



601 E Street, NW | Washington, DC 20049  
202-434-2277 | 1-888-OUR-AARP | 1-888-687-2277 | TTY: 1-877-434-7598  
www.aarp.org | twitter: @aarp | facebook.com/aarp | youtube.com/aarp

May 19, 2015

The Honorable Fred Upton  
Chairman  
Committee on Energy & Commerce  
U.S. House of Representatives  
Washington, DC 20515

The Honorable Frank Pallone, Jr.  
Ranking Member  
Committee on Energy & Commerce  
U.S. House of Representatives  
Washington, DC 20515

The Honorable Diana DeGette  
Committee on Energy & Commerce  
U.S. House of Representatives  
Washington, DC 20515

Dear Chairman Upton, Ranking Member Pallone, and Representative DeGette:

AARP appreciates your collective efforts on the 21st Century Cures Initiative. We have taken a strong interest in this Initiative and appreciate the work you have done over the last year to examine how the U.S. can promote greater innovation in the drug and medical device markets while also maintaining high standards of safety and effectiveness. We also appreciate your willingness to take our input into consideration as you worked to refine the discussion draft released in January. We believe the updated discussion draft released on April 29 is a much improved legislative product, and we look forward to continuing to work with you as the Committee finalizes the 21<sup>st</sup> Century Cures legislation in the coming weeks.

AARP is a nonprofit, nonpartisan organization, with a membership of nearly 38 million that helps people turn their goals and dreams into real possibilities, strengthens communities, and fights for the issues that matter most to families. As noted in our previous comments, medical innovation is important to AARP and all older Americans, who tend to use more prescription drugs and medical devices than any other segment of the population. AARP strongly believes that incentives for innovation must be appropriately balanced with ensuring that new treatments are safe and effective and that medical advances are affordable to those that need them.



AARP has reviewed the updated discussion draft and we would like to share the following comments. *Our March 2 comments on specific provisions continue to reflect AARP's positions unless otherwise noted below.*

## **General Comments**

We applaud the inclusion of critical new resources for the National Institute Health (NIH), particularly the five-year “innovation fund” with \$10 billion in mandatory funding beginning in FY 2016. However, we remain concerned that the updated draft still does not provide parallel financial resources that the Food and Drug Administration (FDA) will need to carry out the new responsibilities and activities required under this legislation. Without these additional resources, FDA will be ill-equipped to perform the new activities required by your legislation.

We also applaud the changes in the most recent draft that will help ensure that high and growing prescription drug costs are not exacerbated by unnecessary new exclusivity periods. We were extremely concerned by a number of provisions in the previous draft that would have granted additional exclusivity to a wide variety of drug products.

However, we remain concerned that the updated discussion draft could do more to ensure that consumers have access to affordable prescription drugs. We strongly urge you to maintain the important balance between access and innovation created by the Hatch-Waxman Act.

## **Title I – Discovery**

Sec. 1027 would remove the NIH’s National Center for Advancing Translational Science (NCATS) phase IIB clinical trial funding restriction. While AARP initially had some reservations about this proposal, after further review and consultation with the NIH, we believe it will allow NCATS to fund key research that might not otherwise be performed. However, AARP urges the Committee to be mindful of the balance between fostering innovation and asking taxpayers to fund research that could rightfully be performed by drug manufacturers, particularly when American taxpayers currently do not receive any remuneration from the sale of products that result from taxpayer-funded research.

Sec 1102 would enhance patient searches for ongoing trials by requiring NIH to standardize certain patient information across all trials housed in the ClinicalTrials.gov web site. AARP supports this effort and believes it will make clinical trial information more accessible to patients.

## **Title II – Development**

Sec. 2041 would require FDA to issue and periodically update guidance intended to help advance the clinical development of genetically targeted treatments. AARP supports the broad goals of this provision, which is similar to the President’s Precision Medicine Initiative.

Sec. 2062 would require the FDA to establish a program to evaluate the potential use of evidence from “clinical experience,” or data about the usage, benefits, or risks of a drug derived from sources other than randomized clinical trials such as observational studies and registries, to help support the approval of a new indication for a drug and to help support or satisfy post-approval study requirements. We remain concerned this proposal could lead drug companies to increase their efforts to encourage off-label prescribing with the goal of gaining new indications. These practices compromise patient safety and have already resulted in billions of dollars in civil and criminal fines.<sup>1</sup> Drug manufacturers that wish to profit from the increased utilization of their products should be willing to finance clinical studies of off-label uses.

Sec. 2101 would facilitate the dissemination of health care economic information to payers, formulary committees, or other similar entities. While this provision might provide helpful information to these entities, it will be important to ensure that such information is not misused, so it will be critical that it is based on competent and reliable scientific evidence. Therefore, we caution against expanding this provision further before we have greater experience with how the information is being used.

AARP is generally supportive of efforts through Sec. 2121-2122 to spur the development of new antibiotic drugs. We appreciate that the transferable exclusivity or “wild card exclusivity” program that was in the previous draft has been removed. We do have concerns that Sec. 2123, which would provide higher Medicare payments for certain new antimicrobial drugs, could be problematic. If new antimicrobial drugs are overused and/or not prescribed appropriately due to financial incentives, it could diminish their effectiveness and lead to greater antimicrobial resistance in the future.

Sec. 2102 requires FDA to issue draft guidance on how drug companies can communicate “responsible, truthful, and non-misleading scientific and medical information” not included on the label of drugs. We are concerned this provision could increase efforts by drug companies to encourage off-label use. We urge the Committee to proceed with caution on efforts to increase the dissemination of information that may not be evidence-based in nature.

Sec. 2151 provides an additional six months of monopoly protection for drugs approved for new indications to treat rare diseases or conditions. While this provision is more narrowly drafted than previous exclusivity provisions, we are concerned that the additional exclusivity could be applied to the most expensive drugs on the market, including blockbusters, at significant expense to consumers. We appreciate that the provision would only allow manufacturers to use the process once and would require manufacturers to notify FDA one year before a product is discontinued solely for commercial reasons. However, we are concerned that the provision would in essence allow companies to get more than six months of added monopoly protections if it is used in combination with other incentives that provide additional exclusivity (e.g., pediatric

---

<sup>1</sup> M. Bobelian, “J&J’s \$2.2 Billion Settlement Won’t Stop Big Pharma’s Addiction to Off-Label Sales,” *Forbes*, November 12, 2013.

use). The current language in this provision would allow incentives to in effect build off one another, which could end up extending the exclusivity of some products well beyond their initial monopoly period. We believe any provision to address the repurposing of drugs must, at a minimum, be narrowly defined, reward manufacturers only for therapies where there is no other therapeutic alternative available, and include protections against the “evergreening” of products already on the market.

Sec. 2181 would establish a process at FDA for the designation and expedited review of devices that represent breakthrough technologies with the potential to address unmet medical needs. AARP strongly urges the Committee to ensure that the final provision is written in a manner that ensures that the definition of a breakthrough device is appropriately narrow and limited to only those devices that represent a clear and demonstrable improvement over what is already on the market.

Sec. 2202 would clarify that FDA evaluations of medical devices can include scientific evidence that may include registry data, studies published in peer-reviewed journals, and data collected in countries other than the United States. Similar to Sec. 2062, we are concerned that this proposal could lead medical device companies to increase their efforts to encourage off-label use. We are also concerned that this provision could weaken the FDA’s regulatory oversight role in evaluating new medical device products for safety and effectiveness.

Sec. 2204 would change the process of government recognition of standards. AARP is concerned that this proposal inappropriately seeks to force FDA to follow standards set by outside organizations that may not be evidence-based. Further, requiring FDA to publicly respond to such standards would be an ineffective use of limited FDA resources.

Sec. 2206 would make changes to the medical device classification panel review process at FDA. AARP remains concerned this proposal seeks to make changes to advisory committee processes that are more appropriately left to the agency to determine in consultation with stakeholders under its administrative authority. It also introduces potential conflict of interest concerns by allowing individuals affiliated with for-profit entities to gain greater influence over medical device review processes.

Sec. 2243 would amend provisions governing the informed consent process for enrolling patients in medical device tests that pose no more than “minimal risk”. AARP is concerned that this provision would lead to a weakening of consumer protections required in medical device testing. It would also be difficult to determine the standard of “minimal risk” and the provision is unclear on who would be responsible for making this determination.

Sec. 2261 would enable the FDA to hire more efficiently and ensure that the agency has the staff required to ensure they keep up with the pace of innovation. AARP strongly supports this provision, which is intended to assist the FDA in recruiting and retaining

the most competitive and qualified scientific and technical experts in the field of biomedical research, clinical research evaluation, and biomedical product assessment.

### **Title III – Delivery**

Sec. 3041 would exempt certain transfers of value to physicians from reporting requirements under the Physicians Payments Sunshine Act. Specifically, the provision would no longer require drug and medical device companies to report payments made to doctors for continuing medical education sessions, peer-reviewed journals, journal reprints, journal supplements, and medical text books. AARP opposes any weakening of the Sunshine Act transparency requirements.

Sec. 3081 would make changes to the Medicare local coverage determination (LCD) process. While less problematic than the previous version of this provision, AARP remains concerned that the changes outlined under this proposal would make it considerably more burdensome for Medicare administrative contractors (MACs) to appropriately deny coverage.

Sec. 3101 would create a new technology ombudsman within Medicare. AARP is concerned this provision is unnecessary and would only provide another conduit for industry to seek preferential coverage determinations.

Section 3131 would allow seniors to better identify the out-of-pocket costs under Medicare for different treatments or services. AARP strongly supports the goal of making out-of-pocket costs more transparent to beneficiaries when they are considering different treatment options in consultation with their physician. We urge the Committee to ensure this proposal is adequately funded and that the information provided is reliable, regularly updated, and easily accessible.

Sec. 3151 would help prevent high-risk Medicare beneficiaries from abusing controlled substances. AARP is generally supportive of efforts to address the problem of prescription drug fraud and abuse in the Medicare Part D program. As previously stated, we have supported proposals to ensure that the Medicare program does not pay for fraudulent prescriptions and diverted medications. We are very encouraged the Committee has included the “lock-in” provisions from the Protecting the Integrity of Medicare Act (PIMA) legislation supported by AARP. We strongly believe any program to address prescription drug fraud and abuse in Part D must focus not only on enrollees, but also focus on prescribers and pharmacies that often contribute to fraud and abuse problems. The program must also ensure that enrollee preferences for a specific prescriber or pharmacy are given special consideration when selections are made to ensure reasonable access.

In closing, we must stress the importance of balancing access and innovation and urge the Committee to take a careful, measured approach that can gain the support of both industry and consumer advocacy groups. While we strongly support promoting the development of innovative treatments and cures, we believe it is equally important that

these treatments are safe, effective and affordable to consumers. Thank you for the opportunity to comment on the 21<sup>st</sup> Century Cures legislation. If you have any questions, please do not hesitate to contact me or Ariel Gonzalez (agonzalez@aarp.org) and KJ Hertz (khertz@aarp.org) on our Government Affairs staff or at 202-434-3770.

Sincerely,



Joyce A. Rogers  
Senior Vice President  
Government Affairs

cc:

The Honorable Joe Pitts, Chairman, Subcommittee on Health  
The Honorable Gene Green, Ranking Member, Subcommittee on Health  
All Committee on Energy & Commerce Members

May 20, 2015

The Honorable Fred Upton  
Chairman  
Committee on Energy and Commerce  
U.S. House of Representatives  
Washington, DC 20515

The Honorable Joseph Pitts  
Chairman  
Energy and Commerce Subcommittee on Health  
U.S. House of Representatives  
Washington, DC 20515

Dear Chairman Upton and Chairman Pitts,

The undersigned organizations, which share a strong commitment to promoting immunization in order to reduce rates of vaccine-preventable disease and its associated human, economic, and societal burden, would like to again share our thoughts on the immunization-related provisions of the 21<sup>st</sup> Century Cures Act, now that it has transitioned from a discussion draft to a bill marked up by the Energy and Commerce Subcommittee on Health.

We previously shared comments with you on the original discussion draft that was released on January 27, 2015, and we appreciate the fact that there were some slight alterations to the text of the immunization-related provisions in the second draft released on April 28, 2015. However, despite these small changes, we still have concerns about the continued inclusion of these provisions in the 21<sup>st</sup> Century Cures initiative.

As we noted in our earlier comments, immunization is considered one of the great public health victories of the twentieth century, when rates of a host of dreaded diseases were slashed dramatically as safe, effective vaccines were introduced. Once-feared diseases like polio, rubella, and pertussis became virtually unknown as routine vaccination cut rates to almost zero. While some of these diseases have recently resurged, this fact should only inspire us to redouble our commitment to maintaining high vaccination rates.

The process of developing, approving, and recommending vaccines for use among the general public is a carefully calibrated system designed to explore the safety and efficacy of immunizations as thoroughly as possible before widespread use occurs. Recommendations on the use of vaccines for the public are considered with great care by all parties involved, because they may have life-or-death consequences for some Americans. The decision whether to recommend a vaccine for universal, limited use, or optional use is undertaken through a well-established system that seeks the best possible public health outcome.

This system involves a number of steps, some of which may be lengthy, as vaccines are developed and tested in target populations by manufacturers before being submitted to the Food and Drug Administration (FDA) for licensure. After licensure, the Centers for Disease Control and Prevention's (CDC) Advisory Committee on Immunization Practices (ACIP) considers whether to recommend the vaccine for use in broad or specific populations, and also recommends any limitations or exceptions. Once the CDC Director accepts or rejects the ACIP's recommendations, the annual childhood and adult immunization schedules are compiled and published. Key health provider associations, including the American Academy of Pediatrics,

the American Academy of Family Physicians, and the American College of Obstetricians and Gynecologists, endorse the schedules and disseminate them to their membership.

Recognizing the well-established, deliberate, methodical nature of this system, we again feel the need to express our concerns about provisions in the legislation that could disrupt this balance by imposing rigid requirements and deadlines for action. It is still unclear whether the Committee has identified a particular issue or problem these provisions are intended to address. In the absence of such an issue, however, we would urge tremendous caution in pursuing changes that could introduce instability or the appearance of impropriety into the existing successful framework.

### ***Rigid Deadlines for ACIP Recommendations Are Inadvisable***

Section 4041 of the original discussion draft would have required the establishment of “standard timelines” for the ACIP to “consider and make recommendations with respect to the route of administration, dosage, and frequency of administration of vaccines for specified populations.” Furthermore, the draft directed that if the ACIP does not make a recommendation within 120 days of licensure, a manufacturer could submit a request that would then require the ACIP to draft and vote on a recommendation within 60 days of receipt of that request.

Although the new section 2141 in the introduced legislation removed the 120 days of licensure deadline, as well as the requirement for ACIP to draft and vote on a recommendation within 60 days of receipt of that request, the new language requires the Director, upon the licensure of any vaccine or any new indication for a vaccine, to direct ACIP to consider the use of the vaccine at its next meeting, and still allows the “sponsor of the vaccine” to request an expedited review if there is no recommendation made at ACIP’s first regularly scheduled meeting after the licensure of the vaccine or any new indication for the vaccine. This new language could actually require ACIP to review the vaccine sooner than the previous language required, as ACIP meets three times a year, and depending on the date a vaccine is licensed, this could require ACIP to review the vaccine in an extremely short time period.

As mentioned in our earlier comments, it is customary for the ACIP to receive regular updates, often over a year or more, regarding ongoing research studies on new and improved vaccines. In general, the ACIP takes up vaccine recommendations for a vote as quickly as possible after vital data and evidence have been made available. When a vote does not occur promptly, it is usually either because the ACIP is still awaiting important data, or the relevant Work Group has found such data unpersuasive and has therefore not developed a draft recommendation for use.

The imposition of a “standard timeline” for the ACIP to consider a vaccine at the next scheduled meeting would fail to recognize the fact that data is sometimes not forthcoming during those time periods, and could force the ACIP to take votes based on incomplete information. In those situations, it seems logical to assume that the body would err on the side of caution and not recommend a vaccine for wider use. This could actually delay the availability of important vaccines to those who would benefit from them.

In addition, the ACIP frequently reviews data related not only to the specific groups for whom the vaccine was licensed by FDA, but also other relevant or vulnerable groups. For example, even though a vaccine may be licensed for all children of a certain age, the ACIP may review its use in immunocompromised children and make a separate recommendation. Similarly, both influenza and pertussis vaccines are licensed for adults, but the ACIP makes separate, specific recommendations for their use in pregnant women. The ACIP may also take several votes on one vaccine over time to refine their recommendations as new evidence becomes available. The establishment of deadlines fails to recognize the complex and often iterative nature of evidence review.

Finally, the establishment of deadlines fails to recognize the fact that not every safe, effective vaccine should be recommended for population-based use. For example, it would be possible for a manufacturer to develop a vaccine for a common health issue that does not present a public health threat. Despite the fact that such a vaccine might be safe, effective, and even in great demand, the lack of a public health burden would fail to meet the standard for ACIP consideration. Once again, deadlines would add burden without benefit.

### ***Transparency Must Be Balanced with Protecting the Integrity of the Recommendation Process***

Mirroring our apprehension regarding Section 4042 of the discussion draft, “Review of Transparency and Consistency of ACIP Recommendation Process,” we are still concerned that Section 2142, now titled “Review of Processes and Consistency of ACIP Recommendations,” could still have unintended consequences for important aspects of the ACIP review process with regard to both transparency and consistency of recommendations. Although references to “transparency” were removed in the new language, and the deadline for the report was extended from 1 year to 18 months after the enactment of the bill, the effect of the directive in Section 2142 to review the ACIP process is effectively the same.

The ACIP currently operates in an atmosphere of considerable transparency. Its meetings are open to the public and webcast; meeting materials are posted online in advance and after meetings; public input is actively welcomed at multiple points in every meeting; and presentations are frequently delivered by industry representatives about studies and data. Work Groups receive and utilize special presentations and material submitted by the public and industry.

At the same time, however, it is vitally important that the ACIP be free of either the appearance or the actuality of undue influence by any party. For example, interested parties are strongly discouraged from contacting ACIP members individually on ACIP business. Furthermore, due to the very strong possibility that advance information about the likelihood of an ACIP recommendation could influence markets and other economic interests, certain discussions – particularly the candid conversations that take place within Work Groups -- take place with the protection of confidentiality. Key information is released publicly at predictable junctures, and votes take place solely at open meetings. We are concerned that Section 2142 could disrupt this careful balance by introducing new opportunities for either the appearance or actual exercise of undue influence.

Like Section 4042 of the original discussion draft, Section 2142 in the introduced legislation would also require a review of the consistency of criteria used by ACIP to evaluate new and existing vaccines, including the Grading of Recommendations, Assessment, Development, and Evaluation (GRADE) approach to reviewing evidence. The development of consistent criteria to evaluate vaccines would be significantly hampered by the fact that vaccines may involve very different target populations, aspects of the immune system, public health burden, quality of data, and other factors. To illustrate, ACIP is called upon to evaluate vaccines for diseases that have a moderate impact on a large population as well as those that have a severe impact on a very small population. The effectiveness of vaccines may vary, as well as the degree and quality of data involved. An attempt to impose a cookie-cutter approach on vaccine evaluation would risk forcing the ACIP to give inappropriate weight to various factors, depending on the vaccine and disease involved.

### ***Congress Should Not Direct CDC Interaction with Vaccine Manufacturers***

Finally, Section 4044 of the discussion draft, “Meetings Between CDC and Vaccine Developers,” has now been labeled Section 2143, but still requires that CDC meet with vaccine industry officials within certain timeframes (90 days in the original discussion draft and 120 days in the introduced legislation), provide specific, detailed information, and “promptly notify” the vaccine developer any time the agency becomes aware of changes to any information provided in such a meeting, including cases where “the change may have implications for the vaccine developer’s vaccine research and development.”

Our concerns over this section did not change with the updated language in the introduced legislation. This section still has any number of troubling implications for the integrity of CDC’s work around immunizations. The requirement that CDC respond to a meeting request within a rigid deadline, whether it is 90 days or 120 days, could still divert precious resources from other, more urgent public health needs. The mandate for CDC to provide specific, detailed information to industry officials still raises any number of questions: Should CDC be responsible for packaging publicly available information for industry? If CDC has access to non-public or preliminary information or data, must that be shared? Is it CDC’s responsibility to track industry interests in order to be able to determine when a change in data or evidence may have “implications” for a manufacturer’s product in development? Finally, it would appear impractical for CDC to update every manufacturer in the wake of every meeting about “any change” to relevant data; for example, disease tracking and prevalence data is updated sometimes as often as weekly, and it is unclear why the public reporting of such data is insufficient to satisfy vaccine manufacturers’ needs.

While we appreciate the minor changes that were made to the sections regarding ACIP, our concerns have not been ameliorated with the new language and we still urge tremendous caution in pursuing changes that could introduce instability or the appearance of impropriety into the existing successful framework.

Again, we deeply appreciate this opportunity to express our views regarding the immunization provisions in the 21<sup>st</sup> Century Cures Act. We look forward to working with you to ensure that this legislation will promote the timely development and approval of safe, effective vaccines for

all Americans. If we can be of further assistance, please contact James Gelfand at the March of Dimes at 202-659-1800 or Pat Johnson at the American Academy of Pediatrics at 202-347-8600.

Sincerely,

American Academy of Pediatrics  
American College Health Association  
American Congress of Obstetricians and Gynecologists  
American College of Preventive Medicine  
March of Dimes  
National Association of County and City Health Officials  
National Association of Pediatric Nurse Practitioners  
National Foundation for Infectious Diseases  
Pediatric Infectious Diseases Society  
Voices for Vaccines



May 19, 2015

The Honorable Fred Upton  
Chairman  
House Energy & Commerce Committee

The Honorable Frank Pallone  
Ranking Member  
House Energy & Commerce Committee

Dear Chairman Upton and Ranking Member Pallone,

On behalf of the Advanced Medical Technology Association (AdvaMed) and the nearly two million men and women whose jobs are supported by the medical technology industry, I am writing to express our support for the 21<sup>st</sup> Century Cures Act. Our industry shares your goal of accelerating the discovery, development and delivery of cures for patients, and believes that your legislation holds the promise to strengthen the American innovation ecosystem.

The medical technology industry is central to the development of technologies and diagnostics that will provide the life-saving and life-enhancing treatments of the future. Patient access to advanced medical technology generates efficiencies and cost savings for the health care system, and improves the quality of patient care. Between 1980 and 2010, advanced medical technology helped cut the number of days people spent in hospitals by more than half and add five years to U.S. life expectancy while reducing fatalities from heart disease and stroke by more than half.

The impact of medical technology on economic growth and competitiveness goes well beyond the jobs and economic activity associated with industry R&D and manufacturing. A recent study by the Milken Foundation examined four diseases and a limited number of technologies used to treat those diseases. It found significant increases in labor force participation and productivity directly attributable to the technologies' contribution to reducing the burden of illness. The study showed billions of dollars in expanded GDP as a result of treating these four disease states alone.

