that direct-to-consumer (DTC) drug ads are accurate and not misleading BEFORE they reach the public. We strongly believe the FDA should seek the authority to require that all DTC ads undergo review before public dissemination. This would enable agency staff to work with the industry to revise materials and content where needed so that misleading information does not reach consumers.

NCL recommends that user fees be allocated to support the hiring of additional staff to review DTC ads and respond to industry feedback in a timely fashion. We

believe that currently there is a dangerous imbalance between the volume of DTC advertising and the resources available for monitoring and reviewing advertisements. Thus, it is more important than ever for FDA to have the necessary resources to ensure that consumers receive balanced information about prescription drug products, because so much of what consumers know and understand about drugs comes from what they see on television and in other media sources.

Conclusion

NCL applauds the FDA for growing PDUFA since its inception in 1992 to a far more robust, efficient and effective program that strives to deliver the world's most safe, effective, and high quality drugs. We recognize that PDUFA VI is a reflection of that and continues to grow and evolve. We are pleased to have the opportunity to work with the FDA and to offer comments on the PDUFA VI User Fee Agreement. We look forward to continuing to work collaboratively with the FDA, the advocacy community, and industry stakeholders to ensure that consumers and patients have expanded and affordable access to the safe and effective drugs and treatments they need to maintain their health and enjoy a positive quality of life.