



May 18, 2015

The Honorable Fred Upton  
The Honorable Diana DeGette  
Committee on Energy & Commerce  
U.S. House of Representatives  
2125 Rayburn House Office Building  
Washington, DC 20515

Dear Chairman Upton and Representative DeGette:

On behalf of the National Alliance on Mental Illness (NAMI), I am writing to offer our support for the current of the 21<sup>st</sup> Century Cures legislation now before the Energy & Commerce Committee. As the nation's largest organization representing people living with serious mental illness and their families, NAMI is pleased to support this bipartisan effort to advance biomedical research and develop new treatments for a broad range of devastating diseases.

An estimated 11.5 million American adults live with a mental illness that is often seriously disabling, such as schizophrenia, bipolar disorder, and major depression. Based on estimates for 2010, mental illnesses accounted for 21.3% of all years lived with disability in the United States. Among the top 20 causes of years lived with disability, five were mental illnesses: major depressive disorder (8.3% of the total), anxiety disorders (5.1%), schizophrenia (2.2%) and bipolar disorder (1.6%).

As you know, suicide is the 10th leading cause of death in the US, accounting for the loss of more than 41,000 American lives each year, more than double the number of lives lost to homicide. The social and economic costs associated with these illnesses are tremendous. A cautious estimate places the direct and indirect financial costs associated with mental illness in the U.S. at well over \$300 billion annually, and it ranks as the third most costly medical condition in terms of overall health care expenditure, behind only heart conditions and traumatic injury. In addition, adults living with serious mental illness represent an enormous public health burden resulting from high rates of medical co-morbidities such as asthma, heart disease, COPD and diabetes.

Unfortunately, the current therapies available to treat serious mental illness fall short of the advances that we have seen in recent decades to treat a range of diseases including HIV-AIDS, cancer, heart disease and stroke. The medications available to treat illnesses such as schizophrenia, bipolar disorder and major depression are largely palliative and help patients manage their symptoms in order to improve functioning in order to move toward recovery. The fact is that there are no disease modifying therapies for these illnesses, much less a cure. Moreover, the medications currently available to treat serious mental illness often have significant side effects that complicate treatment adherence. Thus, NAMI feels strongly that efforts to promote scientific advancement and discovery of new therapies must address the twin goals of achieving breakthrough achievements, as well as incremental improvements that can improve

adherence through less debilitating side effects and new delivery technologies. It is NAMI's hope that 21<sup>st</sup> Century Cures can and will move toward these important goals.

### **NAMI Supports 21<sup>st</sup> Century Cures**

NAMI continues to share the draft legislation's goals to accelerate "the cycle of discovery, development, and delivery of promising new treatments and cures." This goal clearly addresses the continuum of activity from basic to translational to clinical research through the regulatory processes of approval and ultimately to payment by public and private payers. This draft adjusts key activities of the National Institutes of Health (NIH) and the Food and Drug Administration (FDA) to advance scientific discovery.

From NAMI's perspective as an advocacy organization for those affected by mental illness, each of these elements must work in concert to maximize overall benefits. Innovation and discovery in the laboratory do no good if they are never tested in real world treatment settings. Successful treatments that meet high quality standards are useless if they are not covered by programs such as Medicare and private health plans and accessible to patients.

NAMI is pleased that the current draft legislation includes the following provisions to advance research, drug discovery and access to new therapies:

### **NIH Innovation Fund**

NAMI strongly supports Section 1002 of the current draft and the additional resources for the NIH over the next five years. Sequestration in 2013 and ongoing flat funding for the NIH in recent years has had devastating consequences for the ability of NIH institutes to invest in basic and translational research and assisting in development of new generation of young scientists. "Pay lines" for new and competing awards at a number of NIH institutes (including NIMH) are now dipping below 20%, meaning that more than 80% of proposals that meet a standard of high scientific merit are not funded, solely because of lack of available funding. This Innovation Fund will help reverse these negative trends deliver enormous dividends for the nation down the road.

### **Patient-Focused Drug Development**

During stakeholder discussions during development PDUFA V, NAMI supported the concept of creating a series of meetings that would bring patient and caregiver voices as early as possible into the drug development process. The patient-focused drug development (PFDD) meetings undertaken by the FDA thus far have been largely driven by the FDA and patient groups and resulted in valuable publicly-available resources written in the voice of patients to help inform new endpoint development, outcome measure selection in clinical trials, and benefit/risk decision making by regulators. NAMI is hopeful that the expansion of PFDD in this legislation can include more scientific rigor through the development of methodologies to guide structured interactions with patients and their families.