While the gains in health over recent decades have been impressive, past progress pales compared to future opportunities. In this century of the life sciences, technological advances driven by fundamental advances in knowledge of human biology and continued progress in computing, communications, materials science, physics and engineering can be expected to fuel creation of new and better medical technology products—if there is a sound innovation ecosystem supporting not only continued scientific progress but the translation of scientific advances into better health.

But the innovation ecosystem that supports our industry is severely stressed. Venture capital flowing to the medical device sector is both an essential generator of future



**AdvaMed**

Advanced Medical Technology Association

progress and an index of the attractiveness of investing in the development of new treatments and cures. Many of the true breakthrough therapies and diagnostics in the medical technology industry flow from venture funded start-ups. Venture investment in medical technology declined by 42 percent between 2007 and 2013. Even more ominous is the decline in investment for start-up companies at the earliest stage — the seed corn for the next generation of treatments and cures. First time funding for medical technology start-ups dropped by almost three-quarters over the same period.

Policy improvements are essential if America is to retain its world leadership and the potential for medical progress in this century of the life sciences is to be fulfilled. The 21<sup>st</sup> Century Cures Act includes a number of proposals designed to strengthen the innovation ecosystem and support the development of life-saving, life-enhancing medical technology. This includes key improvements to FDA's premarket program for medical devices – most significantly the establishment of an expedited pathway for breakthrough, innovative technologies – which will increase the efficiency, predictability and transparency of the agency's review process and improve patient access to the best in medical progress. The bill also provides increased NIH funding which will help strengthen our R&D infrastructure.

These provisions are an important and significant first step to strengthen our innovation ecosystem. We understand that the committee is still working to address additional items that may be added to offset the budgetary implications of the legislation. We look forward to reviewing these additions and evaluating their impact on our perspective on the legislation.

We support your legislative efforts, and look forward to working with the committee as the 21<sup>st</sup> Century Cures Act moves forward. We also look forward to further dialogue on how we can address other weak points in the innovation ecosystem, including the Medicare coverage delays and payment challenges that are discouraging investment in new medical innovations.

Thank you for your efforts to encourage innovation in medical technology.

Sincerely,



Stephen J. Ubl  
President and CEO  
AdvaMed

Cc: Members of the House Energy & Commerce Committee



8229 Boone Boulevard, Suite 260, Vienna VA 22182 • 800.878.4403 • [www.aanma.org](http://www.aanma.org)

April 22, 2015

The Honorable Fred Upton  
Chairman  
Energy & Commerce Committee  
U.S. House of Representatives  
Washington, DC 20515

Congresswoman Diana DeGette  
U.S. House of Representatives  
2368 Rayburn House Office Building  
Washington, DC 20515

Dear Chairman Upton and Congresswoman DeGette:

Allergy & Asthma Network, a nonprofit patient advocacy and education organization dedicated to eliminating death and suffering from asthma, allergies and related conditions, supports advancing telehealth opportunities in Medicare through the 21<sup>st</sup> Century Cures initiative.

Telehealth is increasingly vital to our healthcare delivery system. The rapid increase in patients with chronic conditions is putting an immense strain on our healthcare system in the United States. To simply tackle these problems with more financial resources is not an option anymore. We concur with the Committee that there is an urgent need to develop services that are more flexible, patient-centered and cost-effective.

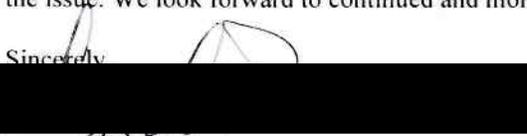
Allergy & Asthma Network strongly agrees with your goal of expanding coverage of telehealth services in Medicare. There is a growing body of evidence that telehealth increases quality, improves patient satisfaction and reduces costs. However, we are concerned that the requirement for the Medicare actuary to certify telehealth cost neutrality for specific services would be difficult for the U.S. Department of Health and Human Services (HHS) to operationalize.

As an alternative to the discussion draft framework, we urge the Committee to consider an approach that gives the Secretary authority to expand or constrict telehealth services according to their budget impact, but rests the power to approve Medicare beneficiaries' access to telehealth and remote patient monitoring to Congress.

To ensure budget neutrality, we further encourage the Committee to consider a proposal set forth by the Alliance for Connected Care that creates an Advisory Committee to the Secretary with expertise in remote patient monitoring and telehealth. The Advisory Committee would advise the Secretary and the Centers for Medicare/Medicaid Services (CMS) on what services should be covered based on evidence of cost savings or cost neutrality.

Allergy & Asthma Network commends your leadership and the other members of the Energy and Commerce Committee 21<sup>st</sup> Century Cures working group for recognizing the need to modernize Medicare's approach to telehealth and seeking stakeholder comment on the issue. We look forward to continued and more detailed discussions on this important topic in the coming months.

Sincerely,

  
Tonya Winders  
President & CEO  
Allergy & Asthma Network



May 18, 2015

The Honorable Fred Upton  
Chairman  
U.S. House of Representatives  
Committee on Energy and Commerce  
2125 Rayburn House Office Building  
Washington, DC 20515

The Honorable Diana DeGette  
U.S. House of Representatives  
2368 Rayburn House Office Building  
Washington, DC 20515

Dear Chairman Upton and Representative DeGette,

The Alliance for Aging Research, [www.agingresearch.org](http://www.agingresearch.org), is the leading non-profit organization dedicated to accelerating the pace of scientific discoveries and their application to improve the experience of aging and health. On behalf of the Alliance, we applaud your work to arrive at a bill that, if fully funded, could improve various aspects of the biomedical research and regulatory approval process. We are encouraged by recent reports of your commitment to ensuring our federal agencies have the resources they need to carry out the increased responsibilities included in the 21<sup>st</sup> Century Cures Act. We urge you to include full funding authorizations for both the National Institutes of Health (NIH) and the Food and Drug Administration (FDA) in the bill that will be considered by the full House Energy and Commerce Committee later this week.

We are pleased to support the most recent version of the 21 Century Cures Act approved by the House Energy and Commerce Subcommittee on Health. We would like to take this opportunity to acknowledge several provisions in the bill and to share our thoughts on how this important initiative can better serve patients in need of new treatments and medical technologies.

## **TITLE I- DISCOVERY**

### **Subtitle A- National Institutes of Health Funding**

The Alliance for Aging Research commends the committee for reauthorizing the NIH and for including an additional \$10 billion in funding over five years. As you are aware, NIH sponsors researchers in every single state, and about 80 percent of its budget goes to fund 300,000 researchers around the world. The agency also supports training programs to increase the country's research capacity, employs about 6,000 scientists at its own labs and runs the world's largest hospital completely dedicated to clinical research. Since the doubling of NIH's base budget between fiscal years 1998-2003, the agency's funding has stagnated at around \$30 billion

and is losing ground to inflation, particularly to the high rate of medical inflation. The deliberative process you have undertaken to arrive at the Innovation Fund has helped rectify this problem.

The mandatory funding the Innovation Fund provides, coupled with meaningful year over year growth in the Institutes' baseline budget, will make a profoundly positive difference in the discovery landscape. This will push the boundaries of knowledge wider to reveal new targets for the development and delivery of transformative drugs, biologics and medical devices. The Alliance urges you to continue to champion the NIH Innovation Fund.

### **Subtitle B—National Institutes of Health Planning and Administration**

Section 1021 would require the Director of the NIH to develop a 5-year “biomedical research strategic investment plan” to make funding allocation decisions. We support the committee’s interest in identifying strategic focus areas that consider “the return on investment to the United States public.”

We would encourage the committee to extend further in its language and to ask the NIH to specifically consider costs to public healthcare programs (i.e. Medicare and Medicaid) as part of its return on investment and subsequent prioritization for research investment in specific conditions. For example, the costs of care for Alzheimer’s disease are enormous—in 2015 Alzheimer’s disease and other dementias will cost the nation \$226 billion, with Medicare and Medicaid paying 68 percent of the costs. Without a treatment, costs are projected to increase to more than \$1.1 trillion in 2050. Yet, federal funding for Alzheimer’s disease and related dementias lags behind investment in other major disease areas by a factor of two to six.

Economic burden on public healthcare programs is not currently considered as part of the existing strategic planning process at NIH. An [April 2014 GAO study](#) found that the five selected ICs—awarding the largest amount of research funding—that it reviewed did so considering similar factors and using various priority-setting approaches. In priority setting, IC officials reported taking into consideration scientific needs and opportunities, gaps in funded research, the burden of disease in a population, and public health need, such as an emerging public health threat like influenza that needs to be addressed.

Section 1022 would create a five-year term for each institute and center director at the NIH (up from four years in the original bill draft). The Alliance opposes this provision and supports its removal. Currently, the directorships of NIH Institutes and Centers (i.e., other than that of the National Cancer Institute, which is appointed by the President under the 1971 National Cancer Act) are filled by the NIH Director. These directorships 1) do not require a Presidential appointment or Congressional approval and 2) do not have terms for their appointment. We believe that the introduction of terms will distract directors with campaigning and will encourage jockeying among colleagues who should be spending their time managing research programs. The NIH Director is already allowed to hire and fire, and IC Directors positions should not be further politicized.

In Section 1022, we appreciate the changes made to the R-series grant review process and support the inclusion of this language in the final bill.

### **Subtitle C-Supporting Young Emerging Scientists**

As in previous comments on 21<sup>st</sup> Century Cures Act, the Alliance supports mechanisms that encourage careers in medical research. We appreciate the changes made to this bill from previous drafts that adjust loan repayment terms for young emerging scientists as opposed to redirecting funds from the Public Health Service Evaluation Set Aside, also known as the “evaluation tap.”

## **TITLE II-DEVELOPMENT**

### **Subtitle A- Patient-Focused Drug Development**

The Alliance agrees that there is a need to develop and use patient experience data to improve the drug development process and to enhance structured risk-benefit assessments. We appreciate that the current bill requires a more scientific and systematic approach to gathering patient experience data. Thank you for defining what individuals and groups are intended to collect this data. The definition clarifies what was meant by an “entity” in the previous draft bill. We support patients, caregivers, patient advocacy groups, and members of the scientific and medical research communities being acknowledged as equal agents capable of conducting this type of research.

We still feel that the loosely structured FDA Patient-Focused Drug Development meetings, established during the fifth reauthorization of the Prescription Drug User Fee Act, have resulted in valuable resources on anecdotal experience that help inform new endpoint development, outcome measure selection in clinical trials, and benefit-risk decision making by regulators. To better contextualize the entirety of patient’s experiences with a disease, the Alliance believes that information on anecdotal experience should still be permitted, either in conjunction with or without the patient experience data framework established by Section 2001. It may be worth refining this section to state that the proposed structured framework is intended to compliment information gathered through unstructured interactions with patients, their caregivers and patient advocacy groups.

### **Subtitle B-Qualification and Use of Drug Development Tools**

The Alliance supports the authorization of \$10 million annually, from fiscal year 2016-2020, to support the qualification and use of drug development tools. As you know, FDA established a process several years ago through which drug development tools like biomarkers, outcome assessments and other endpoints could be qualified for a specific use and then incorporated into clinical trials. Section 2041 intends to build on the existing qualification process. Through this process a company, group of companies or other organization could opt to work with regulators in a collaborative fashion to reduce the cost of developing these tools individually and produce a tool that once qualified became publicly available. This process has been slow to result in qualified tools due in part to the slow pace of science and a lack of resources available at the

FDA. Section 2014 intends to enhance the existing process for qualification and alleviate one of the two main factors for the resulting delay in qualification.

We would like to note that even with an enhanced qualification process in place, there is still the ability for a company, group of companies or other organization to talk directly with the FDA's medical product review divisions on the use of unqualified biomarkers and unqualified endpoints in specific clinical trials. FDA frequently approves the use of unqualified biomarkers and endpoints in trials and unqualified tools served as the basis of many drug approvals. The FDA's Office of New Drugs, medical product review divisions, and the Study Endpoints and Labeling Division should retain the flexibility to decide on the appropriate use of unqualified drug development tools for the purposes of expediting clinical trials.

### **Subtitle D-Modern Trial Design and Evidence Development**

Should funding authorization accompany Section 2061, the Alliance supports the proposed FDA public meeting on broader application of Bayesian statistics and adaptive trial designs. Such a meeting including diverse stakeholders will help to foster a dialogue on the importance of more modern clinical trial infrastructure and uncover possible limitations to incorporating these methods in clinical trials for specific diseases. This would also provide a venue for discussing opportunities for additional research on how best to pursue future directions for adaptive clinical trials. We understand the desire for final guidance in this area; however FDA should have the option to operate under draft guidance, particularly if there is a lack of consensus on the best path forward following the public meeting. We support the call for FDA to update its draft guidance but we suggest removing the requirement to finalize guidance within 18 months.

Thank you for the changes made to the bill from previous drafts on the issue of utilizing evidence from clinical experience to support regulatory decisions pre- and post- approval. We were pleased to see that Section 2062 now calls for the development of a draft framework identifying available sources of clinical experience data, gaps in current data collection activities, current standards and methodologies for clinical experience data collection, and opportunities for the development of pilot programs. We support this moderate approach and believe that it will allow FDA to play a constructive role in ensuring that a future program incorporating real world, clinical experience evidence is well-designed.

### **Subtitle G-Antibiotic Drug Development**

We support sections 2121-2123 for the purposes of addressing the growing crisis of antibiotic resistance in this country. We feel that these sections provide a pathway for needed antibacterial and antifungal drugs to be approved by the FDA for use in limited populations of patients who are vulnerable and unresponsive to other treatments. We know that because older adults are most likely to contract resistant infections, they will benefit from these provisions. We appreciate the added requirements in the bill for monitoring the use of these products and the increased emphasis on making information publicly available regarding trends in resistance and ensuring appropriate stewardship. Safeguards like these can reduce inappropriate off-label use.

### **Subtitle L-Priority Review for Breakthrough Devices**

We believe timely access to cutting-edge medical devices is as important to the health and independence of the older population as access to new drugs and biologics. We continue to support Section 2201 which would establish a priority review process for medical devices. We continue to support the establishment of this process because FDA's Center for Devices and Radiological Health (CDRH) has already taken steps to create the structure for a voluntary program that would expedite access for pre-market approval of devices intended for life-threatening illnesses and for areas of unmet need. We are encouraged that the proposed priority review process in Section 2201 builds upon the work done by CDRH to increase flexibility in device approvals and enhance the level of communication with the developers of medical devices. Since the amount of resources necessary to conduct expedited reviews of new products will increase, we believe that additional authorization of funding is necessary and should be included in the final 21<sup>st</sup> Century Cures Act.

### **Subtitle P-Improving Scientific Expertise and Outreach at FDA**

We are gratified that you and your colleagues agree that for the FDA to be effective it must be populated with highly capable staff that is constantly up to date on new scientific knowledge and developments. The Alliance fully supports inclusion of Section 2281 that addresses FDA's ability to hire and retain qualified scientific and technical experts. This section will allow the FDA to more quickly recruit professionals in the field of engineering, bioinformatics and other emerging fields so that they are able to keep pace with innovation in the private sector. It will also ensure that FDA can provide competitive wages for employees with highly specialized skills. We appreciate that Section 2281 now includes language promoting FDA participation in and sponsorship of scientific conferences and meetings.

## **TITLE III-DELIVERY**

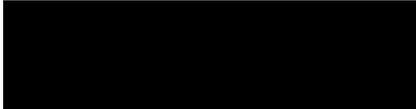
### **Subtitle H- Medicare Part D Patient Safety and Drug Abuse Prevention**

The Alliance opposes the inclusion of Section 3141 in the final version of the bill. We do not believe Section 3141 provides a balanced approach toward addressing the problem of prescription drug abuse in the United States.

Around 100 million Americans live with persistent pain - more Americans than those who are affected by diabetes, heart disease, and cancer combined. Surgery is a common cause of persistent pain. According to the National Hospital Discharge Survey, adults age 65 and older are 2.6 times more likely to have surgery than those ages 45-64. The Centers for Disease Control and Prevention found that around half of adults age 65 and older have been diagnosed with arthritis, another common cause of persistent pain. Section 3141 of this bill has the potential to inappropriately label legitimate pain sufferers as at-risk of abuse and limit their access to needed pain relief by restricting where they can fill their prescriptions. This section should not be included in a bill aimed at providing needed treatments and technologies to patients in need.

Chairman Upton and Congresswoman DeGette, thank you for your leadership on behalf of patients. We look forward to continuing our support for your efforts as the 21<sup>st</sup> Century Cures Act advances. If you have any questions or would like additional information, please do not hesitate to contact us at (202) 293-2856 or via email ([speschin@agingresearch.org](mailto:speschin@agingresearch.org) and [cbens@agingresearch.org](mailto:cbens@agingresearch.org)).

Sincerely,



Susan Peschin, MHS  
President & CEO



Cynthia Bens  
Vice President, Public Policy

**Public Policy Division**  
1212 New York Ave NW  
Suite 800  
Washington, DC 20005

202.393.7737 **p**  
866.865.0270 **f**  
www.alz.org



May 12, 2015

The Honorable Fred Upton  
Chairman  
House Energy and Commerce Committee  
2125 Rayburn House Office Building  
Washington, DC 20515

The Honorable Frank Pallone  
Ranking Member  
House Energy and Commerce Committee  
2125 Rayburn House Office Building  
Washington, DC 20515

Re: 21st Century Cures Act

Dear Chairman Upton and Ranking Member Pallone:

The Alzheimer's Association appreciates the opportunity to comment on the latest draft of the 21st Century Cures Act and applauds you both for your leadership in furthering the 21st Century Cures Initiative. The Association also recognizes the many Representatives who have contributed to this bill and are grateful for the opportunity to provide feedback.

Founded in 1980, the Alzheimer's Association is the world's leading voluntary health organization in Alzheimer's care, support and research. Our mission is to eliminate Alzheimer's disease and other dementias through the advancement of research, and as the world's largest nonprofit funder of Alzheimer's research, the Association is committed to accelerating progress of new treatments, preventions and, ultimately, a cure. Through our funded projects and partnerships, we have been part of every major research advancement over the past 30 years.

No single organization can surmount a challenge as great as Alzheimer's. To help achieve our vision of a world without Alzheimer's, the Association partners with key government, industry and academic stakeholders in the global race to end Alzheimer's. We believe in the value of collaboration and work toward the day when we will have disease-modifying treatments, preventive strategies and gold-standard care for all people affected by Alzheimer's disease.

### **Promoting Patient and Caregiver Engagement in Drug Development**

The Association applauds the Committee for maintaining the provision Patient Focused Drug Development (PFDD; TITLE III: SUBTITLE A) in the bill. The Association agrees that it is crucial to include the patient perspective in such areas as risks and benefits, targeted endpoints, and meaningful outcomes, and thus supports the enhancement of the PFDD program. With a disease like Alzheimer's, it is important to also include the perspective of care partners as well as the individual with the disease. The Association looks forward to working with the Food and Drug Administration (FDA) through the public comment period and at the public workshop on this important topic.

### **Clinical Trial Modernization**

The National Plan calls for the National Institutes of Health (NIH) to identify ways to compress the time between target identification and release of pharmacological treatments. There is evidence that a single Institutional Review Board (IRB) for multi-site studies can lead to enhanced protections for patients through increased accountability, a decrease in conflicts of interest, and improved efficiency through a refocusing of resources. These benefits plus the acceleration of the pace of research is particularly important to individuals affected by Alzheimer's disease and other dementias.

The Alzheimer's research community overwhelmingly supports the concept of a centralized IRB (TITLE II: SUBTITLE N), as have participants in several expert think tank and strategy meetings, including the 2012 Alzheimer's Disease Research Summit and meetings of the Advisory Council on Alzheimer's Research, Care, and Services. Additionally, the Association supports the inclusion of modern trial design and evidence development (TITLE II: SUBTITLE D).

### **Data Sharing**

Establishing a 21st century data sharing framework for public research will help accelerate the development of new medical technologies and advance breakthroughs (TITLE I: SUBTITLE F). The Association has developed the Global Alzheimer's Association Interactive Network (GAAIN) to provide researchers around the globe with access to a vast repository of Alzheimer's research data. GAAIN is a global hub for AD research data that allows researchers to search across multiple data sources instantly and contact these data partners directly for data.

GAAIN aggregates information about our partners' data and shares with researchers without infringing upon data partner data sharing policies and regulations. Data partners always remain in control of their data. It is the first global big data initiative in Alzheimer's disease research and serves as a benchmark for computational research in other complex diseases. The Association supports efforts to facilitate data sharing and hopes the Committee will look to GAAIN as a successful example

### **Validation and Qualification of Biomarkers**

Identifying additional partnership opportunities with the private sector and facilitating collaborative efforts to enhance identification of risk factors and early biomarkers is a key action item in the National Plan to Address Alzheimer's Disease (National Plan). The surrogate endpoint qualification and utilization that was included in the discussion draft, (TITLE I: SUBTITLE B), not only would have established a predictable, transparent process for FDA's consideration and qualification of endpoints, but also allows FDA to use private-public partnerships to qualify other types of biomarkers.

The Association is disappointed that this section did not make it into the draft as this initiative mirrors efforts by the Association that have been called upon by the National Plan. Since 2005, the Association has partnered with the National Institute on Aging, the National Institute of Bioimaging and Bioengineering, the National Institute of Mental Health, the National Institute of Neurological Disorders and Stroke, the National Institute of Nursing Research and the National Institute on Drug Abuse on the Alzheimer's Disease Neuroimaging Initiative (ADNI.) ADNI seeks to find more sensitive and accurate methods to detect Alzheimer's disease at earlier stages and mark its progress through biomarkers. Partnerships like ADNI have made significant inroads into this complex disease and the Association supports these efforts by the Committee.

The Association appreciates the steadfast support of the Committee and the great endeavor in which they are engaged. We look forward to continuing to work with the Committee in order to address the Alzheimer's crisis and hope that the Association will be called upon for our expertise in this area. If you have any questions or need further information please contact Rachel Conant at [rconant@alz.org](mailto:rconant@alz.org) or 202-638-7121.

Sincerely,

A large black rectangular redaction box covering the signature area.

Robert Egge  
Executive Vice President, Government Affairs



THE BRAINS BEHIND SAVING YOURS.™

**CONTACT:**

Erin Heintz, 202.638.7040; [eheintz@alz.org](mailto:eheintz@alz.org)

Alzheimer's Association media line, 312.335.4078; [media@alz.org](mailto:media@alz.org)

**Alzheimer's Association Applauds 21<sup>st</sup> Century Cures Legislation;  
Calls on Reaffirmed Commitment to 2025 Goal Outlined in National Alzheimer's Plan**

*Alzheimer's Stands Alone as the Only Leading Cause of Death in the U.S.  
Without a Way to Prevent, Cure or Even Slow Its Progression*

**WASHINGTON, D.C., May 14, 2015** – Robert Egge, executive vice president of Government Affairs for the Alzheimer's Association, earlier today commented on the draft of the 21<sup>st</sup> Century CURES Act legislation scheduled for markup by the House Energy and Commerce Committee. Egge applauded provisions to address the Alzheimer's epidemic in the current legislation, but encouraged vigilance for other important priorities in the *National Plan to Address Alzheimer's Disease*, adopted in 2012 at the direction of the 2011 National Alzheimer's Project Act.

