

June 1, 2014

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
2125 Rayburn House Office Building
Washington, D.C. 20515

Re: 21st Century Cures: A Call to Action White Paper

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 21st Century Cures initiative and the 21st Century Cures: A Call to Action white paper. ASN commends the Committee's for its commitment to accelerating the discovery, development, and delivery of promising new treatments to patients and stands ready to collaborate to achieve this important objective.

ASN, the world's leading organization of kidney health professionals, represents nearly 15,000 health professionals 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 to care delivery.

Kidney disease affects more than 20 million Americans. There are many 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). There are not enough organs available to transplant all of these individuals. 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. Kidney disease worsens the outcomes of people with diabetes and heart disease and accounts for much of the mortality in individuals with diabetes. Recent data shows that kidney disease in diabetic patients continues to rise despite overall improvement in other outcomes with better management of blood sugar, suggesting controlling diabetes will not be sufficient to stop kidney disease.

As the Committee noted in its report, 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 over several decades.

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

Discovery

The United States' position as the global leader in basic and clinical research is the result of a strong history of leadership from the National Institutes of Health (NIH), academic medical centers, industry, and other federally-funded research agencies, and ASN shares the Committee's belief that maintaining that position is an important goal. Continuation of robust federal funding for the NIH and other federally-funded research agencies, such as the NIH, the Department of Veterans Affairs (VA), and Patient Centered Outcomes Research Institute (PCORI) is the crucial cornerstone to achieving this objective. Ensuring funding at the NIH and other research agencies is necessary not only to sustain the important ongoing work of today's scientists but also to make careers in biomedical research attractive to the next generation of investigators.

The number of U.S. medical graduates pursuing careers as physician-scientists is declining.. The percentage of physicians engaged in research as their major professional activity in the United States has decreased from a peak of 4.6 percent in 1985 to 1.8 percent in 2003, a trend that has only worsened in the last decade. Unless we continue to invest in biomedical research and make careers in this field viable, America will not only develop fewer new discoveries and cures, but also lacking the human capital to do so and defend our leadership role in the longer term.

In addition to traditional federal funding for investigator-initiated research, public-private partnerships and other creative funding mechanisms can also further the discovery process. For instance, while not a substitute for traditional investments in federal research agencies, prize competitions can serve a complementary role spurring scientific and technologic breakthroughs. Unlike traditional research funding models, competitions have the added benefit that the prize is only paid-out if a competitor wins. Moreover, competitions also typically draw competitors from outside the traditionally interested in the disease state or biomedical problem, broadening the scope of innovators and drawing on the creativity of multiple disciplines.

Promoting coordination and collaboration across federal research agencies, such as between NIH, PCORI, and the VA to ensure aligned priorities and sharing of resources and information is one strategy that can help achieve this goal. Similarly, encouraging collaboration between NIH Institute-Centers can make effective use of resources and advance understanding in areas of shared interest.

Encouraging funding mechanisms that promote efficient use of data by making datasets open and accessible for use by other investigators is another strategy to better disseminate knowledge and create opportunity for discovery.

In certain areas, a better understanding at the molecular level about what biological mechanisms trigger the onset or proliferation of a particular condition or disease exists. For instance, NIH-supported investigators recently identified that African Americans with the APOL1 gene are at substantially higher risk for kidney failure. Supporting further investigation and translational research in areas where promising genetic data exist—such as APOL1—is especially important to transform these clues into the cures of the future.

Development and Delivery

As the Committee observes in its report, the size, failure rates, and costs of conducting trials—as well as administrative and regulatory burdens—are at all-time highs. While the randomized, double-blinded, placebo-controlled model remains the gold standard, ASN believes that increasing the adoption of pragmatic clinical trial designs, and encouraging more creative and cost-efficient trial designs, is an appropriate strategy for certain types of research. While recognizing that not every trial is appropriate for alternative trial design, ASN supports exploring creative, cost-effective approaches that maintain scientific integrity and patient safety.

Importantly, movement toward novel trial designs must be inclusive, involving NIH (or other federal research agencies), industry, and FDA. CMS, which will ultimately determine coverage, and should also be included in early in considerations regarding how new products will be evaluated.

Beyond pragmatic clinical trial design, creation of new, clearly defined endpoints is an important step in making the development of new products feasible and cost-effective. In particular, ASN supports the Food and Drug Administration's (FDA) efforts on several fronts to incorporate the patient perspective in the approval process. The society encourages the Committee to prioritize this type of engagement when considering ways to accelerate treatments that are of greatest value from the patient perspective.

ASN would also like to highlight the value of its public-private partnership with the FDA, the Kidney Health Initiative (KHI). The mission of KHI is to advance scientific understanding of the kidney health and patient safety implications of new and existing medical products used for to treat other conditions 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.

Since its inception in 2012, KHI has served as a valuable forum to bring together patient groups, health professionals, and drug, device, and biologic manufacturers with FDA to identify and tackle barriers to the development of new therapeutics and ensure the safety of drugs, devices, biologics, and food products for patients with kidney disease. Nearly 70 companies and organizations joined KHI in its inaugural year, highlighting the stakeholder interest in and need for greater collaboration and partnership with the FDA.

This successful public-private partnership is a testament to the FDA's active participation with the kidney community and to the power of such collaborations to help accelerate the development of therapies and address unmet medical needs for Americans affected by kidney disease. Importantly, KHI has already identified many of the barriers to entering the kidney space and bringing new therapeutics to market, and is working with the FDA to find mechanisms to overcome these barriers. ASN believes that KHI can serve as a model for other areas of medicine that seek to catalyze innovation and attract investment in new therapies.

Conclusion

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 input now and throughout the ongoing initiative and hopes this feedback is helpful.

Again, thank you for your time and consideration. To discuss ASN's input, the Kidney Health Initiative, or any other issues related to kidney research, please contact ASN Manager of Policy and Government Affairs Rachel Meyer at meyer@asn-online.org or at (202) 640-4659.

Sincerely,

Sharon M. Moe, MD, FASN
President