### **Council for 21st Century Cures**

NAMI supports the creation of a Council for the 21st Century Cures that will be aimed at fostering collaborations and establishing an agenda for accelerating the discovery, development, and delivery of innovative cures and treatments. Representatives from the patient and research communities must have a voice as the Consortium develops recommendations on how to fill gaps and realize opportunities in the discovery, development, and delivery cycle.

### **Precision Medicine Guidance**

NAMI is very encouraged by the promise of “precision medicine” as a future arena of innovation to advance treatment for serious mental illness. Work now underway at the NIMH is demonstrating how more precise diagnostics, mapping of the connectomes in the brain and other advances will allow clinicians to individualize the most optimal treatment for people living with schizophrenia, bipolar disorder and major depression. The FDA has a role to play here as well. The requirements in Section 2041 of the draft bill will ensure that potential sponsors of precision drugs and devices have clarity with respect to the regulatory path forward.

### **Streamlined Data Review Program**

NAMI supports Section 2063 and the requirement for the FDA to establish a streamlined data review program to make use of submitted clinical data summaries to support approval of new drug indications under certain circumstances. This and other provisions in Subtitle D will go a long way to creating efficiencies in clinical trial review and speed development of new therapies, without lowering current standards for safety and efficacy in the approval process.

### **Facilitating Responsible Communication of Scientific and Medical Developments**

NAMI has been concerned for years that while off-label prescribing often occurs in treatment for serious mental illness, it is not always informed by peer reviewed research. This is unfortunate since evidence-based treatment guidelines and articles in peer reviewed journals exist that provide a rigorous scientific basis to treat complex disorders such as bipolar disorder and borderline personality disorder for which there is no on-label indication. It is a simple fact that the FDA label rarely keeps up with the latest and most important scientific studies.

In the past, NAMI has urged the FDA to develop clear rules by which peer reviewed research can be shared with prescribing physicians to ensure that off-label prescribing is informed by the most appropriate and best science. NAMI supports inclusion Section 2102 and urges that it create a safe harbor under which the FDA can provide clarity on communication to physicians.

### **Priority Review for Breakthrough Therapies**

NAMI supports Section 2201 and the requirement for the FDA to establish a priority review program for breakthrough devices. NAMI believes that there is enormous promise in research and development toward a new generation of devices to treat a range of conditions including treatment resistant depression and OCD. The FDA needs to establish a process to ensure that patients can access these advanced therapies where possible.

### **Telehealth Services Under Medicare**

NAMI supports the work of the Committee’s Bipartisan Telemedicine Member Working Group. Telemedicine is showing enormous promise in expanding access to treatment in rural and frontier communities. Section 3021 will go a long way toward removing the current barriers in the Medicare program that preventing expansion of promising technologies.

NAMI is hopeful that these provisions in the current draft can change the current research and drug discovery paradigm to deliver new therapies for patients and their families. At the same time, NAMI would also like to express disappointment that a number of important provisions in previous drafts of 21<sup>st</sup> Century Cures legislation have been deleted from the version reported by

the Health Subcommittee last week. In particular, NAMI would like to restate support for the following proposals that we hope the Committee can work to restore as the legislation moves forward:

**Dormant Therapies**

NAMI continues to support the concept of allowing drugs and biologics designated as “Dormant Therapies” to be tested again for areas where few or no other treatment options exist. Given the current recession that exists with respect to investment in new therapies to treat serious mental illness, Congress needs to put in place mechanisms that will provide additional incentives for the redevelopment of medicines in the private sector. NAMI would urge the Committee to add this important proposal back into 21<sup>st</sup> Century Cures.

**New Therapeutic Entities**

NAMI continues to support proposed additional two years of exclusivity for significant improvements to existing molecules under Section 505(b)(2) of FFDCA. NAMI would urge the Committee to add this proposal back into the legislation and clarify that this includes new technologies and delivery systems such as long acting therapies and formulations that improve treatment adherence and fewer adverse events.

**Conclusion**

NAMI continues to support moving 21<sup>st</sup> Century Cures legislation forward in the House Energy and Commerce Committee. It is critical for the House to act on legislation to advance scientific discovery and bring newer and better treatments forward across a broad range of illnesses and diseases, including serious mental illness. Thank you for leadership on this important legislation.

Sincerely,



Mary Gilberti, J.D.  
Executive Director