Alzheimer's, the most expensive disease in the nation according to a NIH-funded study in the *New England Journal of Medicine*, is addressed in the draft legislation in several key sections, including Patient and Caregiver Engagement in Drug Development, Data Sharing and Clinical Trial Modernization.

Said Egge: "The Alzheimer's Association appreciates the steadfast support of the Committee, the bipartisan effort required to develop this legislation and the collaborative spirit in which the Committee has worked. Alzheimer's disease is a triple threat, with soaring prevalence, lack of treatment and enormous costs – to individuals and to our nation's federal budget – and this legislation is another step in the fight to end Alzheimer's."

The Association also underscored its support for identifying additional partnership opportunities with the private sector and facilitating collaborative efforts to enhance identification of risk factors and early biomarkers, a key action item in the national Alzheimer's plan that was not included in the 21<sup>st</sup> Century CURES draft legislation.

"The Alzheimer's Association encourages the Committee to continue to pursue the critical priorities outlined in the national Alzheimer's plan alongside its important work on the 21<sup>st</sup> Century CURES legislation. These combined efforts will go a long way to ensure that we are addressing the Alzheimer's epidemic holistically and with the urgency it requires," said Egge.

Since 2005, the Association has partnered with the National Institute on Aging, the National Institute of Bioimaging and Bioengineering, the National Institute of Mental Health, the National Institute of

Neurological Disorders and Stroke, the National Institute of Nursing Research and the National Institute on Drug Abuse on the Alzheimer's Disease Neuroimaging Initiative (ADNI.) ADNI seeks to find more sensitive and accurate methods to detect Alzheimer's disease at earlier stages and mark its progress through biomarkers.

If nothing is done to change the trajectory of Alzheimer's, as many as 16 million Americans will have Alzheimer's disease by 2050 and annual costs will exceed \$1.1 trillion, creating an enormous strain on families, the healthcare system and the federal budget. As the baby boomers age, near-term costs for caring for those with Alzheimer's will balloon with Medicare and Medicaid covering more than two-thirds of the costs for care.

Earlier this year, the Association released [\*Changing the Trajectory of Alzheimer's Disease: How a Treatment by 2025 Saves Lives and Dollars\*](#), which calculated that a treatment introduced in 2025 that delays the onset of Alzheimer's by five years would reduce the number of individuals affected by the disease by 5.7 million by mid-century and save all payers, including Medicare, Medicaid and families, more than \$220 billion within the first five years.

For more information about Alzheimer's disease, visit [alz.org](http://alz.org).

### **Alzheimer's Association®**

The Alzheimer's Association is the world's leading voluntary health organization in Alzheimer's care, support and research. Our mission is to eliminate Alzheimer's disease through the advancement of research, to provide and enhance care and support for all affected, and to reduce the risk of dementia through the promotion of brain health. Our vision is a world without Alzheimer's. For more information, visit [alz.org](http://alz.org).

###

May 13, 2015

The Honorable Fred Upton  
Chairman  
Energy & Commerce Committee  
U.S. House of Representatives  
Washington, DC 20515

The Honorable Frank Pallone  
Ranking Member  
Energy & Commerce Committee  
U.S. House of Representatives  
Washington, DC 20515

The Honorable Joe Pitts  
Chairman, Subcommittee on Health  
Energy & Commerce Committee  
U.S. House of Representatives  
Washington, DC 20515

The Honorable Gene Green  
Ranking Member, Subcommittee on Health  
Energy & Commerce Committee  
U.S. House of Representatives  
Washington, DC 20515

Dear Chairman Upton, Ranking Member Pallone, Chairman Pitts and Ranking Member Green:

The American Association for Cancer Research (AACR) is the world's first and largest scientific organization focused on every aspect of high-quality, innovative cancer research, from bench to bedside. The mission of the AACR and its more than 35,000 members in all fifty states and around the world is to prevent and cure cancer through research, education, communication, and collaboration. Our members include basic, translational and clinical researchers, physician-scientists, patient advocates and other leaders in the cancer research and care community.

We commend the House Energy & Commerce Committee for its commitment to the discovery, development, and delivery of new therapies to patients, especially those individuals who are suffering from the more than 200 diseases we call cancer. We especially thank House Energy & Commerce Committee Chairman Fred Upton and Congresswoman Diana DeGette for their bipartisan leadership of the 21<sup>st</sup> Century Cures Initiative, and we appreciate the opportunity to submit these comments in response to the second draft bill released on April 29, 2015.

### **Title I: Discovery**

#### **AACR applauds the increased funding for the National Institutes of Health (NIH)**

First and foremost, the AACR applauds the Committee for including language that would authorize increased funding for the NIH through sustained, predictable increases of \$1.5 billion per year over the next three years, and also language that would provide an additional \$10 billion in mandatory funding over the next five years through the creation of a new "NIH Innovation Fund."

We thank the Committee for making NIH funding a top priority in the bill, thereby recognizing the critical importance of NIH funded-research to improving our nation's health, sustaining our leadership in medical research, and remaining competitive in today's global information and innovation-based economy.

The AACR recognizes the federal government has an irreplaceable role in supporting medical research and believes the new provisions in the bill would effectively put the NIH back on a path

of sustained, predictable growth and begin to restore funding that has been lost over the past decade through budget stagnation and outright cuts.

### **AACR appreciates the support for agency personnel to participate in scientific meetings**

Furthermore, we appreciate the Committee's interest in ensuring that NIH and FDA staff scientists participate in scientific meetings and conferences, such as the AACR's Annual Meeting, which this year drew record attendance of more than 19,000 scientists and health professionals from around the world. Attending scientific meetings and research conferences is an important way for NIH and FDA scientific staff to stay connected with their respective communities and keep up with scientific advances. We hope the new "Sense of the Congress" language that is included in Section 1025 of the bill will help relieve some of the restrictions currently placed on agency personnel and will help facilitate the scientific collaborations that lead to breakthroughs and cures. We recommend that the Sense of the Congress language also be written to include scientific and regulatory staff from other agencies such as the Food and Drug Administration (FDA).

### **AACR is concerned about the potential micromanagement of NIH operations**

The AACR strongly supports prudent planning and management of the overall NIH budget, which is actively taking place within the Office of the Director and in each of the twenty-seven NIH Institutes and Centers. Currently each Institute and Center establishes its strategic plan based on its specific mission and scientific opportunity. In addition, NIH Director Francis Collins stated in testimony before the Senate Appropriations Subcommittee on Labor, Health and Human Services, Education and Related Agencies on April 30, 2015, that the development of an overarching NIH strategic plan already was underway. This plan, which according to Dr. Collins will be completed by the end of this year, will link to the plans of the individual Institutes and Centers.

The AACR does not support setting priorities for the NIH through statutory language, such as is outlined in Section 1021, because it limits the discretion and judgment of the scientific leadership at the agency, and in doing so, could hinder the scientific inquiry that for many years has led to breakthroughs in the understanding of many diseases, including cancer, as well as new therapies for numerous diseases and conditions. In fact, the ten "Mission Priority Areas" could be interpreted as narrow in focus and could prevent the agency from responding to exciting scientific opportunities and/or emerging health needs. Research that, on the surface, appears to be directed towards one aspect of biomedical research can lead to major advances in other areas. An example is how discoveries related to the immune system driven by research into HIV are having a major and positive impact on development of cancer immunotherapy.

The AACR strongly believes that the NIH Institutes and Centers should continue to have the flexibility to make the type and size of awards that are best suited to advance science with the ultimate goal of enhancing health and reducing the burden of diseases such as cancer. The peer review process administered by the NIH is second to none in the world, and has set a "gold standard" for the selection of the most meritorious proposals that countries around the world seek to emulate.

### **AACR is concerned with provisions that are duplicative with ongoing activities**

The AACR is concerned that the language in Subtitle B and specifically, sections 1021 and 1023 could establish programmatic redundancies that could decrease efficiency and lead to additional overlapping and duplicative activities. Such activities ultimately could take precious

resources away from what the agency does best—fund and promote the science that leads to new knowledge and discoveries. The NIH has been called the “crown jewel” of the federal government, serving as a beacon of international admiration and favorable opinion.

In addition, we believe the Biomedical Research Working Group in Section 1023 is unnecessary given that there are several advisory groups, including the National Science Board and the National Academies of Science, that already are addressing this issue. Furthermore, NIH already has three separate entities that oversee the grant proposal and submission process. The Scientific Management Review Board (SMRB), the Center for Scientific Review and the Advisory Committee to the Director all consider ways to restructure, streamline and simplify the submission of grant proposals to the NIH. It would appear that these entities collectively have the authority and ability to do what is being asked of this new Working Group.

### **AACR has concerns about the creation of a clinical trial registry and databank**

The AACR also has concerns about language included in Subtitle F, Section 1101 and Subtitle G, Section 1121 that would mandate creation of a publicly available, Clinical Trials Registry and Databank to be administered by a third party entity. Federal research agencies, including the NIH, already are required to develop plans to increase public access to research data, and there is a considerable amount of work taking place at the NIH Institutes and in the private sector to determine the best approach to collecting detailed information from patients in clinical trials. We believe it is too early to mandate, through statute, a new database, as there are additional considerations that must be taken into account, such as duplication with existing registries and privacy concerns. The AACR urges the Committee to consider an interim approach, such as a pilot project, to assist the agency in moving these important efforts forward in the most efficient and effective manner. As mentioned above, we believe that legislative language that supports NIH leaders in their ability to address exciting scientific opportunities and emerging health care needs will best advance the nation’s research agenda. We are concerned that legislatively directing a new program too soon would effectively “put the cart before the horse,” and would result in additional and unnecessary regulations that are costly and inefficient. It is important that any new language that changes oversight or regulation of research should support and facilitate the medical research ecosystem, not hamper the work of the NIH or its grantees.

### **Title II: Development**

The AACR appreciates the Committee’s detailed review of the framework of medical product approval at the Food and Drug Administration (FDA) in its commitment to ensuring improved medical products reach patients in an expedited manner.

### **AACR commends the streamlined data review and expediting patient access provisions**

The AACR commends the Committee for including provisions in Sec. 2063 to streamline data review and thereby streamline the drug development process without compromising patient safety. We are also pleased to see in Sec. 2081 “Sense of the Congress” language urging continued efforts on the part of the FDA to expedite the approval of drugs designated as “breakthrough therapies.” Many of these designations have already resulted in innovative, lifesaving therapies reaching cancer patients faster than they might have due to passage of the landmark 2012 Food and Drug Administration Safety and Innovation Act (FDASIA).

We applaud the Committee’s efforts to develop a sensible expanded access policy for investigational drugs in Sec. 2082. We respectfully request that the Committee consider means

of including creation of a streamlined, transparent, and easily-navigable process for patients and physicians seeking expanded access to unapproved drugs from the FDA and sponsors.

### **AACR supports qualification and use of drug development tools**

The AACR commends the Committee's recognition of the need to integrate advances in research into the regulatory process by establishing a framework to qualify the development of new tools. Qualification of these tools, such as biomarkers and surrogate endpoints, will expedite drug development, as we have already seen in cancer. However, we are concerned that Sec. 2021, if enacted, would require significant resources from the FDA. To date there is no new funding authorized in the draft legislation to assist with these mandatory activities that would be required of the agency in addition to the various product review related tasks that the agency must also carry out within defined user-fee designated timeframes.

The AACR and its members are pleased to see that the efforts to qualify new biomarkers and surrogate endpoints outlined in the bill will involve a transparent, public process that will be conducted in consultation with medical research consortia. We would be pleased to offer the AACR's broad scientific and clinical expertise to the FDA as the agency proceeds with these efforts.

### **AACR is concerned about the lack of resources provided to the FDA to carry out the many additional requirements that have been proposed**

As it is seeking to support the NIH through additional funding, so must the Committee consider a parallel commitment to ensuring the FDA has the resources it needs to carry out its regulatory and oversight functions, as well as recruit, develop, and retain highly qualified staff with diverse backgrounds. Advances in regulatory science should parallel advances in basic, translational and clinical science. If not, promising new medical therapies may never reach patients simply because we lack the tools to recognize their potential or outmoded evaluation methods delay or deny their approval.

In fact, this draft bill would place considerable demands on the FDA, including the requirement to issue more than 15 new guidances and hold several workshops and meetings, all within a relatively short time frame. It is imperative that the Committee include language that would authorize additional funding for the agency so that the mandated requirements in the bill can be carried out efficiently without compromising the quality of medical product reviews.

### **AACR is concerned with provisions that could hinder FDA's ability to be nimble**

Science and technology, our understanding of cancer biology and innovation in our approaches to cancer treatment are quickly evolving. The AACR agrees with the Committee that this rapidly changing environment requires flexibility and modernization of our regulatory approaches. The draft legislation seeks to address the processes by which the FDA considers and approves new therapies, and the AACR is concerned current language in some sections of Title II such as Sec. 2061 could hamper, rather than facilitate, the work of the agency. One of the hallmarks of the FDA is its ability to be flexible and employ discretionary judgment as it considers various medical product applications. This level of autonomy has allowed to agency to make risk/benefit assessments in the context of life-threatening diseases and various unmet medical needs. Thus, while we embrace the Committee's desire for a modernized regulatory framework to oversee regulation of innovative medical products and ensure their safety and efficacy, we strongly suggest that this can be achieved by allowing the agency to incorporate the most evidence-based regulatory science principles in an ongoing basis.

It is important that Congress ensure when drafting language to first “do no harm” and to provide the appropriate level of direction to the agency.

**Conclusion**

The AACR is pleased to be able to provide the Committee with its comments and commends its Members and staff for their bold efforts over the past year to put forward a proposal with the ultimate goal of accelerating the pace of cures and medical breakthroughs in the United States by ensuring that our laws are keeping pace with innovation.

As you know, cancer remains a formidable opponent. In fact, this year it is estimated that more than 1.6 million Americans will be diagnosed with cancer, and we will lose one person, every minute of every day in the United States to this devastating disease. The rate of cancer incidence is steadily increasing; therefore, a continual effort to strengthen our nation’s commitment to medical research, cancer research in particular, is critical now more than ever.

An increased investment in NIH research and training, supporting policies that promote a patient-centered, collaborative approach to cancer research and care, and optimizing our regulatory processes through a well-funded FDA to ensure the development and delivery of innovative medical products, are all required to address the current challenges in cancer research and care, as well as across all diseases.

The AACR and its more than 35,000 members commend the Committee for its commitment to funding the NIH and to an ongoing dialogue. We look forward to continuing to work with you to ensure that the NIH and FDA have the resources and tools needed to continue to spur innovation and deliver hope to patients and their family members all across our great nation and throughout the world.

Sincerely,



Jose Baselga, MD, PhD  
President



Margaret Foti, PhD, MD (hc)  
Chief Executive Officer



William S. Dalton, PhD, MD  
Chair, AACR Science Policy & Government Affairs Committee

May 22, 2015

The Honorable Fred Upton  
Chairman  
House Energy & Commerce Committee  
Washington, DC 20515

The Honorable Frank Pallone  
Ranking Member  
House Energy & Commerce Committee  
Washington, DC 20515

The Honorable Joe Pitts  
Chairman, Subcommittee on Health  
House Energy & Commerce Committee  
Washington, DC 20515

The Honorable Gene Green  
Ranking Member, Subcommittee on Health  
House Energy & Commerce Committee  
Washington, DC 20515

Dear Chairman Upton, Ranking Member Pallone, Chairman Pitts and Ranking Member Green:

On behalf of the American Association for Cancer Research (AACR) and its more than 35,000 members, we thank you for your bipartisan leadership on the 21<sup>st</sup> Century Cures bill, H.R. 6. We are grateful for your extraordinary commitment to ensuring that the National Institutes of Health (NIH) and the Food and Drug Administration (FDA) have the resources they need to carry out their respective missions in this time of unprecedented promise in medical research and innovation.

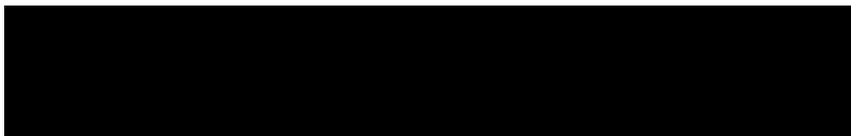
The AACR applauds both the authorization of additional funds for the NIH over the next three years through annual appropriations, and the inclusion of a mandatory NIH Innovation Fund which would provide an additional \$10 billion over five years to the agency. We also commend you for creating a Cures Innovation Fund, which would provide an additional \$550 million over 5 years to the FDA. The prioritization of funding for the NIH and FDA demonstrates your understanding of not only the critical importance of NIH funded-research to improving our nation's health, sustaining our leadership in medical research, and remaining competitive in today's global information and innovation-based economy, but also the parallel importance of the FDA receiving the resources it needs to carry out its regulatory and oversight functions, and to recruit, develop, and retain highly qualified staff with diverse backgrounds.

As H.R. 6 moves through the legislative process, the AACR looks forward to continuing to work with you to ensure that the NIH and FDA have the resources and tools needed to continue to spur innovation and deliver hope to patients and their family members all across our great nation and throughout the world.

Sincerely,



Jose Baselga, MD, PhD  
President



Margaret Foti, PhD, MD (hc)  
Chief Executive Officer



William S. Dalton, PhD, MD  
Chair, AACR Science Policy & Government Affairs Committee



AMERICAN ASSOCIATION FOR RESPIRATORY CARE

9425 North MacArthur Blvd., Suite 100, Irving, TX 75063, (972) 243-2272, Fax (972) 484-2720  
<http://www.aarc.org>, E-mail: [info@aarc.org](mailto:info@aarc.org)

May 4, 2015

The Honorable Fred Upton  
Chairman, House Energy and Commerce Committee  
2183 Rayburn House Office Building  
Washington, DC 20515

The Honorable Diana DeGette  
U. S. House of Representatives  
2368 Rayburn House Office Building  
Washington, DC 20515

**RE: 21<sup>st</sup> Century Cures Discussion Draft, Released April 29, 2015  
Title III – Delivery, Subtitle B, Section 3021 - Telemedicine**

Dear Chairman Upton and Representative DeGette:

The American Association for Respiratory Care (AARC) congratulates you on the recent release of the bipartisan Cures 2015 discussion draft. Advancing this legislation will impact the way that treatments are studied, developed, regulated, and delivered to all Americans.

Of particular importance to AARC are the millions of patients treated by respiratory therapists who suffer from pulmonary diseases such as Chronic Obstructive Pulmonary Disease (COPD), Asthma, and rare disorders like Alpha-1 Antitrypsin Deficiency and Cystic Fibrosis. The AARC believes that delivery of care thru telemedicine will greatly benefit pulmonary patients and, as this is a placeholder within the discussion draft, we appreciate the Committee's invitation to comment.

The AARC is a national professional organization representing 50,000 respiratory therapists who provide clinical care services across the life span for those who suffer pulmonary illness from newborns to geriatrics. As an organization whose mission in part is to serve as an advocate for these patients, the AARC offers its comments on the placeholder for Subtitle B, Section 3021 – Telemedicine, which indicates the intent of Congress to adopt new technologies to promote greater quality care while insuring fiscal integrity. New telehealth services can help meet the unmet needs of pulmonary patients.

The AARC would like to see the Committee adopt draft legislative language that has been developed by Congressmen Harper and Thompson titled the “Medicare Telehealth Parity Act.” The Medicare Telehealth Parity Act offers opportunities to improve health outcomes and reduce hospital readmissions.

In part, the bill will cover telehealth respiratory care services and respiratory therapists as qualified telehealth practitioners. It will also cover remote patient monitoring for those beneficiaries with certain chronic conditions such as COPD. In 2010, according to the Centers for Medicare and Medicaid Services (CMS), Medicare beneficiaries with two or more chronic conditions including COPD and Asthma accounted for almost 98% (1.9 million) of all hospital readmissions.<sup>1</sup> It is also important to note that 52% of Medicare beneficiaries with COPD have 5 or more other conditions; 47% of those with Asthma have 5 or more conditions.<sup>2</sup>

Respiratory therapists are the only allied health professionals educated and competency tested in all aspects of pulmonary medicine and should be covered as qualified telehealth practitioners. Medicare beneficiaries trained by respiratory therapists via telehealth to recognize and reduce symptoms and triggers of their chronic disease can reduce exacerbations and lower the incidence of costly acute care interventions in addition to improving medication adherence and oxygen utilization for those Medicare beneficiaries who require oxygen.

COPD is listed as the third leading cause of death according by the Centers for Disease Control and Prevention. As noted in a 2007 Report to Congress by the Medicare Payment Advisory Commission<sup>3</sup>, COPD is also the fourth most costly condition for which Medicare patients are readmitted to the hospital within 30 days post discharge, which led to its inclusion as one of the conditions subject to the Accountable Care Act’s Hospital Readmissions Reduction Program. Access to respiratory therapists via telehealth for Medicare beneficiaries with chronic lung disease adds another dimension toward improving care and reducing hospital readmissions.

Respiratory therapists are already making a difference in their hospitals by establishing best practices that reduce COPD readmissions that can also be applied via a telehealth delivery system. Further, there are numerous studies related to respiratory care and training to teach patients how to manage their chronic lung disease via telehealth that show these types of services are beneficial in reducing costly acute care interventions. A summary of a few of these studies is attached for your review.

AARC staff and constituents met with Chairman Pitts in March to discuss the Medicare Telehealth Parity Act and express our support for respiratory therapists as qualified practitioners of respiratory telehealth services. As the process moves forward, AARC looks forward to working with the Committee and the Telemedicine Workgroup.

We strongly encourage including the attached legislative draft into the 21st Century Cures initiative. Please feel free to contact Tom Kallstrom, AARC Executive Director at 972-243-2272 or kallstrom@aarc.org.

Sincerely,



Frank R. Salvatore, RRT, MBA, FAARC  
President  
American Association for Respiratory Care

Attachments

*CC: Telemedicine Member Working Group*

Bill Johnson (R-OH)

Bob Latta (R-OH)

Doris Matsui (D-CA)

Frank Pallone (D-NJ)

Gregg Harper (R-MS)

Greg Walden (D-OR)

Peter Welch (D-VT)

- 1 Medicare Payment Advisory Commission. Report to the Congress: Promoting Greater Efficiency in Medicare. 2007. Chapter 5: Payment policy for inpatient readmissions.
- 2 Centers for Medicare and Medicaid Services, "Chronic Conditions among Medicare Beneficiaries, Chartbook: 2012 Edition" (2103).
- 3 <http://www.cms.gov/Research-Statistics-Data-and-Systems/Statistics-Trends-and-Reports/Chronic-Conditions/CCDashboard.html>, accessed 12.8.14.



