National Health Council

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February 13, 2015

Representative Fred Upton
2183 Rayburn House Office Building
Washington, DC 20515

Representative Diana DeGette
2368 Rayburn House Office Building
Washington, DC 20515

RE: 21st Century Cures discussion draft

Dear Chairman Upton and Representative DeGette:

We would like to voice our enthusiasm and support for the 21st Century Cures discussion draft recently released by the House Energy and Commerce Committee. We commend the Committee for advancing this legislation and for bringing many of these critical issues to the forefront of the policy discussion.

The NHC is the only organization that brings together all segments of the health community to provide a united voice for the more than 133 million people with chronic diseases and disabilities as well as their family caregivers. Made up of more than 100 national health-related organizations and businesses, its core membership includes the nation's leading patient advocacy groups, which control its governance. Other members include professional societies and membership associations, nonprofit organizations with an interest in health, and major pharmaceutical, medical device, biotechnology, and insurance companies.

This proposal will impact the way treatments are studied, developed, regulated, and ultimately made available to all Americans, particularly the millions of individuals who suffer from chronic diseases and disabilities. Many provisions in the proposal would enhance aspects of the research and regulatory environments and accelerate pathways for developing promising treatments:

1) *Further Articulating a Process for Patient-Focused Drug Development* (Title I. Subtitle A. Sec. 1001)

   We have been encouraged by the progress made in recent years by FDA and several other stakeholders to help shape and implement Patient-Focused Drug Development (PFDD). We applaud the inclusion of additional provisions in 21st Century Cures to further bolster these efforts by mandating the development of a structured benefit-risk framework for drug evaluation and creation of a clear pathway for the collection, evaluation, and integration of patient

experience data into benefit-risk decisions. These advancements will continue to help ensure that the patient voice is captured when making important regulatory approval decisions.

2) *Facilitating Responsible Communication of Scientific and Medical Developments* (Title I. Subtitle H. Sec. 1141)

We are equally encouraged to see that manufacturers are considering the benefits of engaging with patients throughout the drug development pipeline. However, because manufacturers oftentimes feel their interactions with patients are at risk of being construed as discussions of unapproved medicines or unapproved uses of approved medicines, they are inclined to forgo invaluable input from patients to inform earlier stage research decisions, such as endpoint selection and clinical trial design. We urge the Committee to use this placeholder language as an opportunity to clarify that this type of patient engagement is both legal and encouraged.

3) *Providing FDA the authority to designate new drugs that treat unmet medical needs as "Dormant Therapies"* (Title I. Subtitle L. Sec. 1221-1223)

Bringing promising treatments to all individuals suffering from debilitating and life-threatening diseases remains a critical priority for the patient community. However, existing laws related to patents can discourage investigation of treatments for unmet medical needs. Many promising treatments do not meet the technical requirements of patent eligibility. Additionally, because patent life runs concurrently with research and development, research into products that take longer to develop is less likely to occur. Therefore, we strongly support the creation of Dormant Therapies, which removes technical patent requirements that are unrelated to medical promise and starts the period of protection at FDA approval. This would incentivize researchers to pursue the development of drug compounds on the basis of their promise rather than their patents.

4) *Standardization of data and expansion of data access with appropriate privacy safeguards for the purposes of accelerating research* (Title II. Subtitle F. Sec. 2081, 2082, 2085, 2086, 2087, 2088, 2091, and 2092)

Establishing a data sharing framework to efficiently increase the secure flow of rich data will help unlock the full potential that data holds for robust research and faster cures of debilitating disease. Furthermore, standardizing publicly available registry and research results will help simplify patient access to important information about ongoing clinical trials and therefore facilitate opportunities for patients seeking treatments through these trials.

5) *Allowing patient information to be accessed, used, and shared for research* (Title II. Subtitle M. Sec. 2221)

While privacy is critically important to all people, so too is the need for more research into finding cures and treatments. The use of patient data for research has the potential to unlock information that can be used for research. Therefore, we strongly support these provisions which create flexibility for researchers to share data sets, while strengthening

the security of data, provide one-time patient authorization, expand appropriate remote access, allow entities collecting data to use it for research, and treat research like other public health activities.

6) *Reducing Regulatory Duplication and Unnecessary Delays in IRB Review* (Title III. Subtitle A. Sec. 3001-3002)

The Institutional Review Board (IRB) approval process is a necessary safeguard to ensuring appropriate patient protections; however, aspects of the process have proven to be cumbersome and redundant. We commend the Committee for supporting ways to create efficiencies and reduce duplications, which will help expedite reviews and ultimately allow research to progress faster. We support the regulatory changes proposed and encourage Congress to continue to identify other areas where other redundancies may be eliminated.

Please do not hesitate to contact Eric Gascho, our Assistant Vice President, Government Affairs, if you or your staff would like to discuss these issues in greater detail. He is reachable by phone at 202-973-0545 or via e-mail at egascho@nhcouncil.org. You may also reach me on my direct, private line at 202-973-0546 or via e-mail at mweinberg@nhcouncil.org.

Sincerely,

Myrl Weinberg

Myrl Weinberg, FASAE, CAE
Chief Executive Officer