

April 24, 2012

The Honorable Tom Harkin
The Honorable Mike Enzi
Committee on Health, Education, Labor & Pensions
United States Senate
430 Dirksen Office Building
Washington, DC 20510

Dear Chairman Harkin and Ranking Member Enzi:

On behalf of the National Alliance on Mental Illness (NAMI), I am pleased offer our strong support for the Food and Drug Administration Safety and Innovation Act (FDASIA). As the nation's largest national organization representing people living with serious mental illness and their families, NAMI is pleased to support this important bipartisan legislation. We look forward to working with you and all of your colleagues on the HELP Committee to ensure that this legislation moves forward as quickly as possible. It is critical that this entire legislative package moves forward expeditiously and is signed into law in advance of the expiration of the current FDA user fee agreements on September 30. NAMI deeply appreciates the strong bipartisan cooperation that has occurred thus far on the HELP Committee on this important legislation.

The Food and Drug Administration Safety and Innovation Act Improves Access to Innovative Treatments for Serious Mental Illness

NAMI has always placed the highest priority on expanding access to newer and more effective treatments for serious mental illness. The reality is that all of the current treatments for serious mental illness are palliative in nature and are designed to alleviate symptoms (both positive and negative) and improve functioning. What is desperately needed are a new generation of treatments and therapies that genuinely change the course of disorders such as schizophrenia and bipolar disorder and allow for complete recovery. At NAMI, we refer to this as moving to cure. We are not there yet, but cutting edge research on genetics and biomarkers are demonstrating enormous promise. As this research moves forward, it must be accompanied by a modern and efficient PDUFA process at the FDA.

NAMI also believes that incremental improvements to the treatments we have today for serious mental illness are also critically important for many people living with these disorders. For example, new compounds that can demonstrate innovation with respect to negative symptoms or cognition and executive functioning in schizophrenia can be critical tools in helping a patient attain greater independence, employment or community integration. Likewise, a new compound that offers incremental improvements with respect to a particular side effect profile can make a vast difference in helping with treatment adherence for many patients. Improvements to PDUFA can and should take into account this incremental progress that can genuinely advance treatment for people living with serious mental illness.

NAMI Supports the PDUFA V Technical Agreement

NAMI was pleased to take part in the Patient and Consumer Stakeholder Group that has worked with the FDA throughout this PDUFA V process. We are grateful for the time and resources that the leadership at CDER has put into this process over the past 18 months. NAMI is now pleased to go on record in support of the major provisions in this Technical Agreement and is pleased that they are included in this legislation.

- 1. **Performance Goals** -- NAMI strongly supports the new performance goals that will increase drug review efficiency and predictability and result in more consistent and transparent drug reviews. This Agreement will also more clearly discern how efficacy and safety parameters are balanced as part of the FDA's risk-benefit analysis.
- 2. **Enhanced Benefit Risk Assessment** -- The Agreement should bring greater transparency to the benefit-risk assessments FDA makes that have been opaque and difficult to understand, especially when decisions have been made across multiple divisions and offices. These improvements should also bring more transparency to this process.
- 3. **Electronic Submissions and Standardization of Application Data** -- This new requirement for electronic submission and standardization of application is long overdue and is central to making the overall review process more timely, efficient and less costly.
- 4. **Independent Review of PDUFA Performance Goals** -- In order to ensure that the PDUFA V goals are met, there must be an independent third party assessment, with full opportunity to all stakeholders to offer comments on the assessment.
- 5. **Regulatory Science Improvements** -- NAMI supports the new investments in the Agreement aimed at improvements on pharmacogenomics and biomarkers. The application of qualified biomarkers has enormous potential to accelerate drug development by helping to identify and predict which patient will respond to a particular medication. Biomarkers offer particular promise with respect to treatment for schizophrenia. FDA needs to be able to access new resources to augment its clinical pharmacology and statistical capacity to better address submissions that propose to utilize biomarkers or pharmacogenomics.
- 6. Patient Reported Outcomes (PROs) -- PROs can be critical endpoints for measuring the benefit-risk profile of a new treatment from the patient's perspective. NAMI is pleased that the Agreement includes advancement and validation of PROs. This should help improve the agency's clinical and statistical capacity to address submissions involving PROs and other end point assessment tools.
- 7. **Patient Safety Improvements, REMS & Sentinel** -- NAMI supports the improvements in PDUFA V to enhance FDA's existing safety system. The improvement to REMS and Sentinel are vital to this ongoing effort to ensure patient safety. These improvements should allow FDA to test the feasibility of using Sentinel for evaluating drug safety issues that may require some form of regulatory action such as a label change.