**Roger Jordan, O.D.**  
Chairman, Federal Relations Committee

May 18, 2015

The Honorable Fred Upton  
Chairman  
Energy and Commerce Committee  
U.S. House of Representatives  
2183 Rayburn House Building  
Washington, DC 20515

The Honorable Frank Pallone  
Ranking Member  
Energy and Commerce Committee  
U.S. House of Representatives  
2368 Rayburn House Building  
Washington, DC 20515

Re: Draft 21<sup>st</sup> Century Cures Act

Submitted electronically via [cures@mail.house.gov](mailto:cures@mail.house.gov)

Dear Chairman Upton and Ranking Member Pallone,

The American Optometric Association (AOA) appreciates your ongoing efforts to work together with doctors of optometry and other physicians to advance smart health care solutions that positively impact the lives of millions of Americans. We thank you for this opportunity to provide input regarding the May 13 draft of the *21<sup>st</sup> Century Cures Act* for listening to our policy priorities and heeding our comments and suggestions throughout this process. Overall, we believe that Congress has an important role to play in defining what steps can be taken to accelerate the pace of cures in America. The AOA supports the ongoing 21<sup>st</sup> Century Cures effort and - along with our broad support - we offer additional thoughts below regarding specific sections of the draft.

The AOA represents approximately 33,000 doctors of optometry and optometry students. Doctors of optometry are eye care professionals who diagnose, treat and manage diseases, injuries and disorders of the eye, surrounding tissues and visual system and play a major role in a patient's overall health and well-being by detecting and helping to prevent complications of systemic diseases such as hypertension, cardiovascular disease, neurologic disease, and diabetes - the leading cause of acquired blindness. Doctors of optometry serve patients in nearly 6,500 communities across the country, and in 3,500 of those communities we are the only eye doctors available. Providing more than two-thirds of all primary

eye health and vision care in the United States, doctors of optometry deliver up to 80 percent of all primary vision and eye health care provided through Medicaid. Recognized as Medicare physicians for more than 25 years, nearly six million Medicare beneficiaries receive medical eye care from doctors of optometry annually.

## SECTION 3001 – ENSURING INTEROPERABILITY

As evidenced by statements from the AOA and numerous other 21st Century Cures roundtable participants, the ability to share research and clinical data is a cornerstone of the drive for new cures, but barriers to nationwide interoperability of health technology exist. Section 3001 of *Cures* would refocus national efforts on making systems interoperable. While the AOA generally supports this provision, we urge Congress to remain mindful of and attentive to the difficulties doctors of optometry and other physicians may face as a result of any potential changes, such as the consequences of using an EHR system previously in compliance but fails to meet certification standards as a result of new requirements.

Considering that meaningful use of EHR will play a large role in the new MIPS program, we would urge Congress to remain mindful that a doctor whose EHR product loses certification in 2018 would most likely be unable to meet MIPS targets when that program begins in 2019.

While we believe that Section 3001 would mostly place the pressure on EHR vendors, our major concern stems from the possibility for doctors using an EHR product that loses its certification status based on new interoperability requirements. Typically, if a provider is using an EHR product that is not certified, they would be subjected to CMS financial penalties. While the *Cures* draft includes language that would provide doctors with a hardship exemption from CMS penalties, if the doctor is using an EHR product that loses certification, we would urge that these exemptions be both broad and easy for the provider to apply for and obtain.

If a doctor is dealing with the loss of certification status, that provider's main focus will likely be on finding a new EHR vendor and working to get that system implemented. Doctors likely won't have time to deal with jumping through CMS hoops to apply for an exemption from any new and previously unforeseen requirements. As such, the AOA strongly supports hardship exemptions that would be available for doctors whose EHRs lose certification status and we urge lawmakers to ensure that those who find themselves in that position are not unduly burdened by additional CMS requirements to apply for and obtain exemption status.

Additionally, Section 3001 includes language related to OIG investigations and the potential for civil monetary penalties for health care providers who engage in "information blocking" or interfering with the exchange of electronic health information. Overall, it is unclear to us how many doctors and other providers would even know how to engage in information blocking, much less actively engage in it. Many doctors put a lot of faith in their EHR vendors to ensure that they are doing all they are supposed to with regard to interoperability. While we believe that it is reasonable to punish those who actively engage in information blocking for nefarious reasons, we also worry about the expectation set that doctors are

supposed to be both EHR experts and full-time doctors.

## SECTION 3021 – TELEHEALTH SERVICES UNDER THE MEDICARE PROGRAM

The AOA has been working closely with the Energy and Commerce Bipartisan Telemedicine Member Working Group to find a solution that has plagued Congress and our health system for decades: how to adopt new technologies into our delivery system in ways that promote greater quality care and fiscal integrity. Section 3021 supports the efforts of the working group by requiring specific actions of government bodies identified as critical to developing a long-term solution to this problem. The AOA generally supports this provision. Overall, we support the appropriate use of telehealth, but we continue to caution lawmakers about encouraging inappropriate uses of telehealth.

We greatly appreciate being listened to and heeded on this issue and urge lawmakers to continue the precedent set by this language to ensure that any expansion of telehealth services under Medicare meets or exceeds the scope of direct services to be replaced.

The AOA believes that telehealth services may be beneficial to patients and providers. When used appropriately, the technology can offer new access points for those living in remote or other underserved areas, where providers are often scarce or non-existent. It can help health care providers better communicate with their patients and with their colleagues as well as the broader interdisciplinary health care team. And, it can also help doctors monitor patients with a diagnosed disease, meaning closer and more convenient observation of disease and the impact of treatment. In fact, the AOA supports the use of telehealth to provide greater interaction between patients with diagnosed disease and their eye care provider.

For instance, optometry has long- participated in telehealth efforts to monitor diabetic patients for progression of diabetic retinopathy. However, while telehealth may offer benefits, it also has serious drawbacks when it is not used appropriately, including the potential for disrupting the doctor-patient relationship and putting patients at an increased risk for delayed or even completely-missed diagnosis and care opportunities. This is especially true when telehealth is used as a replacement for an in-person comprehensive eye health and vision care exam provided by an eye doctor, which is the only preventive intervention that can diagnose and ensure treatment for the complete range of issues that may impact a patient.

In general, we support use of telehealth services within the Medicare program, especially when augmenting services that can be easily interchanged with little or no patient impact. We believe that telehealth has great potential to better serve the needs of the public and that it should be encouraged, but only when used appropriately. However, we strongly believe that telehealth should never be used as a substitute for an in-person comprehensive eye health and vision exam provided by an eye doctor nor to bypass doctors who are available to the patient to provide face-to-face care. It may be used to help monitor diseases, including eye diseases, though only for those with diagnosed disease and in- between regular comprehensive eye health and vision exams.

The only way to truly ensure a patient's eye health and vision is through regular comprehensive eye health and vision exams, which cannot be substituted by a telehealth service.

Overall, we feel that Congress must continue to make clear that telehealth services can only be a substitute for an in-person visit when those services are interchangeable and will not negatively impact the patient, or when there is no access to a provider who can provide the service face-to-face. For example, when a disease specific telehealth eye screening - such as a glaucoma screening - is done in place of comprehensive eye health and vision exam provided in-person by an eye doctor, the patient may lose out of the opportunity for diagnosis and early treatment of many other eye and vision problems that they may not know that they have, including a wide range of eye disease that can be successfully diagnosed and treated.

We also share similar concerns with the use of telehealth services to encourage a patient to be moved to a lower level of care. We believe that lawmakers must look out for what is best for patients and not simply encourage a lower level of service or a less costly but not appropriate telehealth service.

#### SECTION 3041 – EXEMPTING FROM MANUFACTURER TRANSPARENCY REPORTING CERTAIN TRANSFERS USED FOR EDUCATION PURPOSES

This section would exempt certain transfers of value to physicians from reporting requirements that have hindered physician participation in important continuing medical education activities. The AOA generally supports Section 3041.

Overall, there is a concern in the provider community that required public reporting has resulted in misleading perceptions regarding relationships between industry and physicians. Likewise, these disclosures alone do not ensure the establishment of ethical standards or physician integrity. In fact, CMS has acknowledged these concerns and noted: “disclosure alone is not sufficient to differentiate beneficial financial relationships from those that create conflict of interests or are otherwise improper. Moreover, financial ties alone do not signify an inappropriate relationship. However, transparency will shed light on the nature and extent of relationships, and will hopefully discourage the development of inappropriate relationships and help prevent the increased and potentially unnecessary health care costs that can arise from such conflicts.”

In the ever-changing and evolving world of health care, it is an imperative for physicians to continuously expand their knowledge of new treatment strategies, technologies, and products to enhance patient care. This information is available in a myriad of ways, including the scientific literature, educational programs, marketing materials, exhibits at professional meetings, and direct contact with industry representatives whose performance is based on sales. However, it is important to understand interactions between physicians and industry representatives can be of high value and lead to improved patient care without necessarily compromising the ethics and integrity of the physician.

## SECTION 3081 – IMPROVEMENTS IN THE MEDICARE LOCAL COVERAGE DETERMINATION (LCD) PROCESS

The LCD process is an important means by which seniors can access treatments that would otherwise not be covered by Medicare due to the length of time it takes for the national process to conclude its work. However, improvements are needed and, at times, the LCD process can be used to restrict patient access to needed care. Section 3061 would increase transparency around the LCD process. The AOA believes that reform of local and national coverage decisions is very much needed and we applaud lawmakers for including a provision toward this end within the *Cures* draft.

In the past, the AOA has witnessed the damage that improper coverage decisions can have on providers and their patients. A few years ago, a Medicare Administrative Contractor improperly used a local coverage determination (LCD) in a way that impacted what constitutes the appropriate scope of practice of a doctor of optometry. While Congress ultimately stepped in to reverse this action, a MAC for Jurisdiction 5 had assembled a list of codes it determined doctors of optometry were allowed to bill Medicare based on its own flawed interpretation of state scopes of practice.

Based on this flawed interpretation, the MAC then issued a LCD based on the list of codes it had assembled and then proceeded to deny coverage for a range of services which doctors of optometry are legally authorized to perform under state law. As a result, seniors were improperly denied access to medically necessary, covered physician services, which they needed when they chose to legally obtain those services from doctors of optometry rather than from other physicians.

The AOA and leading lawmakers objected to these actions as the Social Security Act requires Medicare to cover physician services, including services provided by doctors of optometry within state scopes of practice. Medicare beneficiaries also have the "basic freedom of choice" to obtain health services from any qualified health care provider (Section 1802(a) of the Social Security Act). Also, AOA and lawmakers objected because Medicare beneficiaries have the right to have such services judged by objective clinical standards to determine if they are "reasonable and necessary" for coverage purposes (Section 1862(a)(1)(A) of the Social Security Act).

The role of the MAC is to responsibly make those types of clinical coverage assessments after consultation with the respective health care provider groups (Section 1874A of the Social Security Act). However, the AOA and lawmakers objected because under statute it is not the role of the MAC to determine what is or not within the state authorized scope of professional practice under the guise of establishing what services are clinically reasonable and necessary. That legal function is squarely and exclusively the responsibility of the states, usually delegated to the purview of the state licensing board.

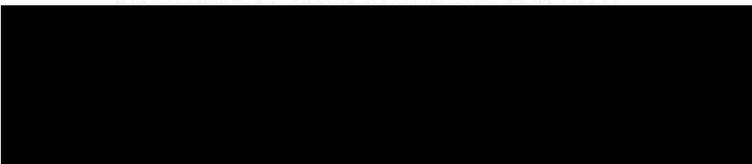
In short, the AOA remains concerned that actions taken by MACs have in the past ultimately superseded state authority to determine optometric scope of practice. These actions have only served to restrict

patient access to a range of services which doctors of optometry are legally authorized to perform. While CMS has admonished a contractor for creating such a list of codes, we believe that congressional action is needed to ensure that MACs respect state authority to determine scope of practice.

As you continue to consider changes and additions to the *Cures* package, the AOA urges you to continue working toward a meaningful and impactful legislative product. We appreciate your ongoing efforts to work together and with doctors of optometry and other physicians to advance smart health care solutions that positively influence the lives of millions of Americans. On behalf of our membership and the millions of patients that doctors of optometry serve each year, we thank you for considering these comments and using our feedback to further improve the *21<sup>st</sup> Century Cures Act*.

Please contact Matt Willette of the AOA Washington office at [mwillette@aoa.org](mailto:mwillette@aoa.org) or (703) 837-1001 if you have questions or need additional information about these comments.

Sincerely,



Roger Jordan, O.D., F.A.A.O  
Chairman, Federal Relations Committee  
American Optometric Association



American Society for  
Biochemistry and Molecular Biology  
11200 Rockville Pike, Suite 302  
Rockville, Maryland  
20852-3110

The Honorable Representative Fred Upton  
United States House of Representatives  
2183 Rayburn House Office Building  
Washington, DC 20515

The Honorable Representative Diana DeGette  
United States House of Representatives  
2368 Rayburn House Office Building  
Washington, DC 20515

Dear Reps. Upton and DeGette,

The American Society for Biochemistry and Molecular Biology applaud your and the entire U.S. House Energy and Commerce committee's work on the 21<sup>st</sup> Century Cures initiative. The ASBMB has been involved in the conversation surrounding this initiative as we commented on two white papers from the committee and the discussion draft released in January.

The ASBMB is supportive of this effort, but we still have some concerns about language in the 21<sup>st</sup> Century Cures Act draft. Our members conduct fundamental research funded primarily by the National Institutes of Health. Thus, we have restricted our comments to the sections that would most affect our members.

**Sec. 1001 – National Institutes of Health Reauthorization**

We appreciate and support the 4.5 percent increase in authorizations through fiscal 2018.

**Sec. 1002 – NIH Innovation Fund**

Our concern: We appreciate the proposed infusion of \$2 billion in mandatory funding for the NIH through fiscal 2020. However, we are concerned what will happen after FY20. Dramatic funding cuts contribute to the loss of faculty and trainee jobs, lost scientific productivity and general instability in the enterprise. Without an extension of the Innovation Fund or increases in appropriations, the NIH will face a \$2 billion shortfall come FY21. This may result in a dramatic disruption of the research enterprise.

Our recommendation: We recommend that the overall \$10 billion in extra funding be maintained, but spread out over 7 years with \$2 billion in each of FY16, 17 and 18, \$1.5 billion in FY19, \$1 billion in each of FY20 and 21 and \$0.5 billion in FY22. This will allow the Innovation Fund to last until the end of the Budget Control Act caps and potentially allow the Appropriations Committee to make up for any lost funding in FY23.

Additionally, we recommend on page 8, line 5 that funds for “Young emerging scientists” include Institutional and Individual training grants for postdocs and graduate students. We also recommend that on page 8, line 7, that “Other” be changed to specify “Investigator-initiated research”.

**Sec. 1021 – NIH Research Strategic Plan**

Our concern: We support the development of an NIH-wide strategic plan. However, fundamental researchers often make discoveries that open up completely new fields requiring the NIH to divert resources to understand and develop breathtaking discoveries that improve human health.

Our recommendation: We suggest adding language that provides the NIH flexibility in addressing research goals. For example, on page 11, line 10, the following language could be added: “(C) Given the unpredictable nature of scientific research, the Strategic Plan should not prevent the NIH from pursuing groundbreaking discoveries that are made during the time covered by the Strategic Plan.”

#### **Sec. 1022 – Increasing Accountability at the National Institutes of Health**

Our concern: We support improving accountability at federal agencies, including the NIH, and the proposed IOM study on duplicative research funded by the agency. However, we are concerned about a literal interpretation of page 14, line 23 that states the director of an institute or center should review and approve each R-series grant. It is not physically possible for an institute director to evaluate every grant that is recommended for funding by peer-review groups.

Our recommendation: We recommend altering the language on page 14 starting on line 21 to read, “the director of such national research institute or center in conjunction with the institute’s or center’s governing council-”

#### **Sec. 1023 – Biomedical Research Working Group**

Our concern: We support harmonizing, streamlining and eliminating regulations that slow the pace of research and the establishment of the Biomedical Research Working Group. However, this proposal is not unique. H.R.1119 would establish an interagency working group that would address onerous regulations at all federal science agencies. Establishing the BRWG and the interagency working group would result in duplicative efforts.

Our recommendation: We recommend adding a provision that would immediately sunset the BRWG should an interagency working group be established, and all of the work of the BRWG should be handed over to the interagency group.

#### **Sec. 1025 – NIH Travel**

Our concern: The travel of federal scientists and administrators to scientific conferences is essential for establishing working relationships with other scientists in the field, producing effective collaborations, and developing professional skills. We appreciate the Sense-of-Congress statement, but we prefer a stronger directive.

Our recommendation: We recommend that language be included that specifically exempts NIH scientists from federal travel restrictions.

#### **Sec. 1028 – High-Risk, High-Reward Research**

Our concern: This section directs all NIH institutes and centers to reserve money for high-risk, high-reward research. Forcing institutes and centers to fund this type of research, which is already done well by the Common Fund, will divert funding from other well established programs that fund many researchers doing excellent work. Furthermore, high-risk, high-reward research is often transdisciplinary. One of the reasons the Common Fund was created was to fund high-risk, high-reward, transdisciplinary research. Furthermore, having the director of the NIH specify how much each institute and center should spend on specific programs devalues the role of the institute and center directors and concentrates authority in the director of the NIH.

Our recommendation: To expand high-risk, high-reward research at the NIH, the language should be rewritten to redistribute funds within the Common Fund.

### **Sec. 1041 – Funding Research by Emerging Scientists**

Our concern: This section would stop the transfer of money from NIH to AHRQ through what is commonly called “the tap.” The money saved by the NIH would be directed to funding “emerging scientists.” We have several concerns with this section.

- (1) The diversion of money from AHRQ damages the research community as a whole. All research is interconnected. AHRQ ensures that the discoveries made by NIH-funded researchers that turn into FDA-approved products are delivered and being used in the most effective and efficient ways possible. This type of research is critical for the NIH, CDC and FDA to improve on these products and ensure that they are available to all who need them. Furthermore, the goal of the 21<sup>st</sup> Century Cures initiative is to improve not only the path from discovery through development and delivery, but also to improve how patient feedback affects research and discovery.<sup>1</sup> The work of AHRQ is critical for this second part. It is not clear how the 21<sup>st</sup> Century Cures initiative benefits from potentially reducing the effectiveness of AHRQ.
- (2) On p.22, line 16, the definition of an “emerging scientist” differs substantially from the NIH’s definition of an Early Stage Investigator. Introducing a new class of investigator on top of a very similar class will cause confusion in the community and lead to inefficiencies in grant awarding and data analysis.
- (3) It is not clear why money is being diverted to “emerging scientists” in the manner indicated here. The NIH policy of ensuring Early Stage Investigators have a reasonable chance at receiving grant money has been largely successful—grant applications from ESIs have nearly the same chance of success as established investigators.<sup>2</sup>

Our recommendation: Section 1041 should be removed from the 21<sup>st</sup> Century Cures Act in its entirety.

### **Sec. 1042 – Improvement of Loan Repayment Programs of NIH**

We appreciate and support efforts to help scientists repay their student loans.

### **Sec. 1061 –Capstone Award**

Our concern: The ASBMB is not in favor of a capstone award. The NIH should grant taxpayer money to scientists who propose to do important research in the service of the public. Capstone awards will not be made in this vein as they will be awarded to senior scientists ending their careers at the bench. When a senior investigator transitions to a new role, it is the responsibility of the institution, financially and in all other manners, to provide the opportunity to acquire the necessary skills for the new role. A capstone award is not a wise use of taxpayer money.

Furthermore, instituting an emeritus award could have several unintended consequences. For example, in a stagnant budget environment, the money for such an award must be taken from other pools of NIH grants that fund investigators at all career stages. Thus, taking money from other programs to fund an emeritus award could harm just as many investigators as the NIH is trying to help.

Our recommendation: Section 1061 should be removed from the 21<sup>st</sup> Century Cures Act in its entirety.

---

<sup>1</sup> <http://energycommerce.house.gov/cures>

<sup>2</sup> <http://report.nih.gov/nihdatabook/index.aspx>. “R01-Equivalent grants, New (Type 1): Success rates, by career stage of investigator”



**AMERICAN  
SOCIETY FOR  
MICROBIOLOGY**

*Public and Scientific Affairs Board*

May 8, 2015

The Honorable Fred Upton  
Chair, House Committee on Energy and Commerce  
2125 Rayburn House Office Building  
Washington, DC 20515

The Honorable Diana DeGette  
Ranking Member, House Committee on Energy and Commerce  
2125 Rayburn House Office Building  
Washington, DC 20515

Dear Chairman Upton and Ranking Member DeGette:

The American Society for Microbiology (ASM) appreciates the opportunity to comment on the House Energy and Commerce Committee's current draft of the 21<sup>st</sup> Century Cures Act, which aims to stimulate both innovation in biomedical research and the development of new medical treatments and cures. We applaud the Act's \$10 billion increase in National Institutes of Health (NIH) funding over five years, which is intended to be mandatory funding outside the annual appropriations process. This proposed increase provides needed additional support for the NIH, which funds much of our Nation's biomedical discoveries. The bill's authorization of an additional \$1.5 billion in discretionary funding for each of the next 3 years will help to set NIH on a path to growth after years of stagnant funding. We recognize the dedication of the Committee during its year-long solicitation of public input while drafting this legislation, beginning with the Committee's launch in April 2014 of its 21<sup>st</sup> Century Cures initiative.

In addition to authorizing increased NIH funding, important features of the current draft include provisions to modernize R&D related strategies like clinical trials, to further develop precision medicine, to encourage young investigators and to stimulate collaborations among all stakeholders, toward the goal of ensuring greater innovation in biomedicine. To support the Committee's further refinement of this legislation, the ASM offers the following comments on specific aspects of the Act:

- In the current draft, the proposed \$10 billion NIH Innovation Fund (a distribution of \$2 billion for each fiscal year from 2016 - 2020) is required to be used only for specified initiatives in the statute that at present include: Precision Medicine, Young Emerging Scientists, and an unidentified "Other" category yet to be determined. While the ASM agrees that the first two initiatives deserve to be a high priority for NIH funding, we believe progress in biomedical research and discovery would be better served if the Innovation Funds were distributed across

NIH's Institutes and Centers, to broadly support meritorious investigator initiated research which has been underfunded for over a decade. With only 18 percent of submitted grant proposals being funded at present, greater resources are needed for R&D efforts across NIH Institutes and Centers. The looming loss of younger scientists is an acute problem, exacerbated by the prospect of never getting a grant funded. The US needs to not only attract new investigators to biomedical research, but also be able to then keep them engaged with the availability of robust grant funding. It is also critical that NIH decide how to allocate its research funding based on scientific opportunities.

- The ASM also asks whether/how the Innovation Fund would overlap with NIH's existing Common Fund and recommends clarification on this specific issue.
- The Act would establish a new Biomedical Research Working Group with federal and non-federal members, directed to advise the NIH Director on restructuring and streamlining the grant proposal process. While the ASM supports a more efficient, simplified grant process, we are concerned that this Group might duplicate efforts already initiated by the existing Scientific Management Review Board. The National Academy of Sciences also is currently conducting a study on R&D administration that might duplicate these efforts.
- The ASM thanks the Committee for including measures that would help reduce the regulatory/reporting burden on NIH funded researchers, who often spend excessive amounts of time on grant related paperwork instead of research.
- We also appreciate the inclusion of language encouraging attendance by NIH supported scientists at scientific conferences and meetings.

