

May 13, 2015

The Honorable Fred Upton
Chairman
Committee on Energy and Commerce
2125 Rayburn House Office Building
Washington, D.C. 20515

The Honorable Diana DeGette
Committee on Energy and Commerce
2368 Rayburn House Office Building
Washington, D.C. 20515

Re: Amendment in the Nature of a Substitute to the Committee Print [21st Century Cures Act]

Dear Chairman Upton and Congresswoman DeGette:

On behalf of the American Society of Nephrology (ASN) thank you for the opportunity to provide input to the Energy and Commerce Committee regarding the “21st Century Cures Act” discussion document. ASN commends the Committee for its continued bipartisan commitment to accelerating the discovery, development, and delivery of promising new treatments to patients. The society also thanks the Committee for its efforts to engage stakeholders and solicit feedback throughout the yearlong process of developing this discussion draft.

ASN, the world’s leading organization of kidney health professionals, represents more than 15,000 health professionals and scientists who are dedicated to treating and studying kidney disease and to improving the lives of the millions of patients it affects. ASN particularly supports efforts that bolster the ability of federal agencies and the American research and development enterprise to solve scientific challenges at every level from basic science through care delivery. The society strongly supports the bipartisan 21st Century Cures initiative and stands ready to collaborate to advance this important objective.

Kidney disease affects more than 20 million Americans. There are many unique causes of kidney disease, but when any type of kidney disease progresses to kidney failure, patients require either dialysis or transplantation to stay alive. Currently, 600,000 Americans have complete kidney failure, called end-stage renal disease (ESRD). Kidney disease disproportionately affects racial and ethnic minority populations, is associated with multiple co-morbidities including heart disease and diabetes, and is one of the most costly chronic conditions in the United States.

While America’s scientific leadership has yielded important treatments for some patients, others still wait because the state of biomedical research and innovation in certain

diseases is not as advanced; kidney disease is among the conditions for which we must accelerate the pace of innovation.

Although people with kidney failure requiring dialysis (ESRD) comprise less than 1 percent of Medicare beneficiaries, they account for nearly 7 percent of Medicare's budget: the Medicare ESRD Program is unique in that it covers every American with kidney failure regardless of age or income. Yet despite these staggering costs, the fundamental principles of dialysis have not changed and patients with ESRD have seen only incremental improvements in their therapy in decades.

The 21st Century Cures initiative is a significant opportunity to spur research and facilitate therapeutic development in kidney care and in other diseases where the state of biomedical research and therapies in certain diseases is not as advanced.

Again, ASN thanks the Committee for its ongoing stakeholder engagement, and offers the following positive comments for consideration:

Title I: Discovery

Section 1001 and 1002. NIH Reauthorization and NIH Innovation Fund.

ASN commends the Committee for proposing reauthorization of the NIH through 2018 at more robust funding levels than the agency has seen in recent years, and thanks the Committee for the addition of the NIH Innovation fund in the discussion draft and strongly supports this provision.

As noted above, there has been relatively little innovation in the treatment of patients with kidney disease since the inception of the Medicare End-Stage Renal Disease (ESRD) Program despite the program's cost – likely directly interrelated, NIH investments in kidney research were less than 1% of total Medicare costs for patients with kidney disease (\$585 million vs. \$80 billion in 2014).

This is just one of many examples highlighting the need for a greater focus on, and resources allocated for, highly innovative research at the NIH. Investing in innovative research is the crucial to reducing the significant burden of disease on patients and the curtailing expenditures. While recognizing that these funds would require appropriation, ASN supports the NIH reauthorization and establishment of an NIH Innovation Fund laid out in the discussion draft.

Section 1021. NIH research strategic plan.

ASN supports Section 1021, directing NIH and each Institute Center to develop and periodically update a strategic plan with input and feedback from patients, scientific experts, and other stakeholders—including health professional organizations—throughout the planning process. The society also supports and asks Congress to also direct the NIH and each Institute Center to examine the federal costs related to the care for each disease area when prioritizing research in such a planning effort.

Section 1025. NIH travel.

While ASN recognizes the importance of reforms to prevent the abuse of federal funding for travel, recent travel bans and budget cuts are negatively affecting federal employee participation in scientific meetings and conferences.

Participation in meetings and conferences is critical for executing and advancing the mission of NIH, the Food and Drug Administration (FDA), the Centers for Medicare and Medicaid Services (CMS), and other federal public health agencies. Not only is participation in these meetings essential for the exchange of knowledge to advance science and medical care, it is also in many cases necessary for maintaining professional licenses for practicing medicine.

ASN concurs that participation in or sponsorship of scientific conferences and meetings is essential to the mission of the NIH and supports provisions that would facilitate NIH staff participation, such as specifically excluding NIH from federal travel restrictions, or other mechanisms.

Section 1028. High-risk, high-reward research.