NAMI Supports Permanent Reauthorization of BPCA & PREA

NAMI is pleased that this legislation includes permanent extension of both the Best Pharmaceuticals for Children Act and the Pediatric Research Equity Act. BPCA and PREA have been remarkably successful in ensuring that medications used in children are tested and labeled appropriately for pediatric use. As a result of BPCA and PREA, the incentives that allow for appropriate drug development studies to be conducted in children have been successful, with more studies conducted over the past 10 years than were conducted over the previous 5 decades. As a result, 426 drug labels have been revised with new pediatric information and an estimated 50% of drugs used in children are now studied as compared to just 20% before the enactment of these two laws. It is critical that BPCA and PREA be permanently extended.

NAMI Supports the Drug Approval and Patient Access Provisions in FDASIA

Title IX contains a number of provisions that NAMI strongly supports to put in place new processes for expediting the development and review of medications intended to treat serious and life threatening diseases. Many of the important provisions in Title IX have been included in previous bipartisan bills including the MODDERN Cures Solution Act and the Advancing Breakthrough Therapies for Patients Act. Given NAMI's previous support for both of these bills, we are pleased to see them included in the Committee's bill.

- 1. Expedited Approval for Serious or Life Threatening Diseases or Conditions NAMI would urge the HELP Committee to consider additional clarity with regard to the definition of surrogate endpoints for complex conditions such as schizophrenia where research has demonstrated irreversible morbidity related to acute episodes of psychosis.
- 2. **Breakthrough Therapies** NAMI supports this provision and recommends that the Committee consider further clarifying the scope of language defining breakthrough therapies and requiring a demonstration of "substantial improvement" over existing therapies on clinically significant endpoints, such as substantial treatment effects observed early in clinical development. In moving forward, it will be important for this definition to integrate improvement over existing therapies not only with respect to clinical symptoms, but also serious side effects associated with existing therapies.

NAMI Supports Bipartisan Efforts in FDASIA to Address Drug Shortages

NAMI is pleased that Title X of the bill includes a range of important reforms to ensure greater accountability and transparency with respect to shortages of prescription drugs. We place the highest value on ensuring that people with mental illness are able to access the treatment they need. Acute shortages of medications can operate as a substantial barrier – particularly with psychotropic medications that are not clinically interchangeable. Provisions in the bill instituting new advance notification requirements for manufacturers before supply disruptions actually occur will help patients and prescribers better cope with shortages. In addition, new authority in the bill for the Secretary to work with the FDA to evaluate the risks of future shortages and take immediate actions to avoid them in the future will also help prevent future shortages.

As this legislation moves on to the full Senate, NAMI would urge you to work with your colleagues on the Judiciary Committee to address continued challenges associated with

shortages of medications to treat Attention Deficit Hyperactivity Disorder (ADHD). Ensuring adequate availability of product is a particular challenge for these medications as the Drug Enforcement Administration (DEA) plays a critical role in regulating manufacturing capacity of both the immediate release and extended release versions. This is required as amphetamine is regulated as a Schedule 2 controlled substance. In particular, NAMI would urge you to work with the Judiciary Committee to integrate DEA into the new federal oversight structure that will work to monitor and prevent shortages and ensure greater accountability on the part of the DEA with respect to consideration of allocation of increased manufacturing quota.

NAMI Supports Efforts to Address Conflict of Interest in FDA Advisory Panels NAMI would like to express support for Section 1121 of the bill and the proposal to streamline the process for appointments to FDA advisory panels. Reforms such as eliminating the cap on the number of conflict of interest waivers the Secretary can grant are long overdue. In addition, simplifying the process will enhance recruitment and ensure greater transparency when waivers are granted. These reforms will help ensure greater balance in FDA advisory panels and help the agency attract needed clinical expertise to ensure that it is making decisions based on the best scientific expertise available.

Conclusion

NAMI would like to express our thanks and support for the Food and Drug Administration Safety and Innovation Act. We look forward to working with you to ensure its passage by the full Senate HELP Committee and expeditious action on the part of the full Senate.

Sincerely,

Michael J. Fitzpatrick, M.S.W.

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Executive Director