The Cures Act draft does not address funding increases for the Food and Drug Administration (FDA), despite mandating new FDA programs that would impact the Agency's already overextended workloads. The ASM recognizes that the Energy and Commerce Committee does not have authority over FDA funding similar to its jurisdiction over NIH funding. However, the FDA's purchasing power has remained level over the past 10 years, and requirements for additional responsibilities should be accompanied by additional resources. The ASM recommends that FDA officials be asked to provide estimates of the additional funding that would be needed to fulfill provisions of the Cures Act, and that the Energy and Commerce Committee actively work with the appropriations committees to find new resources to cover new mandates.

Additional FDA resources factor into draft sections that address drug resistant microbial pathogens and the enhanced development of new antimicrobial drugs and vaccines. The ASM often has warned against threats from increasing drug resistance among disease causing microorganisms, as well as shrinking R&D pipelines of new treatments and preventives. We appreciate that the Committee directly confronts these issues, but are concerned about inadequate FDA funding to cover the necessary regulatory activities. We ask the Committee to ensure that FDA can effectively implement new measures

without compromising standards of safety, efficacy and quality in the drug approval process. For example, creation of a new Interpretive Criteria Website publicly listing updated or new drug susceptibility test criteria standards and related information should help streamline the drug approval process. This will be a major undertaking and should not be yet another unfunded mandate. Specifics must be clarified; e.g., the source of the posted data, which controls will ensure quality and consistency, assurances that the data stream will be constantly fed with real world results, and guarantee of dedicated personnel involved.

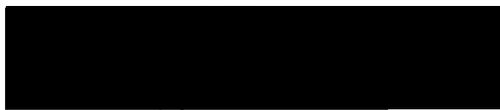
The ASM agrees with the Act's provision for an interagency (NIH, FDA, Centers for Disease Control and Prevention [CDC]) review of current barriers to the development of new antimicrobials, especially if such review leads to removal of identified barriers.

The Act calls for timely review of vaccine candidates by CDC's Advisory Committee on Immunization Practices (ACIP). It also directs an expedited review for breakthrough therapies and for use during public health emergencies. The ASM considers both directives to be reasonable requests. The Act additionally details a required CDC review of ACIP procedures. We recommend that, if included in the final Cures Act, there be sufficient recognition and support provided for both the review and ACIP activities. Tasking ACIP with more work, without adequate technical assistance, would be counterproductive.

We support the Act's provision for meetings between CDC and representatives of vaccine developers, to be convened at the latter's request. Designed to improve communication among stakeholders, the meetings would clearly explain CDC and other federal expectations relevant to vaccine R&D and provide appropriate data to the vaccine developer.

The ASM appreciates the need to address barriers to medical innovation and thanks the Committee for its contributions to this effort, and for the future biomedical improvements that will likely result from the Act's proactive stance on improving public health.

Sincerely,



President, ASM



Chair, Public and Scientific  
Affairs Board

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 [21<sup>st</sup> 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 “21<sup>st</sup> 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 21<sup>st</sup> 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 21<sup>st</sup> 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**

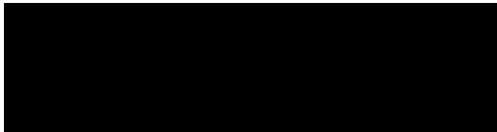
ASN supports the provision outlined in Section 3041 that would clarify that peer-reviewed journals, journal reprints, journal supplements, and medical textbooks are excluded from the reporting requirement under the Sunshine Act.

### **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](mailto:meyer@asn-online.org) or at (202) 640-4659.

Sincerely,



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

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 [21<sup>st</sup> 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 “21<sup>st</sup> 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 21<sup>st</sup> 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 21<sup>st</sup> 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](mailto:meyer@asn-online.org) or at (202) 640-4659.

Sincerely,

A large black rectangular redaction box covering the signature area.

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



**ASSOCIATION FOR MOLECULAR PATHOLOGY**

*Education. Innovation & Improved Patient Care. Advocacy.*

9650 Rockville Pike, Bethesda, Maryland 20814

Tel: 301-634-7939 | Fax: 301-634-7995 | [amp@amp.org](mailto:amp@amp.org) | [www.amp.org](http://www.amp.org)

May 19, 2015

The Honorable Fred Upton  
Chairman  
House Energy and Commerce Committee  
2125 Rayburn House Office Building  
Washington, DC 20515

The Honorable Diana DeGette  
Member  
House Energy and Commerce Committee  
2125 Rayburn House Office Building  
Washington, DC 20515

Sent via e-mail: [Cures@house.mail.gov](mailto:Cures@house.mail.gov)

Re: Regarding the 21st Century Cures Act third discussion draft

Dear Chairman Upton and Congresswoman DeGette,

The Association for Molecular Pathology (AMP) would like to congratulate you on the recent release of the next draft of the 21<sup>st</sup> Century Cures Act. Thank you for the tremendous effort put forth on this important piece of legislation. AMP has worked with the Committee for over a year in anticipation of this bill and would like to provide the feedback below on the most recent draft.

**Local Coverage Determinations**

AMP is pleased that the Committee understands that Medicare administrative contractors' (MAC) local coverage determination (LCD) process should be both uniform across MACs and transparent. However, we are very concerned that important text that was included in the earlier draft has been removed from this draft that would have required MACs to 1) extend comment periods to 60 days for LCDs that would limit or preclude coverage, 2) convene a meeting of its Carrier Advisory Committee (CAC) to secure its advice for LCDs that would limit or preclude coverage, 3) hold meetings with stakeholders upon request, and most importantly, 4) prohibit MACs from adopting another jurisdiction's LCDs only if they have conducted their own public comment period, provided responses to these comments, and held a stakeholder meeting. We have previously provided an explanation of why a transparent LCD process is so crucial for ensuring that patients have access to lifesaving diagnostics. Those comments can be found [here](#).

Section 3081 should be changed to require the following:

- MACs should be required to provide a notice and comment period of no less than 45 days;
- MACs should hold open, public meetings at which draft LCDs are reviewed and the MACs receive comments;
- CMS should create a mechanism to appeal coverage determinations. This should be done by removing the new evidence requirement and including in LCD reconsideration requests, the option of making an appeal to an uninterested body such as a CMS regional office, or the CMS Administrator.
- MACs should be required to meet with specialty societies impacted by their draft LCDs, not just members within their jurisdictions;
- MACs should facilitate participation in their CAC and other public meetings by allowing remote participation;

- MACs should be required to hold webinars open to the public on these draft policies when LCDs would limit or preclude coverage;
- MACs should be required to develop and maintain a listserv or web-portal listing all draft LCDs so they can be easily found by interested parties;
- LCDs that restrict coverage should address only single services (covered under a single CPT), not broad non-coverage policies. Furthermore, MACs should be required to identify services by CPT code and diagnoses by ICD code and should not use or require locally-developed identifier codes;
- MACs may not duplicate the regulatory activities of CLIA such as evaluating analytical validity of diagnostic tests in making coverage or payment determinations;
- MAC may adopt another MAC's policy only after holding its own CAC meeting and public comment process

### **Travel Policy**

In order to advance precision medicine, molecular pathologists must be able to exchange emerging scientific findings, discuss new theories with other thought-leaders in the field, and explore new technological approaches at premiere conferences both locally and abroad. While we appreciate that the Committee understands the importance of travel to medical and scientific conferences (Sections 1025 and 2282), AMP feels that Congress can accomplish a great deal more to reduce the [regulatory burden on Federal employees](#) regarding this issue while at the same time allowing scientists and health professionals to have access to the important data shared at these conferences. AMP respectfully requests that nonprofit scientific and medical associations' meetings for which education is the primary goal, be exempted from a cap or restriction on federal employee travel. We recommend the following language:

"Scientific, medical, and technical conferences are exempt from caps or restrictions on Federal employee travel. A scientific, medical, and technical conference is defined as a gathering, symposium, seminar, workshop or any other organized, formal conference where scientists; engineers of science, technology, engineering and mathematics (STEM) research and development fields; or physicians and other health professionals assemble to present, coordinate, exchange and disseminate information and research; to explore or clarify a defined subject, problem or area of knowledge in the STEM fields; or for continuing medical education."

We understand the Committee has jurisdiction over only select agencies, thus we recommend at the very least that scientific, medical, and technical conferences are exempt from caps or restriction on Department of Health and Human Services employee travel.

### **Precision Medicine**

We thank the Committee for Section 2041 which would require FDA to issue, and periodically update, guidance documents intended to help advance the clinical development of genetically targeted treatments. However, we urge the Committee to edit the line "the development of companion diagnostics in the context of a drug development program" as a topic that FDA would have to address via guidance. AMP recommends that "companion diagnostics" be replaced with "targeted biomarkers". The single test, single drug paradigm as described by the term "companion diagnostic," is obsolete as new technologies allow for the testing of multiple analytes simultaneously with greatly reduced per-analyte costs. AMP strongly recommends that the term "companion diagnostic" not be included in any legislation or regulatory policy. Optimized patient care relies on testing that evolves with new discoveries and technologies. The concept that the only appropriate test is the one co-developed with the drug, or developed with studies using likely unobtainable specimens from patients being treated with that drug, would hinder the application of new technologies and improvements to current tests over the decades the drug is in use and result in suboptimal patient care. An ideal tool to help ensure accuracy and reliability as tests and technologies advance is standard reference materials. AMP strongly recommends that standard reference materials be created for targeted therapies, whether produced in a public-private

partnership such as Pharma-NIST or through Pharma-funded private mechanisms. Furthermore, AMP believes that drug labels should not specify the brand name of diagnostic tests.

We also strongly urge the Committee to require that the FDA withdraw any draft guidance documents that have not been finalized within one year of the draft's release.

Considering that precision medicine will revolutionize our approach to health and disease, and that data sharing will be instrumental as research is done to realize our collective goals in the area, we strongly suggest the following changes:

- Section 1141 – Council for 21st Century Cures – Under Sec. 281C, replace “representatives of the medical device industry” with “representatives of the molecular diagnostics testing industry.”

### **Continuing Medical Education**

AMP is fully supportive of the inclusion of Section 3041 which would clarify those peer-reviewed journals, journal reprints, journal supplements, and medical textbooks are excluded from the reporting requirement under the Sunshine Act. However, the language should be altered to explicitly state that all travel expenses, not just tuition, need to be exempted.

Once again, AMP appreciates the opportunity to provide these comments in response to the 21st Century Cures Act discussion draft. Please do not hesitate to contact Mary Williams, AMP's Executive Director, at [mwilliams@amp.org](mailto:mwilliams@amp.org) if we may be of assistance or provide additional information.

Sincerely,

Janina Longtine, MD  
President, AMP



**Association of  
American Cancer Institutes**

3708 Fifth Avenue  
Medical Arts Building, 503  
Pittsburgh, PA 15213

412•647•6111

mail@aaci-cancer.org  
www.aaci-cancer.org

**OFFICERS**

**President**

*George J. Weiner, MD*  
Holden Comprehensive Cancer Center  
University of Iowa

**Vice President/  
President-Elect**

*Stanton L. Gerson, MD*  
Case Comprehensive Cancer Center  
Case Western Reserve University  
Seidman Cancer Center at  
University Hospitals Case Medical  
Center

**Immediate Past President**

*Michelle M. Le Beau, PhD*  
University of Chicago  
Comprehensive Cancer Center

**Treasurer**

*Jeff Walker, MBA*  
The Ohio State University  
Comprehensive Cancer Center-  
James Cancer Hospital &  
Solove Research Institute

**Executive Director**

*Barbara Duffy Stewart, MPH*

**BOARD OF DIRECTORS**

*Kevin J. Cullen, MD*  
University of Maryland  
Marlene and Stewart  
Greenebaum Cancer Center

*Roy A. Jensen, MD*  
The University of Kansas Cancer Center

*Michael B. Kastan, MD, PhD*  
Duke Cancer Institute  
Duke University Medical Center

*Scott M. Lippman, MD*  
UC San Diego Moores Cancer Center

*Edison T. Liu, MD*  
The Jackson Laboratory Cancer Center

*Patrick J. Loehrer, Sr., MD*  
Indiana University  
Melvin and Bren Simon Cancer Center

*Thomas A. Sellers, PhD, MPH*  
Moffitt Cancer Center

*Dan Theodorescu, MD, PhD*  
University of Colorado Cancer Center

May 28, 2015

The Honorable Fred Upton  
Chairman  
Committee on Energy and Commerce  
United States House of Representatives  
Washington, DC 20515

The Honorable Joe Pitts  
Chairman, Subcommittee on Health  
Committee on Energy and Commerce  
United States House of Representatives  
Washington, DC 20515

The Honorable Frank Pallone  
Ranking Member  
Committee on Energy and Commerce  
United States House of Representatives  
Washington, DC 20515

The Honorable Gene Green  
Ranking Member, Subcommittee on Health  
Committee on Energy and Commerce  
United States House of Representatives  
Washington, DC 20515

Dear Chairman Upton, Ranking Member Pallone, Chairman Pitts, and Ranking Member Green:

On behalf of the Association of American Cancer Institutes (AACI), an association of 94 leading cancer centers dedicated to reducing the burden of cancer, we would like to thank the House Energy and Commerce Committee for its unanimous approval on May 21 of H.R. 6, the 21<sup>st</sup> Century Cures Act.

We appreciate that the Committee reached a bipartisan deal to include language to authorize \$1.5 billion per year in increased funding for the National Institutes of Health (NIH) over the next three years, and also language to provide an additional \$10 billion in mandatory funding through Fiscal Year 2020 through the NIH Innovation Fund. We are pleased that the NIH Innovation Fund will focus on biomarkers and precision medicine. Investing in precision medicine and precision drugs will improve public health and advance cancer prevention, diagnosis and treatment.

AACI also applauds the inclusion of funding through the Cures Innovation Fund for the Food and Drug Administration (FDA). Predictable and sustained growth in FDA funding remains a priority for the cancer community and is essential to research and innovation. Hiring experienced FDA staff is central to the effective and efficient approval of breakthrough devices. The science of molecular testing is advancing rapidly; it is vital that regulators not stifle the innovation behind this progress.

AACI maintains that the time and effort that would be required of the NIH to fulfill a Strategic Plan would be costly, and that such a plan might also duplicate efforts. However, we are pleased to see that language was included to require the NIH director to report on plans to attract, retain, and develop emerging scientists. Scientists who are part of such groups are a key component of cancer research and discovery, yet many young people who represent the next generation of cancer researchers are pursuing other career options when they perceive no professional future in cancer research.

Lastly, AACI is encouraged by the creative solution offered to pay for the bipartisan legislation. The nation's cancer centers are steadfast in their efforts to reduce the burden of cancer for all Americans and, with enhanced and sustained support, we are making progress in helping the U.S. retain its lead in biomedical research. Unfortunately, the negative impact of flat or decreased funding on the NIH and the National Cancer Institute, coupled with sequestration, has limited our nation's ability to accelerate scientific progress in recent years.

AACI congratulates the Committee on the passage of this bipartisan bill and expresses its sincere thanks for your tireless work to bring improvements to biomedical research. Restoring NIH's lost purchasing power and providing researchers with the funds needed to continue with research and expand discovery is vital to reducing the pain and suffering caused by cancer, as well as the economic future of our country.

Many thanks to you, your staff, and the Committee for your dedication to 21<sup>st</sup> Century Cures. We look forward to continuing to work with you toward a future without cancer.

Sincerely,



George J. Weiner, MD  
Director  
Holden Comprehensive Cancer Center  
University of Iowa



Barbara Duffy Stewart, MPH  
Executive Director  
Association of American Cancer Institutes

cc: House Energy and Commerce Committee Members



**Association of  
American Cancer Institutes**

3708 Fifth Avenue  
Medical Arts Building, 503  
Pittsburgh, PA 15213

412•647•6111

mail@aacI-cancer.org  
www.aaci-cancer.org

**OFFICERS**

**President**

*George J. Weiner, MD*  
Holden Comprehensive Cancer Center  
University of Iowa

**Vice President/  
President-Elect**

*Stanton L. Gerson, MD*  
Case Comprehensive Cancer Center  
Case Western Reserve University  
Seidman Cancer Center at  
University Hospitals Case Medical  
Center

**Immediate Past President**

*Michelle M. Le Beau, PhD*  
University of Chicago  
Comprehensive Cancer Center

**Treasurer**

*Jeff Walker, MBA*  
The Ohio State University  
Comprehensive Cancer Center-  
James Cancer Hospital &  
Solove Research Institute

**Executive Director**

*Barbara Duffly Stewart, MPH*

**BOARD OF DIRECTORS**

*Kevin J. Cullen, MD*  
University of Maryland  
Marlene and Stewart  
Greenebaum Cancer Center

*Roy A. Jensen, MD*  
The University of Kansas Cancer Center

*Michael B. Kastan, MD, PhD*  
Duke Cancer Institute  
Duke University Medical Center

*Scott M. Lippman, MD*  
UC San Diego Moores Cancer Center

*Edison T. Liu, MD*  
The Jackson Laboratory Cancer Center

*Patrick J. Loehrer, Sr., MD*  
Indiana University  
Melvin and Bren Simon Cancer Center

*Thomas A. Sellers, PhD, MPH*  
Moffitt Cancer Center

*Dan Theodorescu, MD, PhD*  
University of Colorado Cancer Center

May 13, 2015

The Honorable Fred Upton  
Chairman  
Committee on Energy and Commerce  
United States House of Representatives  
Washington, DC 20515

The Honorable Joe Pitts  
Chairman, Subcommittee on Health  
Committee on Energy and Commerce  
United States House of Representatives  
Washington, DC 20515

The Honorable Frank Pallone  
Ranking Member  
Committee on Energy and Commerce  
United States House of Representatives  
Washington, DC 20515

The Honorable Gene Green  
Ranking Member, Subcommittee on Health  
Committee on Energy and Commerce  
United States House of Representatives  
Washington, DC 20515

Dear Chairman Upton, Ranking Member Pallone, Chairman Pitts, and Ranking Member Green:

On behalf of the Association of American Cancer Institutes (AACI), an association of 94 leading cancer centers dedicated to reducing the burden of cancer, we would like to thank the House Energy and Commerce Committee’s 21st Century Cures Initiative for the release of the third draft focused on advancing biomedical research and cures for deadly diseases such as cancer. AACI is pleased to provide our comments on the bipartisan draft which clearly reflects considerable input from researchers, patients, federal agencies, and the biomedical industry.

Cancer research has made great strides since the signing of The National Cancer Act in 1971. With enhanced and sustained support for biomedical research, the possibilities for research and development advances in the U.S. are limitless. Progress can be made to help the U.S. maintain its lead in biomedical research, but the negative impact of flat or decreased funding on the National Institutes of Health (NIH) and the National Cancer Institute (NCI) is of great concern.

We appreciate that the Committee has provided a draft that includes language to authorize \$1.5 billion per year in increased funding for the NIH over the next three years, and also language to provide an additional \$10 billion in mandatory funding through Fiscal Year 2020 through the NIH Innovation Fund. Predictable and sustained growth in federal funding remains a priority for the cancer community and is essential to allow the U.S. to maintain its standing in research and innovation. It is imperative that the Committee consults with appropriators to see that new funding is provided in order to pay for the provisions in the discussion draft.

While AACI applauds elements in the third draft, we have several suggestions for improving the draft:

## **Title I- Discovery:**

- **Subtitle B, Section 1021, to require NIH to develop a 5-year biomedical research strategic investment plan to make funding allocation decisions, including strategic investment and focus areas for each institute.** The time and effort that would be required of the NIH to fulfill such a plan would be costly. Additionally, each NIH institute and center is presently required to produce their own 5-year strategic plan that includes goals and plans for achieving them. This requirement not only appears to duplicate efforts, but might also undermine strategic plans already in place. Setting priorities through statutory language could hinder the work of the NIH, and specifying Mission Priority Focus Areas could impact research and discovery in the cancer community and in the healthcare field overall.

## **Title II- Development:**

- **Subtitle C, Section 2041, to advance precision medicine by way of the Food and Drug Administration (FDA).** AACI is pleased that the advancement of precision medicine may be part of the final Cures bill. Investing in precision medicine and precision drugs would improve public health and advance the prevention, diagnosis, and treatment of cancer. The discussion draft prescribes in Title I, Subtitle A, Section 1002, that the NIH Innovation Fund would fund precision medicine initiatives. The funding source is of concern to AACI as the progress of precision medicine would require adequate funding to ensure its success. Therefore, we ask the Committee to consult with appropriators to secure new funding in order to advance precision medicine.
- **Subtitle M, Section 2228, to recommend the Secretary of Health and Human Services publish guidance clarifying Clinical Laboratory Improvement Amendments (CLIA) waivers for in vitro diagnostics.** AACI cancer centers continually focus on improving patient safety and avoiding unwanted or unwarranted treatments and diagnostic testing. Academic labs are highly regulated through CLIA, state laws, and accreditation by organizations such as the College of American Pathologists (CAP). In addition, the science of molecular testing is advancing incredibly quickly, and it is vital that we do not stifle the innovation behind this rapid speed of progress. Any guidance published by the Secretary of Health and Human Services should be crafted carefully and eliminate bureaucratic burdens.

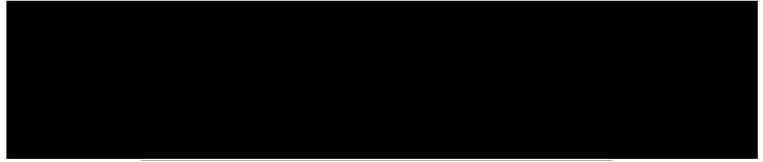
AACI appreciates the Committee's proposal to bring greater awareness of, and improvement to, biomedical research. We ask that the Committee amend the aforementioned sections as indicated, and that you and your colleagues collaborate with appropriators to provide sustained funding growth for the NIH and FDA and to lift the sequestration cap to make room in the budget for a funding increase for FY 2016 and beyond. Doing so would be an important step toward putting NIH and NCI back on a path of predictable growth, which is critical if our nation's cancer centers are to increase the pace of progress in cancer research and bring new therapies to the patients who depend on them.

Many thanks to you, your staff, and the Committee for your dedication to 21<sup>st</sup> Century Cures.  
We look forward to working with you toward a future without cancer.

Sincerely,



George J. Weiner, MD  
Director  
Holden Comprehensive Cancer Center  
University of Iowa



Barbara Duffy Stewart, MPH  
Executive Director  
Association of American Cancer Institutes



**Association of  
American Medical Colleges**  
655 K Street, N.W., Suite 100, Washington, D.C. 20001-2395  
T 202 828 0400 F 202 828 1125  
www.aamc.org

May 19, 2015

The Honorable Fred Upton  
Chair  
Committee on Energy and Commerce  
U.S. House of Representatives  
Washington, D.C. 20515

The Honorable Joe Pitts  
Chair  
Subcommittee on Health  
Committee on Energy and Commerce  
U.S. House of Representatives  
Washington, DC 20515

The Honorable Diana DeGette  
Ranking Member  
Subcommittee on Oversight and  
Investigations  
Committee on Energy and Commerce  
U.S. House of Representatives  
Washington, DC 20515

The Honorable Frank Pallone, Jr.  
Ranking Member  
Subcommittee on Health  
Committee on Energy and Commerce  
U.S. House of Representatives  
Washington, DC 20515

The Honorable Gene Green  
Ranking Member  
Subcommittee on Health  
Committee on Energy and Commerce  
U.S. House of Representatives  
Washington, DC 20515

Dear Chairmen Upton and Pitts and Representatives DeGette, Pallone, and Green:

The Association of American Medical Colleges (AAMC) is pleased to provide some preliminary thoughts on the amendment in the nature of a substitute to the 21<sup>st</sup> Century Cures Act released on May 19. The AAMC represents all 141 accredited U.S. and 17 accredited Canadian medical schools; nearly 400 major teaching hospitals and health systems, including 51 Department of Veterans Affairs medical centers; and nearly 90 academic and scientific societies. Through these institutions and organizations, the AAMC represents 148,000 faculty members, 83,000 medical students, 115,000 resident physicians, and thousands of graduate students and postdoctoral scientists. More than 50 percent of the extramural funding awarded by the National Institutes of Health (NIH) supports groundbreaking medical research at AAMC-member medical schools and teaching hospitals.