ASN supports NIH investments in novel and innovative science that could lead to breakthroughs. NIH's history of funding primarily investigator-initiated research has yielded unparalleled dividends in medical discoveries and cures. This successful model of research funding should continue to be robustly and stably funded. However, ASN also supports the pursuit of other high-risk, high-reward funding models in addition to extramural, investigator-initiated grants.

The private and philanthropic sectors have successfully been using prize competitions for years as a mechanism for spurring scientific and technologic breakthroughs in a number of fields. Unlike traditional research and development models, competitions have the added benefit that the prize is only paid out if a competitor wins, and the competitions also draw competitors from outside those traditionally interested in the space. The 2007 America Creating Opportunities to Meaningfully Promote Excellence in Technology, Education, and Science Act of 2007 (also known as the America COMPETES Act) authorizes federal agencies to conduct prize competitions.

As such, ASN believes Congress should investigate dedicating funding towards prize competitions, especially in fields where innovation has been stagnant, including nephrology. However, the society emphasizes that prize competitions must not come at the expense of traditional research funding models, and that this approach to promoting innovation should be used only in certain, carefully considered situations.

Sec. 1041. Funding research by emerging scientists.

Investments in basic and clinical research are the foundation of future therapies and cures. Yet funding increases for the National Institutes of Health (NIH) have not kept pace with rising inflation, compromising our nation's ability to fund promising scientists. This trend is likely a contributing force behind the historic low application success rates and all-time high average age an investigator receives their first research grant.

Not surprisingly, these figures have a chilling effect on the number of young scientists choosing to dedicate their careers to medical research. As the brightest minds turn elsewhere, America's position as the global leader in research and innovation—and in bringing cures to patients—is compromised. ASN consequently supports congressional efforts as laid out in this section to help young, emerging scientists gain a successful start to their research careers.

The society also suggests the Committee consider directing NIH to expand the agency's loan repayment program to specifically include adult trainees who pursue bench science. ASN believes all research—basic, clinical, and translational—has equal merit and ought to be recognized as such. The current exclusion of adult trainees who pursue bench science signals that it is less important and, as a consequence, dis-incentivizes bench science.

Section 1141: Council for 21st Century Cures.

ASN believes the proposal described in Section 1141 to establish a public-private partnership to accelerate the discovery, development, and delivery in the United States of innovative cures, treatments, and preventive measures for patients has substantial promise to assist in the development and delivery of new therapies for patients. The society applauds the Committee for including the concept of the Council for 21st Century Cures in the discussion draft, and offers insights from a similar, successful public-private partnership with the FDA.

To respond to the serious and under-recognized epidemic of kidney disease in the United States, the Food and Drug Administration and the American Society of Nephrology in 2012 founded the Kidney Health Initiative (KHI)—a public-private partnership designed to create a collaborative environment in which the FDA and the greater kidney community can interact to optimize the evaluation of drugs, devices, biologics, and food products. The mission of this public-private partnership between ASN and FDA is to advance scientific understanding of the kidney health and patient safety implications of new and existing medical products and to foster development of therapies for diseases that affect the kidney by creating a collaborative environment in which FDA and the greater nephrology community can interact to optimize evaluation of drugs, devices, biologics, and food products.

Similar to the proposed Council on 21st Century Cures, the KHI membership and board of directors—which is co-chaired by an ASN member and an FDA staff person—includes the breadth of stakeholders, including patient, health professional, pharmaceutical, device, and dialysis company members, as well as the Centers for Medicare and Medicaid Services (CMS), FDA, and NIH.

Current projects, driven by multi-disciplinary workgroups, focus on the development of clinical trial endpoints, assessment of patient preferences in the approval of medical devices, data standards, value and utilization of pragmatic trials, and much more. With more than 70 members and nearly a dozen active projects tackling the barriers to innovation in kidney disease underway, ASN believes that the collaborative KHI approach to fostering innovation can serve as a model for other areas of medicine where scientific advancements are needed. The society supports the proposed Council on 21st Century Cures.

Title II: Development

Section 2001: Development and Use of Patient Experience Data to Enhance Structured Risk Benefit Assessment Framework

ASN applauds the Committee for prioritizing the inclusion of patient perspectives in the regulatory approval process. The society concurs that the meaningful incorporation of patient experiences into product development and regulatory decision making for medical products is an important objective. While ensuring the safety and effectiveness of medical products remains a paramount responsibility of the Food and Drug Administration (FDA), the FDA also supports the use of patient-reported outcomes (PRO) tools and patient preference metrics. However, the lack of clarity surrounding best practices for their development and application has resulted in slow adoption of these patient-centered tools.

Given that a patient's tolerance for risks will vary based on numerous factors including the severity of the disease or condition, the stage of the chronic disease, and the availability of alternative treatment options, a need exists for another set of tools that would allow regulators to better understand how affected patients would assess the overall benefits and risks associated with a product.

