

Daniel Perry – April 12, 2010

Thank you for the opportunity to offer comments on the Prescription Drug User Fee Act and in particular the current PDUFA IV program.

I speak today on behalf of the Alliance for Aging Research. The Alliance was established in 1986 as a non-profit organization to advocate for public policies that promote medical and scientific research in aging and chronic diseases so as to improve the quality of life for people as they grow older.

Also on behalf of the Alliance for Aging Research, I would like to begin by thanking the FDA employees here today for the challenging and important jobs they perform each day that improve the lives of millions of people of all ages.

We are familiar by now with the rapid and consequential aging of populations throughout the developed world.

Beginning next January 1 the first of some 77 million American Baby Boomers will begin turn to age 65 and crowd onto the nation's Medicare rolls. Soon the U.S. population will go from having approximately 6,000 Americans turn age 65 every day, to 10,000 marking a 65th birthday; and we will stay at that higher level for the next 18 years. That is the full weight and duration of the post-war Baby Boom generation

We also know that many in this population will experience increasing risks to a long list of age-associated chronic ailments: coronary artery disease, stroke, heart failure, type II Diabetes, bone and joint disabilities, cancers, vision and hearing loss, clinical depression, and neurological diseases such as Alzheimer's and Parkinson's diseases to name just a few.

Unless we succeed at discovering better and more effective means to prevent, postpone or reduce the impact of diseases of aging, the U.S. could face a crushing wave of infirmities and disabilities carrying enormous social, cultural and economic costs.

At the Alliance for Aging Research we view the federal agencies that monitor public health and advance medical research and regulatory science as heroes. These agencies and their employees make up America's first line of defense as we face a future Silver Tsunami of chronic ailments.

We believe that FDA's processes by which new therapies are reviewed and approved are critical to ensure the continued translation of basic science discoveries into therapies older patients and their families want and need.

For the past four years the Alliance for Aging Research has chaired a coalition of more than 50 national non-profit groups focused on one disease of aging in particular – Alzheimer's disease.

This is the ACT-AD Coalition – which stands for Accelerate Cure/Treatments for Alzheimer's Disease.

It is comprised of dozens of prominent groups representing the interests of Alzheimer's patients, senior citizens, consumers, women's health advocates, caregivers, health care providers, and researchers.

The coalition is working with the nation's leading researchers, clinical trialists, drug developers as well as the FDA to take a closer look at regulatory challenges as new Alzheimer's therapies are developed, tested, reviewed and eventually brought to market. Several issues have come to light that are likely implicated in the scant number of treatments currently available for this disease, and the complete absence so far of therapies that truly change the course of the disease.

Alzheimer's is fast becoming the leading public health threat for older Americans. The disease afflicts at least 5.2 million Americans at present including half of all Americans over the age of 85 and half of the nursing home population today.

By 2030, when even the youngest Baby Boomer will be at least age 65, even these intolerable numbers will triple from where they are today.

The financial and social burdens of this disease are breathtaking. Five years ago Medicare spent \$91 billion on this one disease. In another five years, at \$189 billion, Alzheimer's will cost twice as much, and in 25 years Medicare spend as much on Alzheimer's as its entire budget today.

Our only hope is to make discoveries in the lab that then prove themselves in clinical trials and lead to better means to prevent, postpone or reduce the devastating impact of this disease. Much of our ACT-AD coalition's work on this disease focuses on:

- how to select patients for clinical trials testing treatments that can intervene at earlier stages of Alzheimer's disease,
- how to appropriately balance the benefits of potential therapies against the ever-present level of risk of harm from the treatment;
- how to generalize the results in a specific trial population to the larger patient population;
- And how to measure the clinical benefit of treatments for patients at the earliest discernable stages of the disease.

The Alliance for Aging Research and the ACT-AD Coalition looks forward to continuing a productive interaction with FDA on ways to design optimally productive clinical trials for Alzheimer's disease.

This, then, represents the context in which we view the prescription drug user fee act and PDUFA IV in particular.

To begin with, we applaud FDA's commitment under PDUFA IV to increase stakeholder involvement in discussions surrounding the advancement of science and the development of guidance documents help overcome unnecessary inhibitions in the development of new therapies for a host of age-associated diseases. We would like to see this involvement continued as part of PDUFA V.

We were pleased to see the inclusion of pre-market review enhancements specifically targeted to expedite drug development under PDUFA IV.

Guidance on topics such as adaptive and enriched trial designs is important not only for Alzheimer's disease but also for other diseases and conditions.

Guidance on the qualification of biomarkers for use in drug development for age-related diseases will positively impact patients by informing decisions on a treatment's efficacy and safety while reducing the time and efficiency of bringing a therapy to market.

Ongoing public exchanges with all parties interested in making more meaningful treatment options available to patients who need them, are initiatives we hope to see continue as the agency moves forward with PDUFA V negotiations.

The Alliance for Aging Research supports the approach in PDUFA IV to address the risk and benefits of treatments in the post-market environment. We support a "lifecycle" approach to drug evaluation that will allow better access to treatments for patients who need them, and a faster response in identifying and reducing risks for harm. All drugs come

with risks and FDA should have the tools it needs to evaluate the risks and benefits a new drug provides simultaneously.

But we believe when physicians and patients are presented with the best available information, patients, particularly those with little or no therapeutic options for life-threatening illnesses, are best able to determine what level of risk is acceptable. We support continued communication with the patient advocacy community as the FDA continues to implement its PDUFA IV post-market authorities to ensure that information is being made available to patients and health care providers in an effective way.

We know that new mandates placed on the FDA under PDUFA IV for enhanced post-market safety surveillance required rapid development of systems capable of active risk identification and analysis. These also increase the workforce demands on FDA staff.

These new responsibilities were placed on the Agency at a time when the FDA's own Science Board had identified the Agency's IT infrastructure as inadequate for its current operations. We hope that once FDA's 5-year drug safety plan is fully implemented and new staff fully trained in these areas, the new PDUFA IV mandates will become less of a strain on the Agency and not contribute to problematic delays in bringing therapies to market.

Finally, the Alliance for Aging Research recognizes the important funding user fees currently provide to the FDA. For the last four years we have served on the Board of the Alliance for a Stronger FDA.

This diverse and very effective coalition is committed to increasing the resources necessary for the FDA to carry out its ever-expanding responsibilities.

Historically, the user fee program has been successful in meeting initial goals of reducing application backlogs and ensuring that important therapies became available to patients sooner by enabling FDA to hire more staff, improve systems, and better manage drug review processes.

User fees are properly limited in their scope of use and cannot be used to cover many of the increasing costs the Agency sees each year. Therefore, user fees do not offer flexibility for the Agency to adopt initiatives that could increase its capacity to modernize as science and technology evolve.

There are diverse ways in which FDA can facilitate biomedical innovation, such as by adopting more modern preclinical testing methods, but these can only be realized if the Agency has the ability to allocate funds where they are most needed.

This cannot be achieved through user fees alone. Therefore, our organization and others will actively call on Congress to provide appropriate funding to the Agency so that it can play a leading and proactive role in improving the health of aging Americans.

Thank you for the opportunity to share these comments on PDUFA and the current PDUFA IV process. We welcome the opportunity to provide additional information as the PDUFA V process moves ahead and look forward to working with the Agency directly on initiatives that will more quickly move needed treatments to patients and their families in a safe and effective manner.