The AAMC applauds you for producing legislation that continues the bipartisan and open manner in which the 21<sup>st</sup> Century Cures initiative has been conducted, and that addresses many of the issues we raised with the earlier draft.

We commend you especially for including language that would reauthorize the National Institutes of Health (NIH) for three years at funding levels that represent an increase of \$1.5 billion per year, and for proposing \$10 billion over the next five years in mandatory funding through an NIH Innovation Fund. This is a most welcome infusion of funding that will help revitalize our nation's biomedical research effort, and demonstrates an opportunity to facilitate sustainable, predictable, long-term growth for the agency. We are deeply grateful for this recognition of the critical importance of maintaining NIH as a national priority.

In addition, we renew our recommendation that NIH be granted multi-year budget authority to carry over funding into the next fiscal year and enable more strategic management of grant funding, particularly in years when appropriations are not finalized until late in the fiscal year.

At the same time, we note the current proposal mandates a number of new responsibilities and activities to the Food and Drug Administration (FDA) without a concomitant increase in funding, and we encourage you to consider the need for additional resources to enhance the agency's capacity for regulatory science and to address the additional mandates included in the legislation.

In our comments of March 18 on the earlier discussion draft, we urged the committee to ensure that the bill presents a comprehensive vision for the funding and regulation of medical research and is internally consistent. We are heartened by the bill in its current form, which organizes its proposals into the three broad categories of Discovery, Development, and Delivery.

In particular, we concur with NIH's support for the proposals in the proposed legislation to enhance accountability, and we believe that these proposals will assist the agency's efforts to invest in the highest research priorities, foster creative collaborations, and sustain the biomedical research workforce.

The AAMC believes the revisions proposed for the NIH Strategic Plan required in section 1021 are appropriate and will better coordinate the overall NIH plan with the strategic planning that is already occurring within the Institutes and Centers.

We acknowledge and thank you for deleting the provision in the initial discussion draft within the NIH Research Strategic Investment Plan (Section 4001) requiring the Director of NIH to ensure at least 55 percent of extramural research funding goes to support basic biomedical research.

The AAMC welcomes the proposal to ease the administrative burden on NIH, and supports section 1024, which would exempt certain NIH research activities from the requirements of the Paperwork Reduction Act.

We acknowledge the "sense of Congress" expressed in section 1025 "that participation in or sponsorship of scientific conferences and meetings is essential to the mission of the National Institutes of Health." We encourage the committee to exempt NIH from OMB Memo 12-12 to help build and maintain the connections within and across disciplines that do help drive research innovation.

The AAMC commends and supports section 1124, which mandates the Secretary to review or clarify regulations under the Health Insurance Portability and Accountability Act (HIPAA) for conducting research. In particular, we appreciate that the legislation allows the use and disclosure of protected health information (PHI) by a covered entity for research purposes to be treated as health care operations; lets researchers access data remotely for “reviews preparatory to research” without authorization (currently, they must be physically on site to look at medical records to determine if research is feasible); and allows a one-time authorization of use and disclosure for future research (currently prohibited). All of the proposed revisions would be beneficial and remove barriers to research without jeopardizing or disadvantaging patients or research subjects.

The AAMC appreciates the language in section 3041 to exempt certain transfers for educational purposes from the manufacturers’ transparency requirements. This language appears to address concerns that have been raised with the chilling effect the current reporting requirements might have on legitimate continuing medical education (CME) programs.

We also appreciate that the legislation does not include language regarding the 340B Drug Pricing Program at this time. As you know, the Health Resources and Services Administration (HRSA) is expected to release comprehensive guidance that would address many components of the program and would provide the full stakeholder community an opportunity for public comment. We believe it would be premature to issue legislation on an administratively complex program like 340B in advance of this opportunity, particularly as part of the Cures initiative that has demonstrated a commitment to an open, collaborative, and transparent process.

We continue to review other provisions of the legislation with our members, and look forward to working with you as this legislation moves forward.

Again, the AAMC thanks you and your staff for your tireless efforts to identify opportunities to accelerate scientific discovery in the service of improved health, and we look forward to working with you as this legislation moves forward. Should you or your staff wish to discuss any of these points, please contact David Moore, AAMC Senior Director for Governmental Relations, at 202-828-0559 or [dbmoore@aamc.org](mailto:dbmoore@aamc.org).

Sincerely,



Atul Grover, M.D., Ph.D.  
Chief Public Policy Officer

cc: House Energy and Commerce Members

**OFFICERS:**

**President**

Steven L. D'Amato, BSPHarm, BCOP  
New England Cancer Specialists  
Scarborough, Maine

**President-Elect**

Jennie R. Crews, MD, FACP  
PeaceHealth St. Joseph Medical Center  
St. Joseph Cancer Center  
Bellingham, Washington

**Treasurer**

Thomas A. Gallo, MS  
Virginia Cancer Institute  
Richmond, Virginia

**Secretary**

W. Charles Penley, MD  
Tennessee Oncology  
Nashville, Tennessee

**Immediate Past President**

Becky L. DeKay, MBA  
University Health Shreveport  
Shreveport, Louisiana

**TRUSTEES**

Nicole A. Bradshaw, MS, MBA  
St. Luke's Mountain States Tumor Institute  
Nampa, Idaho

Catherine Brady-Copertino, BSN, MS, OCN  
Anne Arundel Medical Center,  
DeCesaris Cancer Institute  
Annapolis, Maryland

Neal Christiansen, MD  
Robert H. Lurie Comprehensive Cancer  
Center at Northwestern Medical Faculty  
Foundation  
Lake Forest, Illinois

Faye Flemming, RN, BSN, OCN  
Southside Regional Medical Center  
Petersburg, Virginia

Colleen Gill, MS, RD, CSO  
University of Colorado Cancer Center  
Aurora, Colorado

John E. Hennessy, MBA, CMPE  
Sarah Cannon Cancer Center  
Kansas City, Missouri

Ali McBride, PharmD, MS, BCPS  
The University of Arizona Cancer Center  
Department of Pharmacy  
Tucson, Arizona

Randall A. Oyer, MD  
Lancaster General Hospital  
Lancaster, Pennsylvania

Mark S. Soberman, MD, MBA, FACS  
Frederick Regional Health System  
Frederick, Maryland

Cecilia R. Zapata, MS, MHA  
Seattle Cancer Care Alliance  
Seattle, Washington

**EXECUTIVE DIRECTOR**

Christian G. Downs, JD, MHA

May 21, 2015

The Honorable Fred Upton  
Chairman  
House Energy & Commerce Committee

The Honorable Frank Pallone  
Ranking Member  
House Energy & Commerce Committee

**Re: 21<sup>st</sup> Century Cures**

Dear Chairman Upton and Ranking Member Pallone,

The Association of Community Cancer Centers (ACCC) commends the Energy and Commerce Committee for passing HR 6, 21<sup>st</sup> Century Cures legislation. ACCC is committed to improving health outcomes for cancer patients. HR 6 is a tremendous step toward advancing research in the field of oncology and healthcare, as well as improving the process for bringing new technologies into oncology programs nationwide.

ACCC members include hospitals, physicians, nurses, social workers, and oncology team members who care for millions of patients and families fighting cancer. ACCC represents more than 20,000 cancer care professionals from approximately 1,100 hospitals and more than 1,000 private practices nationwide. These include Cancer Program Members, Individual Members, and members from 32 state oncology societies. It is estimated that 60 percent of cancer patients nationwide are treated by a member of ACCC.

Please feel free to contact Leah Ralph, Manager, Provider Economics and Public Policy, at (301) 984-5071 if you have any questions or need any additional information. Thank you again for your attention to this very important matter.

Respectfully submitted,



Steven D'Amato, BSPHarm, BCOP  
President  
Association of Community Cancer Centers



May 19, 2015

Chairman Fred Upton  
House Energy and Commerce Committee  
Subcommittee on Health  
2125 Rayburn House Office Building  
Washington, DC 20515

Ranking Member Frank Pallone  
House Energy and Commerce Committee  
Subcommittee on Health  
2125A Rayburn House Office Building  
Washington, DC 20515

Dear Chairman Upton and Ranking Member Pallone,

The American Society for Radiation Oncology (ASTRO), representing more than 10,000 radiation oncology medical professionals treating more than 1 million Americans with cancer each year, is encouraged by the potential benefits of the House Energy and Commerce Committee's comprehensive approach in the 21<sup>st</sup> Century Cures legislation, which identifies methods to accelerate the pace of curing diseases in America. ASTRO also is working to improve the quality of cancer care to bring us closer to a cure, and therefore we urge the Committee to consider suggested improvements to the Cures' legislation, including increased funding for radiation oncology research and other policy recommendations below.

**Title I—Discovery, Subtitle A – National Institutes of Health Funding Section 1001 –National Institutes of Health Reauthorization**

ASTRO commends the Committee for increasing funding for the National Institutes of Health (NIH) for precision medicine and to ensure that young scientists have a secure future in biomedical research.

As you know, Congress has demonstrated longstanding support for NIH and cancer research and ASTRO is committed to accelerating recent advances. With your support, major advances in cancer diagnosis and treatment, including radiation oncology, are happening at a faster pace than ever. ASTRO awards nearly \$1 million each year to fund research as part of our overall effort to prevent, treat and cure cancer. These research awards and grants support work in radiation and cancer biology, radiation physics, comparative effectiveness research, translational research and outcomes/health services research. We are proud to do our part to support cancer research funding and applaud the Committee for recognizing and pledging to fill its irreplaceable role supporting the important work of the NIH.

**Subtitle B – National Institutes of Health Planning and Administration** ASTRO was disappointed that the legislation does not include language to address funding levels for radiation therapy-related research at the National Institutes of Health (NIH) and National Cancer Institute (NCI). In a 2013 report to Congress, NIH acknowledged that less than one percent of its total budget was spent on radiation oncology specific research and just over four percent of the NCI’s budget went toward radiation oncology research. With more than half of cancer patients receiving radiation therapy as a part of their cancer treatment, the funding for radiation oncology research is not adequate to achieve new discoveries in the field. We are deeply concerned that this lack of radiation oncology research funding is resulting in promising young researchers leaving the field.

As part of Congress’ oversight duties to know how NIH research funds are allocated and to ensure that funding levels are appropriate, ASTRO urges you to include the attached language in the final legislation. We believe ensuring appropriate funding levels of research projects related to radiation oncology will create a clearer understanding of NIH’s priorities.

**Title II—Development, Subtitle L – Priority Review for Breakthrough Devices Section 2201**

ASTRO applauds the Committee’s commitment to require the Food and Drug Administration (FDA) to fast-track the approval process for so-called “breakthrough devices” that could have a direct impact on patient outcomes. However, we urge the Committee to include a definition of a breakthrough device, without which FDA may have difficulty in promulgating rules surrounding these types of devices.

Additionally, the draft language does not include additional appropriations for the FDA to carry out its new regulatory authority. ASTRO urges the Congress to provide appropriate funds for the agency to effectively comply with the requirements set forth in the draft legislation.

**Title III, Subtitle C – Encouraging Continuing Medical Education for Physicians, Sec. 3041. Exempting From Manufacturer Transparency Reporting Certain Transfers Used for Educational Purposes**

ASTRO supports the language of Section 3041, which would exempt from reporting under the Open Payments program transfers of value in the form of peer reviewed journals, journal reprints and supplements, medical conference reports and medical textbooks, which are given by manufacturers to physicians. All of these items are aimed at improving physician education and patient care and carry with them certain assurances that they will not be mere marketing materials for manufacturers. These items should be exempt from reporting, whether they are for physician use or for the physician to share with his/her patients.

Section 3041 would also exempt payments that manufacturers give in support of independent certified and/or accredited continuing education programs, such as those held by ASTRO, that ultimately fund speaker honoraria or tuition support for attendees. As a direct provider of accredited continuing educational programs and a leader in the professional and educational development for the radiation oncology community, ASTRO follows strict policies to avoid commercial influence on its educational content. ASTRO complies with the Standards for Commercial Support: Standards to Ensure Independence in CME Activities, which were promulgated by ACCME and adopted by the US continuing medical education (CME) credit systems currently named in the regulations. These standards provide

the framework for independent continuing education and distinguish rigorous continuing education from promotional sessions sponsored by manufacturers. Payments that ultimately support physicians who participate in ASTRO's programming, whether as speakers or attendees, should not be reported under the Open Payments. For these reasons, ASTRO supports Section 3041.

**Subtitle E—Local Coverage Determination Reforms**

ASTRO is pleased that the Committee has included language that would increase transparency around the Local Coverage Determination (LCD) process by amending Sec. 1874A(g) of the Social Security Act to require public notification of new and revised LCDs at least 45 days prior to the effective date. ASTRO encourages the Committee to consider establishing a comment period similar to CMS' proposed rule process to ensure that all stakeholders benefit from increased transparency.

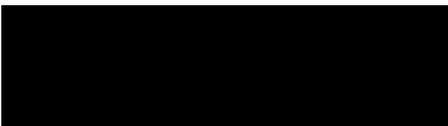
**Subtitle G—Facilitating Collaborative Research, Section 1121, Clinical Trial Data System**

ASTRO commends the Committee's intention to create a third-party scientific research sharing system for clinical trials funded by the government to ensure that the findings of those trials are available to the public. As specifics for this system are built, ASTRO encourages the Committee to ensure that all physician specialties are represented, including all three segments of oncology (radiation, medical, surgical) in the consultation. Additionally, ASTRO urges the Committee to ensure this new system is compatible with vendors and eHealth systems of radiation oncology for true integration.

ASTRO also commends your longstanding support of the radiation oncology community and cancer patients nationwide. As the Committee examines policies to offset the costs of this important legislation, we ask you to continue protecting cancer patients' access to radiation treatment and consider using the \$3.5 billion in savings achieved by legislation to close the physician self-referral law's loophole.

In conclusion, ASTRO supports the Committee's work on these very important issues and urges inclusion of the above policy recommendations. Thank you for your work on behalf of the health of Americans. We welcome the opportunity to discuss these issues in detail. Please feel free to contact Shandi Barney, Congressional Relations Manager for ASTRO at 703-839-7382 or [shandi.barney@astro.org](mailto:shandi.barney@astro.org) if you have any questions.

Sincerely,



Laura I. Thevenot  
Chief Executive Officer



**VINCENT MOR, PHD**  
Professor of Medical Science  
Florence Pirce Grant University Professor  
Health Services, Policy & Practice

The Honorable Fred Upton, Chairman  
The Honorable Frank Pallone, Ranking Member  
Energy and Commerce Committee  
United State House of Representatives  
2125 Rayburn House Office Building  
Washington, DC 20515

May 17, 2015

Dear Chairman Upton and Ranking Member Pallone:

The Brown University School of Public Health and particularly faculty in the Department of Health Services, Policy & Practice thanks you and your Committee members for your leadership in advancing the 21<sup>st</sup> Century Cures initiative. We are especially pleased to support the provision adding 'research' to the definition of 'health care operations'. This will allow unprecedented and assured access to HIPAA-protected data so that investigators can better understand how care in all settings affects the outcomes and needs of all patients. Today, this data is often inaccessible and therefore wasted by unworkable requirements to obtain individual consent for every single use of clinical records for research purposes.

This change is long overdue. Efficient access to records by researchers while maintaining patient privacy will unlock a information on how care is delivered in different places to different patients, including smaller care settings where much care across the nation is delivered today. These data include people with multiple comorbidities who are increasingly common as a result of our aging population. Academic clinical trial sites commonly exclude such patients. Without access to data on the care and outcomes experienced by such patients we will not be able to systematically examine which treatments are most effective for which types of patients.

The evidence we can gain from accessing such rich data sources touches all levels and kinds of biomedical and health research from supporting hypothesis generation for translational research to making clinical care process improvements and using this information relating to access, quality, and safety of care for public health advancements, including the development of better measures for evaluation and continuous improvement. This is particularly important as insurers are increasingly considering strategies that would pay hospitals and physicians for how well their patients do as a result of their treatment. Without well documented we can't develop and rigorously test the kinds of measures needed to make sure that providers performance can be legitimately compared.

Similarly, we support the language that seeks to remove the duplicative research reviews by both the FDA and OHRP for some clinical trial research. This duplication is confusing at the minimum. Furthermore, the capacity of the FDA to perform inspections puts them in the best position to assure appropriate patient protections without introducing another level of bureaucratic oversight.

Thank you for your important leadership. We wish to assure that Committee that HIPAA will not be weakened by this legislation which is certain to improve the nation's capacity to deliver better care at lower cost. Please contact me at 401-863-3172 if you would like follow up on these comments.

Sincerely,



Vincent Mor, PhD



Give **Kids** a Chance to Grow Up

May 18, 2015

**CancerFree KIDS** is a non-profit organization committed to funding innovative research on childhood cancers so that one day every child will be cancer-free. We enthusiastically support Sections 2082 and 2083 of the 21st Century Cures bill and encourage its passage so that children will have access to better treatments and cures.

Thank you, on behalf of the childhood cancer community and the parents of children with cancer everywhere.

Sincerely,

Ellen M. Flannery  
Founder & Executive Director

---

CancerFree KIDS • 420 W. Loveland Avenue • Loveland, OH 45140 • (513) 575-5437 • [info@cancerfreekids.org](mailto:info@cancerfreekids.org)





**Kristen D.W. Morris**  
Chief Government and Community  
Relations Officer

May 18, 2015

The Honorable Fred Upton  
Chairman  
US House of Representatives Committee on Energy and Commerce  
2125 Rayburn House Office Building  
Washington, DC 20515

Dear Chairman Upton,

The Cleveland Clinic would like to thank you and the House Energy and Commerce Committee for the opportunity to provide comment and feedback regarding your May 13, 2015 discussion draft, 21<sup>st</sup> Century Cures Act.

Cleveland Clinic is a not-for-profit, integrated healthcare system and academic medical center dedicated to patient care, teaching and research. Our health system is comprised of a main campus, eight community hospitals and 18 family health centers with over 3,000 salaried physicians and scientists, and nearly 44,000 employees. Last year, our system had nearly five million patient visits and over 157,000 hospital admissions. We appreciate the dedication of the Committee members and staff to protecting the safety, quality and value of the American health care system, and to ensuring that each provider in that system has all of the necessary tools to deliver care in the right time and place for each patient.

We want to thank the Committee for its exceptional thoughtfulness and dedication to excellence in patient care and safety and to the development of new therapies and cures to help ensure excellence in health for all Americans. We appreciate the time and effort that are reflected in this document. In general, we wish to add Cleveland Clinic's voice to the overwhelming chorus of support for both the intent and the content of this document

## **TITLE II: DEVELOPMENT**

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

This provision would require the Secretary of Health and Human Services to develop a process for collection, analysis and application of patient experience data to the development of structured risk-benefit framework for identification, funding and evaluation of research and development of new therapeutics to treat disease.

Cleveland Clinic believes that the voice of patients and family caregivers – those most directly affected by human health conditions – has been largely missing from the process of prioritizing medical research and therapy development. We salute the Committee’s recommendation to make patient reported outcomes and patient experience a strong component of critical evaluations by the National Institutes of Health, the Centers for Medicare and Medicaid Services and the Food and Drug Administration. We further encourage the development of most relevant patient measures, often called MCIDs or Minimally Clinically Important Differences, for quantitative evaluation of treatment effectiveness.

However, we do question how the proposed framework will integrate with existing efforts to better incorporate and respond to patient experience data, such as the Patient Reported Outcomes program of the National Quality Forum, the NIH PROMIS (Patient Reported Outcomes Information System) and most recently, Patient Reported Outcomes Research Institute created by the Affordable Care Act in 2011. We would strongly encourage that any stakeholder meetings and advisory boards be required, in their recommendations, to explain how any new programs will leverage, integrate with, and avoid duplication with, the efforts of these existing programs.

### **Sections 2241 and 2242: Protection of Human Subjects in Research; Applicability of Rules and Use of Non-Local Institutional Review Boards for Review of Investigational Device Exemptions and Human Device Exemptions.**

As an academic medical center heavily involved in clinical research, Cleveland Clinic is always sensitive to delays and administrative burden inherent in conduct of these trials when balanced with ensuring adequate human subjects protection. Cleveland Clinic has taken a strong position in the past in favor of maintaining separate Institutional Review Board (IRB) review and consent at each research site where clinical trials are conducted

The language proposed here is the latest in a series of proposals with the intent to reduce administrative burden associated with conduct of clinical trials. In reality the vast majority of IRBs function efficiently, and return approval in 30 days or less. While this proposed consolidation of IRBs may benefit some smaller research sites, we are concerned that the implementation will require additional infrastructure at a considerable cost and time to both the lead site and the relying sites and may delay the initiation of research at some participating sites. For example, creating a single IRB model requires establishing individual IRB authorization agreements with

each of the relying sites and will take considerable time and effort to complete but more importantly, both the lead and the relying sites will need to modify or create new infrastructure and technology to implement this arrangement. The relying sites cannot abandon their regulatory obligations and ethical responsibilities for oversight, tracking and monitoring. The cost to upgrade staffing and technology could be considerable and take considerable time to implement. The additional cost to serve as the single IRB could run around \$50,000-80,000 per project and could reduce direct cost available for conduct of the research.

We believe the NIH should consider creating a separate human subjects review group to complement the scientific review. This review would focus on the assessment of research risk and benefits and develop an informed consent document in accordance with IRB approval criteria. This information would then be provided to each of the multi-site IRBs to assist with their IRB review. Inconsistency in IRB reviews is often the result of Investigators giving inaccurate or inconsistent information. A central human subjects review would provide consistency that could streamline the site IRB process without dividing their responsibilities or degrading the existing comprehensive human subject protection program of efficient IRBs. Ceding IRB review is contrary to developing an IRB culture of a shared responsibility working with Investigators and the Research team to protect research participants. IRBs that present significant administrative burden should be addressed by requiring specific corrective action plans to resolve their inefficiencies.

### **TITLE III: DELIVERY**

#### **Section 3001: Interoperability**

Cleveland Clinic has made integration of health information through the Electronic Health Record (EHR) a high priority across its health system. We have commented elsewhere about some of the specific challenges inherent in implementation of EHR requirements under Meaningful Use and our strong advocacy for interoperability among EHR vendors. We are aware that the American Hospital Association has commented on this subject at length and in particular, we voice strong support for its recommendation that EHR interoperability be facilitated through the National Institute of Standards and Technology.