As proposed in the discussion draft, the use of patient experience data and patients' willingness to accept various levels of risk based upon potential benefit are all important considerations for a framework that would facilitate the incorporation of patients' experience data into regulatory decisions. ASN also supports the concept of convening workshops for patients, representatives from advocacy groups and disease research foundations, FDA staff, and methodological experts to provide input. The society specifically encourages that representatives from health professional organizations be added to the list of attendees included in such a workshop.

Reflective of ASN's commitment to facilitating the incorporation of patient preferences into the regulatory process, the society's public-private partnership with the Food and Drug Administration (the Kidney Health Initiative (KHI) mentioned under Title I section 1141 of this letter) is confronting this topic. KHI's workshop (planned for the second half 2015) will engage kidney disease patients, in conjunction with regulators and industry, to understand their preferences and define future opportunities to develop tools that will assess benefit and risk of medical devices.

Section 2261. Protection of human subjects in research; applicability of rules.

ASN supports granting the Department of Health and Human Services Secretary more authority and flexibility to reform the Internal Review Process as laid out in this provision.

ASN specifically supports the establishment of a single Institutional Review Board (IRB) for multi-site studies. While IRBs assure that appropriate steps are taken to protect the rights and welfare of clinical trial participants, review of a multi-site study by the IRB of each participating site involves significant administrative burden in terms of IRB staff and members' time to perform duplicative reviews.

When each participating institution's IRB conducts a review, the process can take many months and significantly delay the initiation of research and patient recruitment for clinical trials. Use of single IRBs in multi-site studies, on the other hand, has been shown to decrease approval times for clinical protocols and may be more cost effective than local IRB review.

Section 2282: Encouraging Scientific Exchange at the FDA

ASN concurs that participation in or sponsorship of scientific conferences and meetings is essential to the mission of the FDA. Remaining current on the latest scientific knowledge and participating in the exchange of new findings at such conferences is vital for FDA staff. The society would strongly support provisions in the 21st Century Cures legislation that would facilitate FDA staff attendance at scientific conferences.

Title III: Delivery

Section 3021: Telehealth Services Under the Medicare Program

ASN commends the Committee for seeking input and feedback from stakeholders on telehealth opportunities in the Medicare program as part of its larger 21st Century Cures initiative. ASN shares the Energy and Commerce Bipartisan Telemedicine Member Working Group's conviction that telehealth has significant possibility to facilitate better access to care and holds great promise for improving the health and quality of life for patients nationwide.

ASN believes that patients at every stage of kidney disease—from those with early-stage CKD who may be at risk to progressing, to those who are on dialysis, to those who have received a kidney transplant—may be uniquely poised to benefit from expansion of telehealth opportunities. More than 51% of patients with kidney disease have 5 or more co-morbid conditions. Effective management of these co-morbidities is especially important to slow the progression of kidney disease as well as prevent the advancement of costly co-morbidities that are caused or worsened by kidney disease, such as hypertension. Besides improving patient outcomes, facilitating patient access to subspecialists via telehealth technologies may contribute to long term cost-savings—particularly to the Medicare ESRD Program by preventing people from requiring dialysis.

These are among the many reasons the society supports the provisions requiring specific actions of government bodies identified as critical to developing a long-term solution to adopting new technologies into our delivery system. These are a first step towards adopting new technologies in ways that promote greater quality care and fiscal integrity.

ASN also continues to support eliminating existing limitations on what qualifies as an originating site as defined under section 1834 (m). In particular, the society supports permitting patients' homes to qualify as originating sites for the provision of telehealth services. Lifting these limitations would facilitate patient access to care, eliminating the need to travel to interface with their nephrology care team—which would likely connote quality of life benefits as well as reduced expenditures for patients receiving CKD, ESRD, and transplant-related care.

ASN thanks the Committee for its interest in telehealth and for the opportunity to provide input regarding the draft legislation on advancing telehealth opportunities. ASN stands ready to answer any questions the Committee may have and looks forward to continuing to work with Committee and the Working Group in order to support thoughtful, appropriate adoption of telemedicine nationwide.

Section 3041: Continuing Medical Education for Physicians

ASN supports the provision outlined in Section 3041 that would clarify that peer-reviewed journals, journal reprints, journal supplements, and medical textbooks are excluded from the reporting requirement under the Sunshine Act.

Conclusion

Again, ASN applauds the Committee for its work on this initiative and its commitment to ensuring that the United States continues its preeminence in the discovery, development, and delivery cycle and thus, remains the world leader in innovation. The society is grateful for the opportunity to provide on the discussion draft and hopes this feedback is helpful.

Again, thank you for your time and consideration. To discuss ASN's input please contact ASN Manager of Policy and Government Affairs Rachel Meyer at meyer@asn-online.org or at (202) 640-4659.

Sincerely,

A handwritten signature in black ink, appearing to read "John R. Sedor". The signature is fluid and cursive, with the first name "John" being the most prominent.

John R. Sedor, MD, FASN
Chair, Public Policy Board
Secretary-Treasurer