

December 15, 2015

The Honorable Lamar Alexander  
Chairman  
Committee on Health, Education, Labor, and Pensions  
428 Dirksen Senate Office Building  
Washington, D.C. 20510

The Honorable Patty Murray  
Ranking Member  
Committee on Health, Education, Labor, and Pensions  
428 Dirksen Senate Office Building  
Washington, D.C. 20510

**Re: Innovation for Healthier Americans Initiative**

Dear Chairman Alexander and Ranking Member Murray:

On behalf of the American Society of Nephrology (ASN), thank you for your continued commitment to accelerating the discovery, development, and delivery of promising new treatments to patients. The society looks forward to seeing the draft Innovation for Healthier Americans bill and stands ready to collaborate with the Committee in advancing this important initiative.

ASN, the world's leading organization of kidney health professionals, represents nearly 16,000 health professionals and scientists who are dedicated to treating and studying kidney diseases and to improving the lives of the millions of patients they affect. 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.

Kidney diseases affect more than 20 million Americans. There are many unique causes of kidney diseases, but when any type of kidney disease progresses to kidney failure, patients require either dialysis or transplantation to stay alive. Currently, 650,000 Americans have complete kidney failure, called end-stage renal disease (ESRD). Because there are not enough donor kidneys for everyone who needs one, most patients with kidney failure are on dialysis. Moreover, kidney diseases disproportionately affect racial and ethnic minority populations, are associated with multiple co-morbidities including heart disease and diabetes, and are among the most costly chronic conditions in the United States.

While America's scientific leadership has yielded important treatments for some conditions, many patients with chronic diseases have few therapeutic options because the state of biomedical research and innovation for their disease is not as advanced as it should be; kidney diseases are among the conditions for which we must accelerate the pace of innovation.

Although patients with kidney failure 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 kidney failure have seen only incremental treatment improvements in recent decades.

The Innovation for Healthier Americans 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. As such, ASN offers the following comments for your consideration as the initiative moves forward.

### **Increasing Investments in NIH Innovation**

The lack of progress in treating patients with kidney diseases directly reflects the lack of adequate investment in research. Currently, NIH investments in kidney research (\$585 million) are less than 1% of total Medicare costs for patients with kidney diseases (\$80 billion). In fact, costs of care for patients with kidney failure alone—the only health condition that Medicare automatically provides coverage for regardless of age or disability—are more than NIH's entire budget (\$35 billion vs. \$30 billion annually). Despite the staggering cost, NIH investments in kidney research is one of the lowest of any of the major chronic diseases both in absolute dollars and per patient (Table 1).

While ASN commends Congress for increasing its budget in 2016, NIH funding has been relatively stagnant. NIH has the same purchasing power today as it did in 2003. As a consequence, NIH grant application success rates are at an all-time low and researchers wait longer than ever to receive their first grant.

With precision medicine and other advances in technology, the opportunities for accelerating the discovery, development, and delivery of promising new treatments to patients have never been greater. ASN supports additional investments and resources for research, especially highly innovative research at NIH to reduce the burden of disease on patients and curtail expenditures.

### **Reconsidering NIH Research Priorities**

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. ASN has recommended that NIH budgeting take into account disease burden, both prevalence and cost of care to the federal government, when setting research funding priorities.

Besides examining the cost of various diseases as a percent of the Medicare budget versus population affected, NIH could also consider examining the most costly chronic disease dyads and triads as identified in the "Chronic Conditions among Medicare Beneficiaries Chartbook" online at

<https://www.cms.gov/Research-Statistics-Data-and-Systems/Statistics-Trends-and-Reports/Chronic-Conditions/Chartbook.html>.

Moreover, ASN suggests that NIH consider prioritizing funding to study diseases with a disproportionate burden on racial and ethnic minority populations. African Americans make up approximately 13% of the United States population but account for 32% of Americans with kidney failure. Hispanics and Native Americans are twice as likely as Caucasians to develop kidney failure. Investing in science to explain the causes of these and other disparities, and to develop targeted therapies to treat and prevent them, should be a national research priority.

### **Easing NIH Travel Restrictions**

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 necessary for maintaining professional board certification.

ASN recognizes 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.

### **Supporting More High-Risk, High-Reward Research**

In addition to the traditional funding of discovery science, ASN supports the pursuit of high-risk, high-reward funding models. The private and philanthropic sectors have successfully used 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 dedicate additional funding towards prize competitions, especially in fields where innovation has been stagnant, including prevention and treatment of kidney diseases. 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.