With regard to the language proposed under section 3010 regarding individual health system and hospital attestation to ensure that individual systems do not to limit or obfuscate sharing of data to support care, we echo the AHA's assertion that there are manifold and compelling incentives to share data for the best interest of patients. However, in the absence of a data sharing infrastructure and industry standard to support this mission, compulsory attestation and compliance may be significantly impeded and unfairly punitive.

#### **Section 3021: Telehealth**

The Cleveland Clinic wishes to voice its strong support for this bold position on expanded use of telehealth services. Further, we are encouraged by the Sense of the Congress that diligent and

safe provision of distance health services needs to be accompanied by a set of national standards and compacts for licensure that ensure consistency and quality across multi-state practices.

**Section 3151: Programs to Prevent Prescription Drug Abuse under Parts C and D**

As a health care provider who cares for patients with a range of chronic and often painful diseases and trauma, The Cleveland Clinic is keenly sensitive to the balance between adequate relief of pain and potential for abuse of the medications that provide that relief. We applaud the intent of this section, which calls for improved coordination between providers to reduce fraudulent prescribing of drugs of abuse. However, the language as currently written does present cause for concern. At present the language calls upon each Prescription Drug Program (PDP) to establish guidelines for identification of beneficiaries who are at risk for abuse, either because of their history or because of the medications they have been prescribed. This system has the potential to invite confusion or potential misapplication of the statute, as it will be left to each PDP to determine the criteria for this beneficiary class. A beneficiary who changes Medicare Advantage (MA) plans could be faced with being classified in one manner in his or her old plan and being re-classified in the new plan. Further, the language does not anticipate exceptions for terminal patients and those transitioning from acute to hospice care and could have the unanticipated consequence of delaying or denying comfort medications to the dying. Cleveland Clinic suggests that an expert panel be convened to develop consistent guidelines for identification of at-risk beneficiaries and that this definition be applied consistently across PDPs. Further, we suggest that this same panel be charged with identifying exceptions to the classification system (such as for terminally-ill patients).

Cleveland Clinic has, in prior comments and public statements, sought to underscore the effect of the HCHAPS survey questions on patient expectations and perceptions of pain control, and the possible deleterious effect on subsequent provider prescribing behavior. Ultimately, it is the decision of the provider whether and how to prescribe pain relief medications, including those on the FDA Schedules of Controlled Substances. However, it is naïve to think that patient expectations of pain relief will not have an effect on whether, how much and which medications are prescribed, especially when providers are cognizant that their reimbursement may be dependent in part on fulfilling those expectations. For this reason, we have communicated strongly with CMS and the National Quality Forum to modify the HCHAPS survey to focus more strongly on communication with patients about pain and properly setting expectations, rather than focus solely on pain control efforts. We wish again to express that any program to control misuse of Schedule medications must begin and end with the patient-provider relationship and that education must be at the heart of those programs.

Finally, control and regulation of access to potentially addictive medications is only part of the strategy needed to curb the abuse of prescription pain relievers. In addition to the programs proposed in this subtitle, we would propose, as a section within Subtitle A of Title II, specific creation of funded programs for development and testing of alternative pain-relief strategies that are effective and non-addicting, such as pain mitigation implants and devices, leveraging of

alternative pathways for neuropathic pain, and alternatives for opioids for relief of inflammatory and traumatic pain.

Again, we are grateful to the Committee for the opportunity to provide these comments on the discussion draft and welcome further dialog. If the Committee has questions or wishes for clarification to any of the items here, feel free to contact me, or Carlos Jackson, Senior Director of Government Relations (216-448-1200; [jacksoc7@ccf.org](mailto:jacksoc7@ccf.org))

Respectfully submitted,



Kristen D.W. Morris  
Chief Government and  
Community Relations Officer

May 21, 2015

The Honorable Fred Upton  
Chairman  
House Energy & Commerce Committee

The Honorable Frank Pallone  
Chairman Ranking Member  
House Energy & Commerce Committee

**Re: Support for Section 2228 in 21<sup>st</sup> Century Cures**

Dear Chairman Upton and Ranking Member Pallone:

On behalf of the Coalition for CLIA Waiver Reform, I am writing to express the Coalition's appreciation for the Committee including Section 2228 in the 21<sup>st</sup> Century Cures Act (HR.6) which calls on FDA to revise part of its current guidance for evaluating CLIA waiver applications.

As you know, point-of-care testing, or "POCT," is a cornerstone of patient care, and in coming years will play an increasingly important role in improving patient outcomes and the public health. POCT's benefits are intuitive: results are received in the exam room or at the patient bedside, speeding diagnosis and treatment decisions and reducing instances in which patients get tested but never return to receive their results (a common occurrence with dangerous consequences).

Roughly 80% of POCT facilities are so-called "Certificate of Waiver" laboratories, which include physician offices, health clinics, urgent care centers and other points of care. By law, Certificate of Waiver laboratories may only use FDA-approved/cleared *in vitro* diagnostic tests ("IVD") which are designated as low ("waived") complexity by FDA. Therefore, expanding the number of CLIA-waived tests is essential to realizing the full benefits of POCT.

Unfortunately, the current FDA guidance's interpretation of the standards for granting CLIA waivers differs from standards that Congress set out in 1997 as part of the FDA Modernization Act ("FDAMA"). As a result, the FDAMA standards that Congress enacted to encourage development of waived tests are not having their intended effect, and patients are not getting access to the point-of-care testing options they deserve.

Section 2228 is a significant step forward toward improving the CLIA waiver process. Through its enactment, Section 2228 would make CLIA waiver reform a priority within FDA. Further, the guidance process mandated by the legislation would provide an opportunity for stakeholders from the patient, medical, and IVD innovator communities to come together and work with FDA to develop guidance that is consistent with FDAMA and will advance the public health.

Chairman Upton and Ranking Member Pallone  
May 21, 2015  
Page 2

We thank the Committee for its excellent work on this issue, and stand ready to assist in any way we can to advance this important issue.



James A. Boiani  
General Counsel  
Coalition for CLIA Waiver Reform

cc: Members of House Energy and Commerce Committee



# Coalition for Clinical Trials Awareness

April 28, 2015

The Honorable Fred Upton  
Chairman  
Committee on Energy and Commerce  
Washington, DC 20015

The Honorable Diana DeGette  
Member  
Committee on Energy and Commerce  
Washington DC 200015

Re: Inclusion of Public Awareness Campaign on Clinical Trials in the 21<sup>st</sup> Century Cures Initiative

Dear Chairman Upton and Representative DeGette:

We are writing on behalf of the Coalition for Clinical Trials (CCTA), a national group of patient, provider and research organizations advocating for a federally sponsored public awareness campaign to increase the public's understanding of the benefits of clinical trials.

We thank you for your continued leadership on the 21st Century Cures initiative. The members of CCTA share your commitment to this initiative's goal: to take a comprehensive look at how Congress can accelerate the pace of cures in America through medical innovation.

One major source of delay for the development of new medical breakthroughs is the lack of awareness and participation in clinical trials. Yet clinical trials are an essential step in bringing new drugs, biologics, and medical devices to patients. Every modern medical treatment that we have today for conditions such as cancer, arthritis, and Parkinson's disease was first proven safe and effective through clinical trials.

For years we have known that the public does not understand that clinical trials are an essential step for new medical treatments. A 2003 article published in the Journal of Clinical Oncology revealed that 40% of surveyed adults did not understand the idea of a clinical trial. And because the public is unaware of the benefits of clinical trials, many trials fail due to lack of enrollment. A recent study conducted by the Tufts Center for the Study of Drug Development involving 150 clinical trials and nearly 16,000 study sites found that 11% of sites fail to enroll even one patient. Thirty-seven percent do not meet their enrollment goals.

The value that new treatments provide for public health makes more robust clinical trials a societal imperative. In fact, we perceive many similarities between the current need for improved clinical trials enrollment and the late 20th century need for organ donation. The latter prompted a 1990s federal public awareness campaign, Donate Life, to increase the public's awareness about organ donation. The campaign highlighted not only the need for organ donation but also the benefit that one person could provide another, and society as a whole, by registering to donate organs. By applying a similar approach, the federal government could once again spur citizens to act in the interest of their neighbors and the greater national community - this time by enrolling in a clinical trial.

2000 M Street, NW, Suite 850, Washington, D.C. 20036  
888-507-5675  
[contact@cctawareness.org](mailto:contact@cctawareness.org)

Therefore, in addition to considering regulatory reforms for clinical trials, CCTA urges you to also support a federally sponsored public awareness campaign to increase the public's understanding of clinical trials and their benefits. We would propose that the following elements for this campaign:

- *Creation of an Advisory Council on Clinical Trials Awareness* – Membership would include Federal agencies with regulatory and financing interests in clinical trials, private sector experts, clinical research groups, health care provider groups, and patient advocacy organizations;
- *Creation and dissemination of educational materials* – The branded education materials would tout the importance of clinical trials and urge patient participation; and
- *Financing and undertaking of a public service campaign to promote awareness* – This multi-year public service campaign would highlight clinical trials and the need for participation, similar to other health care public service initiatives in the past.

Increasing the number of people willing to consider participating in clinical trials will lead to increased trial enrollment. More efficient clinical trials will save money, increase drug development opportunities, and give patients and health care providers more options.

We appreciate the opportunity to share our comments with you on these issues, and we thank you again for your commitment to the discovery, development and delivery of innovative health care products and services. We look forward to continuing to work with you regarding the 21<sup>st</sup> Century Cures initiative.

Sincerely,

David

David Charles, MD  
Chair  
Coalition for Clinical Trials Awareness

John

John Barnes  
Executive Director  
Coalition for Clinical Trial Awareness

May 19, 2015

Dear Members of the United States House of Representatives:

The undersigned organizations represent healthcare providers, clinical researchers, public health experts, and consumer advocates. We thank you for this opportunity to comment on the 21<sup>st</sup> Century Cures draft legislation. Acknowledging the need for increased discovery, development, and distribution of new treatments for a variety of diseases, **we are concerned that the proposed legislation as written fails to ensure a comprehensive and scientifically based approach that supports patients' access to affordable treatments.** Instead, the draft legislation would allow for unsafe and ineffective drugs and medical devices to enter the market while further limiting access to effective medicines for patients.

Although we strongly support increases in funding for the National Institutes of Health (NIH), this positive component of the draft legislation comes at the expense of too many provisions that we cannot support. Moreover, the authorization of NIH funding increases does not guarantee the appropriation of such funds.

Rather than addressing the true scientific bottleneck in drug and device development, the bill includes unnecessary, costly, and potentially harmful regulatory changes and financial incentives for pharmaceutical and medical device companies that would put patient safety at risk and undermine public health. **We therefore are unable to support the current version of the 21<sup>st</sup> Century Cures Draft legislation.**

Our specific concerns regarding the 21<sup>st</sup> Century Cures draft legislation are as follows:

**1. 21<sup>st</sup> Century Cures would further undermine the FDA's ability to ensure the safety and efficacy of medical devices (Sections 2222 and 2221).**

Section 2222 would allow for new high-risk medical devices to be approved by the FDA based on case studies or medical journal articles alone. High-risk devices should not be approved on the basis of uncontrolled case studies of just one, two, or even a series of patients (in essence, clinical anecdotes). Medical journal articles often leave out critical information because of space limitations or because concerns that admitting shortcomings in study design or conduct will make it difficult to get the article published. A recent study found that out of 78 clinical trials for which FDA inspectors identified significant research misconduct (including involving submission of false information), the associated journal article reported the misconduct in only three cases.<sup>1</sup> Journal editors and peer reviewers rely on the accuracy and integrity of the authors; they do not examine raw data or inspect clinical trial sites. Given this lack of oversight, it is not surprising that a disturbing number of articles

---

<sup>1</sup> Seife C, Research misconduct identified by the US Food and Drug Administration: Out of sight, out of mind, out of the peer-reviewed literature. *JAMA Intern Med.* doi:10.1001/jamainternmed.2014.7774. Published online February 9, 2015.

are later found to be inaccurate, misleading, or fraudulent.<sup>2</sup> FDA reliance on journal articles as the sole basis for device approvals, as permitted under Section 2222, could prevent the FDA from learning of important problems with clinical testing, which could lead to serious patient harm.

In addition, Section 2221 would allow companies to make changes to even the highest-risk devices (like artificial heart valves) without first notifying the FDA or documenting that the modified device remains safe and effective. Instead, device manufacturers would pay third-party contractors to certify that the manufacturer had an adequate “quality system,” after which the manufacturer would be authorized to determine for itself whether each device remained safe and effective following important changes. Changes to high-risk devices can be dangerous, as illustrated by recent cases of massive bone and tissue damage caused by changes to the materials used in certain “metal on metal” hip implants.<sup>3</sup> These changes should not be exempted from FDA oversight.

**2. 21<sup>st</sup> Century Cures would allow for antibiotics and antifungals to be approved based on lower FDA standards, putting patients at risk of being treated with unsafe and ineffective drugs (Section 2121).**

Included in the legislation is the *Antibiotic Development to Advance Patient Treatment (ADAPT) Act* (Section 2121), which presents a fast-track pathway for FDA drug approval based on surrogate clinical endpoints and data from animals, test tubes, mathematical modeling, and small, early-stage clinical trials in humans with diseases, rather than larger, later-stage trials. Results from clinical trials based on surrogate clinical endpoints must be confirmed with phase III trial data. Data from non-clinical trials or early, small-scale clinical trials can offer misleading evidence of efficacy or miss important safety risks. Approving antibiotics based solely on this evidence violates the FDA’s mission to protect public health by ensuring the safety and efficacy of these drugs. The provision could also allow the FDA to approve drugs based on preclinical data that actually show the drugs to be inferior to existing drugs. Drugs approved by the FDA should improve efficacy and/or decrease harm to patients, and/or otherwise meaningfully improve therapy.

FDA regulations already give the agency the authority to expedite drug approval for limited, well-defined sets of patients. Studies have shown that more than half of all newly approved novel drugs already receive the benefit of at least one special expedited development or review designation, making another pathway unnecessary.<sup>4</sup> In fact, compared to other drug classes, antibiotics already have a higher rate and speed of approval.<sup>5</sup> In addition, the agency has just issued a new draft guidance to expedite the “compassionate use” of investigational drugs for individual patients. Physicians can make requests far more readily than in the past

---

<sup>2</sup> Many of these issues, including inaccuracies, misleading information, and fraud, have been documented by bloggers Adam Marcus and Ivan Oransky on the blog: Retractionwatch.com.

<sup>3</sup> Meier B, Concerns over “metal on metal” hip implants. New York Times. March 3, 2010. <http://www.nytimes.com/2010/03/04/health/04metalhip.html>. Accessed May 19, 2015.

<sup>4</sup> Kesselheim, A. S., & Darrow, J. J. (2015). FDA Designations for Therapeutics and Their Impact on Drug Development and Regulatory Review Outcomes. *Clinical Pharmacology & Therapeutics*, 97(1), 29-36.

<sup>5</sup> DiMasi, J.A., Success rates for new drugs entering clinical testing in the United States. *Clin Pharmacol Ther*, 1995. 58(1): p. 1-14.

for individual patients with unmet medical needs who are willing to take an informed risk while the drugs are still being studied for the wider public. Patients can obtain access quickly through these expanded access pathways while waiting for appropriate clinical trials data to be obtained. This mechanism protects the broader public.

As ADAPT would fail to truly address antibiotic resistance and would lower FDA standards for approving antibiotics and antifungals, resulting in harm to patients, we strongly urge you to withhold your support for this legislation. Instead, we urge you to consider a broader, more effective approach such as that in the newly-released House bill, the *Helping Effective Antibiotics Last (HEAL) Act*, sponsored by Representatives DeLauro, Schakowsky, and Meng.

**3. 21<sup>st</sup> Century Cures could introduce serious conflicts of interest into the process for defining when bacteria are considered to be antibiotic-resistant, promoting overuse and misuse of new antibiotics and accelerating development of resistance to new antibiotics (Section 2122).**

ADAPT also would allow susceptibility testing of breakpoints for antibiotic resistance to be determined by a “nationally or internationally recognized standard development organization” which may include members that have disclosed potential financial conflicts of interest or ties to the pharmaceutical industry. The statute requires only that the standard development organization establish and maintain “procedures to address potential conflicts of interest and ensure transparent decision-making,” but does not bar conflicted members. Since many conflict of interest policies merely require disclosure of conflicts, but do not bar conflicted members from serving, this language could allow key decisions to be made by a committee for which the majority of members have disclosed potential financial conflicts of interest. For example, currently, 11 of the 14 members of the Subcommittee on Antimicrobial Susceptibility Testing at the Clinical and Laboratory Standards Institute (CLSI), an organization likely to be selected under the proposed new provision, have reported financial conflict of interest. In fact, four out of the 14 members of the subcommittee are pharmaceutical industry employees. This organization has been criticized for adopting antimicrobial susceptibility criteria that expand the definition of “antibiotic resistance” to dramatically increase the use of newer, broader-spectrum antibiotics while offering no improvement in clinical outcomes.<sup>6</sup>

Shifting the goalposts of defining antibiotic resistance in this way would lead paradoxically to greater resistance, by encouraging the unnecessary, increased use of these broader-spectrum antibiotics that should instead be reserved for infections against which they are truly needed. Rather than delegate this work to a committee mainly comprised of conflicted members, the legislation should require that the process for determining antimicrobial susceptibility criteria be transparent, independent of financial conflict of interest, and based on patient-centered outcomes from clinical studies.

---

<sup>6</sup> Tamma PD, Wu H, Gerber JS, Hsu AJ, Tekle T, Carroll KC, Cosgrove SE. "Outcomes of Children with Enterobacteriaceae Bacteremia with Reduced Susceptibility to Ceftriaxone: Do the Revised Breakpoints Translate to Improved Patient Outcomes?" *Pediatr Infect Dis J* 32, no. 9 (2013): 965-9. <http://www.ncbi.nlm.nih.gov/pubmed/23470679>. Accessed May 19, 2015.

**4. 21<sup>st</sup> Century Cures would weaken the reporting requirements under the Physician Payment Sunshine Act, allowing for secret influence from pharmaceutical and medical device companies on the practice of medicine and medical education (Section 3041).**

Section 3041 of the bill would create an exemption under the Physician Payment Sunshine Act for drug and medical device manufacturers, allowing them to not report speaker fees or gifts to doctors that are intended for “continuing medical education” (CME) purposes, regardless of cost. Speaking fees can be a lucrative source of income for physicians, and gifts intended for medical education may include lavish items, such as admission to an expensive conference at a fancy resort, as long as the gifts are represented as intended for medical education. An additional provision would exempt expensive medical textbooks and journals from reporting by classifying them as “educational materials that directly benefit patients.” These valuable gifts should be reported under the Physician Payment Sunshine Act.

The Center for Medicare and Medicaid Services (CMS) has already provided flexibility to drug and medical device manufacturers in its clarification of the Final Rule.<sup>7</sup> Here, CMS has allowed for a reporting exemption where a manufacturer provides funding for a CME provider and does not select or pay the speaker directly. Arguably, this exemption already raises troubling opportunities for potential abuse. Certainly it is not necessary for the 21<sup>st</sup> Century Cures proposal to expand this exemption, which would further weaken the already accommodating reporting requirements under the Physician Payment Sunshine Act. Such additional exemptions for CME and educational materials would allow for the secret influence of industry on physician prescribing behavior and medical education, undermining the intent of the Physician Payment Sunshine Act to reveal, and therefore discourage, potential industry influence over physician behavior.

**5. 21<sup>st</sup> Century Cures would hasten the rise of resistant superbugs by incentivizing hospitals to use new antibiotics rather than conserving them for appropriate use (Section 2123).**

Section 2123 of the bill would give hospitals a reimbursement incentive to use new antibiotics. Such a provision would encourage the overuse of antibiotics by giving hospitals a financial bonus each time these drugs are prescribed, rather than encouraging hospitals to use older, effective antibiotics before using new ones that may not be medically necessary. This practice will only speed the rise of antibiotic resistant infections, as bacteria will increasingly become resistant to these new drugs as they are used more often. This provision, coupled with the financial conflict of interest in the selection of breakpoints for antibiotic resistance would further exacerbate antibiotic resistance and further limit the number of drugs available to treat patients. These changes would directly undermine recent efforts by the President and the Centers for Disease Control to slow the emergence of resistant bacteria through judicious

---

<sup>7</sup> Policy and Medicine. Physician Payments Sunshine Act: CMS Proposes Removing CME Exemption, Some Speaker Pay May Still Fall Under "Indirect Payment" Exclusion. July 7, 2014. <http://www.policymed.com/2014/07/physician-payments-sunshine-act-cms-proposes-removing-cme-exemption-some-speaker-pay-may-still-fall-under-indirect-payment.html>. Accessed May 19, 2015.

use of antibiotics in health care and other settings.<sup>8</sup> Rather than encourage this type of use, the bill should be re-drafted with incentives for good stewardship to encourage hospitals to preserve these drugs.

**6. 21<sup>st</sup> Century Cures 21st Century Cures will bar generic entry of medicines for a longer period and will deny patients access to affordable, life-saving medicines (Section 2151)**

Under Section 2151, the bill provides an additional 6 months exclusivity on top of a drug's existing exclusivity period if a new "orphan" indication is approved that involves treatment of a rare disease. This provision will extend the exclusivity period for all of the drug's indications, not just the orphan indication, increasing healthcare costs and limiting patient access to new drugs for a potentially broad range of diseases. The impact of monopoly pricing on patient access was illustrated by a recently published study, which found that in the year after marketing exclusivity was awarded to a common treatment for gout, a highly prevalent chronic condition in the United States, patients were less likely to receive a prescription, and that healthcare costs rose significantly for this population during this same time period.<sup>9</sup> These provisions would only further bar generic entry for an extended period, restricting patient access to affordable life-saving medicines they need.

In summary, the 21<sup>st</sup> Century Cures draft legislation as written would allow for the increased barriers for patients' access to care as well as for approval of unsafe and ineffective treatments. **We urge you to withhold support for this legislation, as it carries real and serious dangers for public health.**

Sincerely,

National Physicians Alliance  
Public Citizen  
American Medical Student Association  
Treatment Action Group  
Consumers Union  
AIDS United  
Knowledge Ecology International  
Young Professionals Chronic Disease Network

cc: Members, Committee on Health, Education, Labor and Pensions, United States Senate

---

<sup>8</sup> Burwell SM, Vilsack T, Carter A, Our plan to combat and prevent antibiotic-resistant bacteria. The White House Blog. March 27, 2015. <https://www.whitehouse.gov/blog/2015/03/27/our-plan-combat-and-prevent-antibiotic-resistant-bacteria>. Accessed May 19, 2015.

<sup>9</sup> Kesselheim, A. S., Franklin, J. M., Kim, S. C., Seeger, J. D., & Solomon, D. H. (2015). Reductions in Use of Colchicine after FDA Enforcement of Market Exclusivity in a Commercially Insured Population. *Journal of general internal medicine*, 1-6.

