National Health Council
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October 6, 2015

The Honorable Lamar Alexander
Chairman
US Senate Committee on Health, Education, Labor, and Pensions
455 Dirksen Office Building
Washington, DC 20510

The Honorable Patty Murray
Ranking Member
US Senate Committee on Health, Education, Labor, and Pensions
154 Russell Senate Office Building
Washington, DC 20510

RE: Innovation for Healthier Americans

Dear Chairman Alexander and Ranking Member Murray:

We would like to voice our support for the Innovation for Healthier Americans Initiative and commend the Senate Health, Education, Labor, and Pensions (HELP) Committee for bringing many of these critical issues about medical innovation 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 and 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 health insurance companies.

We agree with the Committee that the fundamental challenge to medical innovation in the United States is the lengthy and costly development and review process for new products. As the Committee weighs potential solutions to this fundamental problem, the NHC offers the following solutions:

1) *Increase funding for the National Institutes of Health (NIH).* Basic research conducted through NIH funding is the lifeblood of research to find new treatments and cures for people with chronic diseases and disabilities. As the HELP Committee works to craft legislation to bring lifesaving cures and treatments to patients, increasing the funding of NIH through mandatory spending should be a top priority.

2) *Incorporate the patient perspective in product development.* We strongly believe engaging patients throughout the product development process is critical to ensuring that innovative therapies reach patients faster. Many stakeholders are beginning to seek patient input early in the development process with the intent of ensuring not only that those products are being designed to meet patient needs, but also that the studies conducted to inform regulatory approval and eventual clinical use are capturing information that is highly relevant and specific to the end-users themselves: the patients.

   As the Senate works to craft legislation, we strongly urge the inclusion of language that will further the use of patient input in drug development and regulation. One recommendation is to create a process under which an entity can submit patient data to be incorporated into the FDA's structured benefit-risk framework. Accompanying guidance should be included to define the types of activities that entities may undergo to seek such input.

   Another area we believe warrants further consideration and action from Congress is the current uncertainty and lack of predictability that comes with engaging with patients, particularly from the perspective of manufacturers. 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 early-stage research decisions, such as clinical trial design. Many companies conduct trials only to learn after their drug is approved that the outcomes they chose to study are of no value or interest to the patients who will take the drug. A more rational approach would be to allow manufacturers to engage patients appropriately in order to inform drug design, research, and development so that the end product is a drug that is optimally valuable to patients. Clearer guidance or policies are required to encourage these collaborations and to create a more predictable environment for manufactures to engage patients.

3) *Mitigate the impact that issues related to patent protection have on drug development.* 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 a significantly longer period of time to develop is less likely to occur. Therefore, we strongly support the creation of an approval pathway for Dormant Therapies, which would remove technical patent requirements that are unrelated to medical promise and that would start the period of protection at the point of FDA approval. This pathway would incentivize researchers to pursue the development of drug compounds on the basis of their clinical promise, rather than their patent life.

   We were disappointed that the House did not address this issue in the 21st Century Cures Act. Our support for the provision was shared by more than 40 other patient organizations via a support letter that was sent to Representatives Upton and DeGette. The letter is attached for your reference.

4) *Allow patient information to be accessed, used, and shared for research*. While privacy is critically important to all people, those with chronic diseases and disabilities typically consider the benefits of research to outweigh this risk. Therefore, we should grant researchers the flexibility to share data sets, while strengthening the security of data; provide one-time patient authorization for the use of their information; expand appropriate remote access; allow entities collecting data to use it for research; and treat research like other public health activities.

5) *Reduce regulatory duplication and unnecessary delays in IRB review*. 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. Solutions are needed to create efficiencies and reduce duplications, which will help expedite reviews and ultimately allow research to progress faster.

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.

Sincerely,

Marc Boutin, JD
Chief Executive Officer

March 17, 2015

The Honorable Fred Upton
2183 Rayburn House Office Building
Washington, DC 20515

The Honorable Diana DeGette
2368 Rayburn House Office Building
Washington, DC 20515

RE: 21st Century Cures Discussion Draft

Dear Chairman Upton and Representative DeGette:

We, the undersigned organizations, wish to express our sincere gratitude for the inclusion of the Dormant Therapies Act into the 21st Century Cures discussion draft bill and offer our support to ensure passage of the legislation. The dormant therapies provisions contained in Title I, Subtitle L of the discussion document create a truly vital pathway for the development of cures and new treatments that have the potential to improve the lives of millions of Americans living with chronic diseases and disabilities.

We endorse and support the creation of the dormant therapies pathway.

Millions of Americans live with diseases and disabilities that currently have few or no treatments to affect the course of the illness. The people with these conditions are desperately in need of therapies that will stop or slow the progression of the disease or offer a cure. With this legislation, Congress can provide the incentive for researchers to pursue innovative therapies that would otherwise be abandoned for reasons having nothing to do with efficacy or safety.

The dormant therapies provisions will provide a fixed period of protection from generic competition, which begins when a medicine is approved by the Food and Drug Administration (FDA). Certainty regarding a medicine's patent protection can be a significant driver of whether or not a company develops a promising therapy, despite the medicine's potential benefit to patients. The risk of not having enough patent term left when a product gets approved discourages companies from pursuing new treatments that take a long time to develop – such as medicines that prevent diseases, treat early-stage disease, or treat a disease that has no existing treatments, is not well understood, or progresses slowly.

The dormant therapies provisions will remove this barrier and accelerate the development of promising new medicines that treat an unmet medical need, regardless of patent protection. Further, the legislation's unique patent waiver provisions will create a predictable timeline for generic or biosimilars manufacturers to bring their products to market, giving patients and their providers realistic expectations of when these lower-cost alternatives will be available.

The dormant therapy designation is only granted to a treatment if the FDA determines that it meets an unmet medical need.

While the undersigned organizations understand that Congress must thoroughly debate the length of the period of protection, we believe that this debate must occur to realign incentives to ensure that products that treat the most challenging conditions come to market. We believe that creating a time-

certain period of protection that starts at the date of FDA approval will allow science, not patents, to drive biopharmaceutical manufacturers to develop products for patients with the greatest need.

We look forward to working with Congress to ensure passage of this urgently needed legislation to speed new treatments and greatly improve existing care for millions of Americans living with chronic conditions.

Sincerely,

Alpha-1 Foundation
The ALS Association
Alzheimer's Association
American Association for Respiratory Care
American Association on Health and Disability
American Autoimmune Related Diseases Association
American Brain Coalition
American Foundation for Suicide Prevention
American Kidney Fund
Amputee Coalition
Arthritis Foundation
Asthma and Allergy Foundation of America
Caregiver Action Network
COPD Foundation
Epilepsy Foundation
Global Liver Institute
HealthHIV
Huntington’s Disease Society of America
Hydrocephalus Association
Immune Deficiency Foundation
International Cancer Advocacy Network
International Essential Tremor Foundation
Lupus Foundation of America
The Marfan Foundation
National Alliance on Mental Illness
National Alopecia Areata Foundation
National Eczema Association
National Health Council
National Hemophilia Foundation
National Kidney Foundation
National Osteoporosis Foundation
National Psoriasis Foundation
Osteogenesis Imperfecta Foundation
Parkinson's Action Network
PKD Foundation
Prevent Cancer Foundation

Sjögren's Syndrome Foundation
Spina Bifida Association
Tremor Action Network
Us TOO International Prostate Cancer Education and Support Network
The Veterans Health Council
Vietnam Veterans of America

Signature List as of April 8, 2015