### **Strengthening the Research Pipeline for Emerging Scientists**

Investments in basic and clinical research are the foundation of future therapies and cures. Yet funding increases for 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 and physicians 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 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 and specifically include trainees who pursue bench science focused on adults. ASN believes all research—basic, clinical, and translational—has equal merit and ought to be recognized as such. The current exclusion of bench science focused on adult research signals that it is less important and, as a consequence, disincentivizes trainees from pursuing it.

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

ASN believes public-private partnerships have significant potential to accelerate the discovery, development, and delivery in the United States of innovative cures, treatments, and preventive measures for patients. These partnerships show great promise in promoting the development and delivery of new therapies for patients. The society supports the concept of the Council for 21st Century Cures in H.R. 6. The society also offers insights from a similar, successful public-private partnership with the FDA.

In response to the serious and under-appreciated epidemic of kidney diseases in the United States, the FDA and ASN began a public-private partnership in 2012 called the Kidney Health Initiative (KHI) to create a collaborative environment in which the FDA and the greater kidney community could interact to optimize the rapid testing of new and existing drugs, devices, biologics, and food products that may help patients with kidney diseases. The mission of this public-private partnership is to advance scientific understanding of the kidney health and patient safety implications of medical products and to foster development of therapies for diseases that affect the kidney.

Similar to the Council on 21st Century Cures included in H.R. 6, the KHI membership and board of directors—co-chaired by an ASN member and an FDA staff member—represents the broad spectrum of stakeholders, including patients, health professionals, pharmaceutical, device and dialysis companies, the Centers for Medicare and Medicaid Services (CMS), and the NIH.

Current projects 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 attacking the barriers to innovation in kidney diseases, ASN believes that the collaborative KHI approach to fostering innovation can serve as a model for other areas of medicine. The society supports the proposed Council on 21st Century Cures.

## **Developing and Using Patient Experience Data**

ASN encourages the Committee to prioritize the inclusion of patient perspectives in the regulatory approval process in the Innovation for Healthier Americans legislation. The society believes 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 remain a paramount responsibility of the FDA, the agency 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.

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 encourage the addition of representatives from health professional organizations 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 FDA is addressing this topic. KHI's workshop engaged patients with kidney diseases, 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.

## **Protecting Human Subjects in Research**

ASN recommends granting the Department of Health and Human Services Secretary more authority and flexibility to reform the internal review process for ensuring the protection of human subjects in research.

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.

However, ASN is concerned about proposed changes to the Common Rule, which pertain to the use of de-identified archival and discarded biospecimens. The revisions require informed consent prior to use of these samples, which will not only increase regulatory burden and cost, but will essentially eliminate a rich source of human specimens for discovering cures.

### 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. Keeping abreast of the latest scientific knowledge and participating in the exchange of new findings at conferences is vital for FDA staff. The society strongly supports provisions that would facilitate FDA staff attendance at scientific conferences.

### Continuing Medical Education for Physicians

ASN encourages the committee to include language that would clarify that peer-reviewed journals, journal reprints, journal supplements, and medical textbooks used for educational purposes be excluded from the reporting requirement under the Sunshine Act.

Again, ASN thanks the Committee for its work on this initiative, its role in expanding NIH funding, and its commitment to ensuring that the United States continues its preeminence in the discovery, development, and delivery of new breakthrough treatments. The society appreciates the opportunity to share and hopes this feedback is helpful. To discuss this letter please contact ASN Associate Director of Policy and Government Affairs Rachel N. Meyer at [meyer@asn-online.org](mailto:meyer@asn-online.org) or (202) 640-4659.

Sincerely,



John R. Sedor, MD, FASN  
Chair, Public Policy Board

**Table 1. NIH Research Funding by Disease**

Disease	Prevalence, Millions	2014 Budget,a (Million)	% of 2014 NIH Budget	NIH Spending per Patient
HIV/AIDS	1.2b	\$3677	12%	\$3064
Cancer	14c	\$7957	27%	\$568
Heart Disease	27d	\$1645	5%	\$61
<b>Kidney Disease</b>	<b>20e</b>	<b>\$585</b>	<b>2%</b>	<b>\$29</b>

aAccording to NIH at [http://report.nih.gov/categorical\\_spending.aspx](http://report.nih.gov/categorical_spending.aspx).

bAccording to the Centers for Disease Control and Prevention (CDC) at <http://www.cdc.gov/hiv/basics/statistics.html>.

cAccording to the American Cancer Society <http://www.cancer.org/cancer/cancerbasics/cancer-prevalence>.

dAccording to CDC at <http://www.cdc.gov/nchs/fastats/heart-disease.htm>.

eAccording to CDC at [http://www.cdc.gov/diabetes/pubs/pdf/kidney\\_factsheet.pdf](http://www.cdc.gov/diabetes/pubs/pdf/kidney_factsheet.pdf).