May 7, 2015

The Honorable Fred Upton  
Chairman, House Energy and Commerce Committee  
United States House of Representatives  
Washington, DC 20515

The Honorable Diana DeGette  
Member, House Energy and Commerce Committee  
United States House of Representatives  
Washington, DC 20515

Submitted electronically to: [ures@mail.house.gov](mailto:ures@mail.house.gov)

Re: 21st Century Cures Discussion Draft Legislation – Interoperability Section

Dear Chairman Upton and Congresswoman DeGette:

The College of Healthcare Information Management Executives (CHIME) appreciates the opportunity to submit comments concerning the Energy & Commerce Committee's discussion draft of the 21<sup>st</sup> Century Cures legislation.

CHIME has more than 1,400 members, composed of chief information officers (CIOs) and other top information technology executives at hospitals and clinics across the nation. CHIME members are responsible for the selection and implementation of clinical and business information technology (IT) systems that facilitate healthcare transformation.

Healthcare CIOs have experience implementing technology that must interoperate with dozens of disparate systems, ranging from diagnostic imaging and biomedical devices, to financial and remote access systems. The frustrations voiced by providers and policymakers regarding the systems deployed in over 80 percent of hospitals and 60 percent of physician offices are real. CHIME shares the vision of an e-enabled healthcare system as necessitated by many of the reforms proposed in 21<sup>st</sup> Century Cures Initiative. As the nation's premier organizations of senior health IT executives, we offer a focused set of recommendations on Sec.3001 -- Interoperability.

We must first acknowledge that the lack of a consistent patient identification strategy is the most significant challenge inhibiting the safe and secure electronic exchange of health information. As our healthcare system begins to realize the innately transformational capabilities of health IT, moving toward nationwide health information exchange, this essential core functionality – consistency in accurately identifying patients – must be addressed. As data exchange increases among providers, patient data matching errors and mismatches will become exponentially more dangerous and costly.

May 7, 2015

A consistent strategy does not mean a single technology or solution, but an approach that will facilitate the realization of the full benefits and cost savings of nationwide health information exchange, while protecting patient safety and privacy. **CHIME calls on Congress to remove the prohibition barring federal regulators from developing standards to improve positive patient identification.** With the removal of the outdated prohibition, we believe then that the nation can experience robust information exchange and interoperability.

We point to the concept of a longitudinal care record as an illustration of what is possible when the promise of health information technology becomes a reality. Unfortunately, the development of longitudinal healthcare records - reflecting the patient's experience across episodes of care, payers, geographic locations and stages of life - remains only an ideal at this time. We believe longitudinal care records should consist of provider, payer and patient-generated data, and be accessible to all members of a patient's care team, including the patient, in a single location. An information-rich record, supported by widely adopted standards, also should improve a patient's ability to manage consent privileges and diminish privacy concerns related to the digitization of personal health information (PHI).

We believe interoperability cannot be achieved across our fractured healthcare system in a matter of weeks, or even months. Interoperability will not be achieved through a command and control structure, where federal lawmakers or agency rule makers mandate "interoperability." Interoperability is not something that can be achieved by the electronic health record (EHR) vendors alone, it will require coordination across providers, vendors, exchanges and government entities and will only become more complex as new data sources enter the care continuum. The kind of interoperability that healthcare needs will only happen *over time* as local settings of care and technology implementations iterate towards conformance to usable, stable and extensible standards.

Further, as patient health data become digital, we must ensure stringent privacy and security standards are employed to protect health information as it becomes more fluid. **CHIME calls upon the Committee to ensure that security is included in the development of interoperability policy as to recognize the expanded nature of threats to patient data.** As the nation's healthcare systems become more interoperable, additional threats to data integrity will arise without proper safeguards and safe and secure transmissions of sensitive data will continue to be a challenge.

CHIME urges the Committee to continue the dialogue relative to expanding Medicare reimbursement for telemedicine services and other meaningful reforms that will expand opportunities to Medicare beneficiaries outside of a traditional care setting using innovative technologies. Medicare reimbursement policies have not kept pace with doctor-patient encounters occurring outside of a traditional care setting, and legal and regulatory barriers continue to impact the ability of providers to initiate or expand their telehealth services. **We respectfully request the Committee include language to improve existing telemedicine policies as a component of the 21<sup>st</sup> Century Cures Initiative, to represent the multitude of care encounters that may not look like the traditional doctor-patient interaction.**

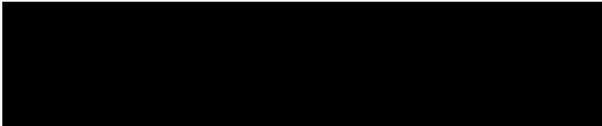
May 7, 2015

CHIME, once again, appreciates the opportunity to provide comments on this important, bipartisan effort. The attached document provides details of our recommendations for federal government actions to foster widespread healthcare interoperability. Without question, nationwide interoperability and robust health data exchange will be instrumental in executing the innovation espoused by the 21<sup>st</sup> Century Cures Initiative.

If there are questions about CHIME's recommendations or more information is needed, please contact Leslie Krigstein, Interim Vice President of Public Policy, at [lkrigstein@chimecentral.org](mailto:lkrigstein@chimecentral.org) or (202)507-6158.

We look forward to a continuing dialogue with your offices on this and related matters.

Sincerely,



Russell P. Branzell, CHCIO, LCHIME  
President and CEO  
CHIME



Charles E. Christian, CHCIO, LCHIME, FCHIME  
FHIMSS  
Chair, CHIME Board of Trustees  
Vice President & Chief Information Officer  
St. Francis Hospital

May 7, 2015

Attachment

Below, CHIME offers recommendations concerning healthcare interoperability.

### **Interoperability**

***There is a fundamental lack of interoperability across electronic health record systems throughout the country. Patient data does not flow seamlessly in usable forms across care settings and vendor types.***

CHIME members wish to highlight the enormously difficult socio-technical challenge of making technology systems interoperable; not only does it require harmonizing complex technology systems, but complex systems of policies, processes and people must also align to achieve interoperability. We stress the need for simplicity as an overarching goal of future drafts, but we also caution against any notion that interoperability will come easily or cheaply.

### **Background:**

While the HITECH Act enabled the rapid, wide-spread adoption of electronic health records (EHRs) across the nation, it has not enabled seamless data sharing across care settings. Consistent with other technology sectors, health IT interoperability requires consistent use of consensus standards and an infrastructure to exchange data. Providers can capture data into an EHR, but they struggle to share it with other providers, especially those using different technology systems.

### **Recommendations:**

In order to achieve easy access to patient data across care settings, or interoperability, we must fundamentally reevaluate the policies and programs currently at the disposal of the federal government. Consistent with other technology sectors, health IT interoperability requires consistent use of consensus standards and an infrastructure to exchange data. CHIME calls on the federal government to bolster their involvement in health IT programming through:

- The development and wide-spread adoption of data standards; and
- The definition of robust testing requirements for certified EHR technology and other components of health IT referenced in federal policies.

### *Standards*

While we have made impressive progress as a nation on basic EHR adoption, we must refocus our efforts on facilitating interoperability, based on clear, defined and enforceable standards. Industry stakeholders have repeatedly called on the government and private industry to develop a way to test, refine and update health IT standards over time. We commend the Office of the National Coordinator on the release of the proposed 2015 Interoperability Standards Advisory, to help identify key health IT standards. The Standards Advisory signifies a strategic prioritization of federal resources toward the adoption and continued evaluation of common standards to be universally employed by healthcare providers nationwide.

May 7, 2015

CHIME calls on the federal government to drive the use of standards related to the following eight priority areas:

1. Patient identifiers
2. Standards for resource locators (e.g. provider directories)
3. Standard terminologies
4. Detailed clinical models
5. Standard clinical data query language based on the models and terminology
6. Standards for security (standard roles and standards for naming types of protected data)
7. Standard Application Program Interfaces (APIs)
8. Standards for expressing clinical decision support algorithms

Industry and government stakeholders need to collaborate on a process that will allow for rapid standards development and refinement, while not impulsively requiring the use of immature standards. The government should support private sector-run test-beds for users and developers of standards as an intermediate step between draft standard for trial use and consensus standard. Further, the government should fund pilot programs to enable providers and software developers to test and enhance implementation guides. The establishment of clear maturity criteria will be necessary in order to assist in the determination of a standard/implementation guide readiness to be endorsed.

#### *ONC Certification Program*

ONC's Certification Program should be valued as a lynchpin for interoperability and acknowledge the existing voluntary certification program as the only existing means to enforce technology developers' compliance to federal law.

CHIME maintains our request of ONC to reconsider the role and composition of its certification program to address patient safety risks and interoperability. ONC's certification program was built out of regulatory necessity to accommodate misguided timelines driven by Meaningful Use, not in acknowledgement of how technology is developed, tested, implemented and optimized. This has led to a market dynamic that incentivizes data silos, vendor lock-in and rewards developers who are "first-to-certify" rather than a market characterized by usable, safe and mature health IT products.

In so far as certification appears to be one of the government's best tools to promise adherence to technical standards and specifications, we believe the form and function of certification needs to adapt. Therefore, Congress should enable ONC to enhance its enforcement tools meant to ensure health IT functionalities are effective and they adhere to interoperability standards.

Further, we recommend that ONC re-tool its certification program to have a specific focus on beta-testing, post-certified performance and live-setting standards adherence, maintaining many of the proposals set forth in the proposed *2015 Edition Health Information Technology Certification Criteria*. We believe the results from these more robust tests should be made publicly available, to ensure providers know which products are performing well and adhering to standards in the real-world. By reorienting and leveraging its certification program, ONC could help the private/non-profit sector

May 7, 2015

establish a learning health system to support a 21<sup>st</sup> century healthcare system, characterized by continuous improvement and consistent accountability.

# Legislative Guidance Toward 21<sup>st</sup> Century Clinical Regulatory Science: A Moonshot for Patient-Centric Drug Development for Chronic Disorders

## Rationale

The rationale for offering this input to the 21<sup>st</sup> Century Cures Act is as follows:

1. **Clinical Regulatory Science:** Offering specific ways to enhance “clinical regulatory science” per se would strengthen the current draft.<sup>1</sup> Although improvements in clinical regulatory science are crucial to accelerate 21<sup>st</sup> Century Cures, there is no mention of “regulatory science” in the current draft.<sup>2</sup>
2. **Clinical Trial Design:** Offering guidance about specific ways to improve clinical trial design<sup>3</sup> would strengthen this draft. Many advances in life sciences and technology must be demonstrated to be safe and effective in clinical trials before they can accelerate cures and improve the human condition. Enhanced clinical trial designs would accelerate 21<sup>st</sup> Century prevention and cures.
3. **Chronic Disorders:** This draft addresses “APPROVAL OF CERTAIN ANTIBACTERIAL AND ANTIFUNGAL DRUGS FOR USE IN A LIMITED POPULATION OF PATIENTS” (page 105). However, there is *no* mention of “chronic.” Chronic disorders are the leading causes of death and disability in the United States.<sup>4</sup>
4. **High Expenditures for Chronic Disorders:** “As a nation, we spend 86% of our health care dollars on the treatment of chronic diseases.”<sup>5</sup> These annual expenses are about \$3 trillion in the United States alone.<sup>6</sup> Chronic disorders also merit specific attention in the 21<sup>st</sup> Century Cures Act.
5. **Neurological and Mental Disorders:** “In America, neurological illnesses and mental disorders cost more than \$760 billion a year.”<sup>7</sup> The current draft does call for a “National Neurological Diseases Surveillance System” on page 37. Strengthen the current draft to include guidance about prevention, management, and cures.
6. **Central Nervous System (CNS) Drug Development:** “CNS drugs take longer to develop and have lower success rates than other drugs.”<sup>8</sup> Strengthen the current draft with legislative guidance about how to improve regulatory science and clinical trial design for CNS drugs. The same advice would apply to many other chronic disorders.

---

<sup>1</sup> <http://energycommerce.house.gov/sites/republicans.energycommerce.house.gov/files/114/20150429DiscussionDraft.pdf>

<sup>2</sup> This draft does include some 17 mentions of “regulatory” as in “regulatory process improvements,” “regulatory efficiency,” “regulatory entities,” “regulatory decisionmaking,” “regulatory review,” “regulatory authorities,” “regulatory duplication” and “regulatory and legal liability.”

<sup>3</sup> Some 34 mentions of “clinical trial” include “clinical trial data system,” “streamlining clinical trials,” “clinical trial network,” “clinical trial registry,” “qualified clinical trials,” and “shorter or smaller clinical trials.” There is one mention of “adaptive trial designs and Bayesian methods in clinical trials.” The following quote points in a helpful direction that can be further developed: “In this section, the term ‘evidence from clinical experience’ means data regarding the usage, or potential benefits or risks, of a drug derived from sources other than randomized clinical trials, including from observational trials, registries, and therapeutic use” (page 90).

<sup>4</sup> <http://www.cdc.gov/chronicdisease/overview/>

<sup>5</sup> <http://www.cdc.gov/chronicdisease/>

<sup>6</sup> <http://www.cms.gov/Research-Statistics-Data-and-Systems/Statistics-Trends-and-Reports/NationalHealthExpendData/downloads/highlights.pdf>

<sup>7</sup> <http://www.brainfacts.org/policymakers/global-burden-of-neurological-and-mental-disorders/>

<sup>8</sup> [http://csdd.tufts.edu/news/complete\\_story/pr\\_ir\\_nov\\_dec\\_ir](http://csdd.tufts.edu/news/complete_story/pr_ir_nov_dec_ir)

This input is intended to strengthen the 21<sup>st</sup> Century Cures Act by suggesting specific legislative guidance to improve clinical regulatory science for patient-centric drug development for chronic disorders. The FDA has expressed interest: “Moving Regulatory Science into the 21<sup>st</sup> Century.”<sup>9</sup> This document offers enhanced guidance.

## Moonshots

“Here's Google's definition of a moonshot:

A project or proposal that:

1. Addresses a huge problem
2. Proposes a radical solution
3. Uses breakthrough technology”<sup>10</sup>

According to Larry page of Google, moonshots need to be “10 times better than the competition.”<sup>11</sup>

NIH Director Francis Collins has used “moonshot” terminology repeatedly.<sup>12</sup>

Moonshot terminology is appropriate and apt for 21<sup>st</sup> Century Cures.

## Legislative Guidance Toward a Clinical Regulatory Science Moonshot

Enhance clinical regulatory science for patient-centric drug development for chronic disorders in accord with this guidance:

1. **Data Collection Infrastructure:** Collect, integrate, and secure more *multivariate time series* (data with changing values over time for two or more quantities) about *individual* living systems and their environments. Treatments are environmental exposures delivered with therapeutic intent.
  - a. Compared to data collected at a particular time such as a clinic visit, multivariate time series can include orders of magnitude more information for *computing* better diagnoses of many chronic functional disorders.
  - b. Compared to baselines, endpoints, and change scores often are used in clinical trials, multivariate time series can include orders of magnitude more information for *computing* better measures of apparent treatment effect (treatment response phenotypes).
  - c. Clinicians often evaluate treatment effects for individual patients with experience gained over time as through response to drug challenge, de-challenge, and re-challenge – often with multiple doses. Record such experience as multivariate time series and process the data by computation in contrast to subjective impressions.
  - d. Vast quantities of health-related multivariate time series already exist as from the Human Connectome Project for brains.<sup>13</sup> Monitoring devices used in hospitals also collect time series.

---

<sup>9</sup> [http://www.fda.gov/ScienceResearch/SpecialTopics/RegulatoryScience/default.htm?utm\\_campaign=Goo](http://www.fda.gov/ScienceResearch/SpecialTopics/RegulatoryScience/default.htm?utm_campaign=Goo)

<sup>10</sup> <http://whatis.techtarget.com/definition/moonshot>

<sup>11</sup> <http://www.wired.com/2013/01/ff-ga-larry-page/> This link also includes this about Page: “As an undergrad at the University of Michigan, he found inspiration in a student leadership-training program called LeaderShape, which preached ‘a healthy disregard for the impossible.’” 21<sup>st</sup> Century Cures Act warrants a moonshot with leadership that includes Michigan.

<sup>12</sup> <http://directorsblog.nih.gov/2014/09/30/brain-launching-americas-next-moonshot/> and [https://www.youtube.com/watch?v=N6g\\_TpQsL0I](https://www.youtube.com/watch?v=N6g_TpQsL0I) are examples.

<sup>13</sup> <http://www.humanconnectome.org/data/> Functional brain imaging multivariate time series can include information about brain activity in hundreds of thousands of small brain regions collected about every two seconds for prolonged periods of time.

- e. Wearable, implantable, and other mobile health devices are rapidly expanding data collection capabilities.
  - f. Engage with private sector initiatives involving health data.<sup>14</sup>
2. **Compute Patient Diagnoses and Diagnostic Phenotypes:** Use these data to compute better diagnoses of many chronic disorders.
    - a. Drug developers and regulators need better diagnoses to target drug development and approval.
    - b. Clinicians need better diagnoses as indications for treatment.
    - c. Better diagnoses and diagnostic phenotypes are more:
      - i. Objective
      - ii. Reliable
      - iii. Specific
      - iv. Mechanistic
      - v. Actionable
  3. **Expand use of randomization in clinical trials.**
    - a. Randomization is the best way to eliminate the effects of unknown confounding factors while evaluating treatment effects.
    - b. The one prevailing use of randomization in current clinical regulatory science is to randomize patients to different treatment groups.
    - c. In addition and often as an alternative, make more use of *randomizing doses to different periods of time for each individual* – within-patient randomization of doses.
    - d. **Repeated within-patient randomization of doses:**
      - i. Helps assure that treatment evaluation results are **patient-centric** and valid for each individual.
      - ii. Often should include placebo as zero dose.
      - iii. Can help optimize dosing for each individual.
      - iv. Often should be accompanied with methods to help account for temporal phenomena such as disease progression, spontaneous recovery, delay of treatment response, persistence of treatment response, etc.
  4. **Explicitly distinguish treatment effects as scientific facts from how treatment effects are valued.**
    - a. Different people value the same facts differently.
    - b. Enhance methods for eliciting patient preferences for various health outcomes.
    - c. Distinguishing facts from values:
      - i. Facilitates personalized medicine by enabling individual patients in consultation with their clinicians to apply their own preferences for each of various health outcomes.
      - ii. Recognizes that science still has much to learn about how treatment effects with respect to time-varying response variables and biomarkers affect risk of severe health events and death.
  5. **Adopt a common metric** to evaluate beneficial and harmful treatment effects with respect to multitudes of dependent or response variables for each patient. This innovation has potential to:
    - a. Dramatically reduce clinical drug safety problems.
    - b. Profile benefit and harm with respect to multitudes of response variables starting at the level of each individual.

---

<sup>14</sup> Here is a prime example of a recent and relevant private sector initiative: <https://www-03.ibm.com/press/us/en/pressrelease/46580.wss>.

- i. Use such profiles to identify indications and contra-indications of treatment.
  - c. Assure that both clinical safety and clinical efficacy evaluations are conducted with the same new high standards of scientific rigor.
  - d. Reduce the dimensionality of treatment evaluation problems from many to one or just a few.
    - i. One hypothesis can test *overall benefit and harm* with respect to a multitude of response variables or biomarkers differentially weighted in accord with clinical significance and patient preferences.
  - e. Scientifically integrate and balance clinical safety evaluations with clinical efficacy evaluations.
- 6. **Use information from more repeated measurements** to:
  - a. Separate treatment effect signals from noise that results from measurement error and uncontrolled variables.
  - b. Increase the reliability of measures of treatment effect for each individual patient.
  - c. Reduce the number of subjects required to achieve statistical significance in many group clinical trials.
  - d. Reduce the cost of many clinical trials.
- 7. **Validate three new Randomized Controlled Trial (RCT) designs in practice.**
  - a. New single-patient (enhanced capability N of 1) RCT design
    - i. Repeated within-patient randomization of doses
    - ii. The ultimate in personalization and precision
    - iii. Results apply to that individual, the N of 1
    - iv. New gold standard for much clinical practice
    - v. Empower individuals in the quantified self community
  - b. Single-group, multiple N of 1 RCT design
    - i. A coordinated set of advanced design N of 1 RCTs
    - ii. Analyze group results statistically to generalize from a sample of individuals to the population sampled
    - iii. For drug development and regulation
  - c. Multiple-group, multiple N of 1 RCT design
    - i. **Double randomization**
      - Patients to groups defined by *type* of treatment
      - Within-patient randomization of *doses*
    - ii. For comprehensive and integrated comparative safety and efficacy research
- 8. **Foster recruitment of patients** into clinical trials by doing more to protect patient safety and making results applicable to improve continued care of each individual clinical trial participant.
- 9. **Elucidate mechanisms of treatment effect**
  - a. Treatments often work by up- or down-regulating proteins, lipids, carbohydrates, metabolites, brain activity, electrophysiological measures, mood, behavior, mental performance, physical performance, etc. at biological, psychological, and social levels of investigation.
  - b. Adopt methods to measure normal regulation, dysregulation, up-regulation, and down-regulation starting at the level of each individual.
  - c. Knowledge of treatment mechanisms connects with the new diagnoses introduced by Point 2 above.

10. **Identify predictors:** Use the improved diagnostic and treatment response phenotypes to help identify genetic and other predictors of differential disease susceptibility, treatment response, dose requirements, etc.
  - a. Do more to capitalize on genomics.
11. **Coordinate 21<sup>st</sup> Century clinical regulatory science for patient-centric drug development for chronic disorders with the following to enhance the following:**
  - a. FDA's Critical Path Initiative<sup>15</sup>
  - b. Clinical Trial Transformation Initiative<sup>16</sup>
  - c. Research Domain Criteria (RDoC) of the National Institute of Mental Health<sup>17</sup>
  - d. Human Connectome Project<sup>18</sup>
  - e. BRAIN Initiative<sup>19</sup>
  - f. Patient-Centered Outcomes Research Institute methodology standards<sup>20</sup>
  - g. Precision Medicine Initiative.<sup>21</sup>

## Concluding Comments

These concluding comments are about this proposed legislative guidance. These comments are not intended to be part of this guidance.

All this legislative guidance is technically *possible* now. Advances in data collection, data processing, and communication appear to be making this guidance *feasible* now.

This guidance is informed and made possible by what is proffered as being a breakthrough in measurement science.

- The International System of Units is comprised of “a coherent system of units of measurement built on seven base units. It defines twenty-two named units, and includes many more unnamed coherent derived units.”<sup>22</sup>
- The proffered breakthrough in measurement science is presented as a new category of derived or computed measurements.
  - These derived measures are computed with an algorithm.
  - These measures are computed from data with changing values over time for two or more quantities – multivariate time series – about *one individual* Complex Adaptive System (CAS).
  - These derived measures are IoT scores that quantify Interactions over Time (IoT).
  - IoT scores are in standard deviation units and are internally standardized.
  - Each IoT score is one score from a distribution of potential scores.
  - Each distribution of potential scores has a mean of 0 and a standard deviation of 1 unless 0 is the only potential score.

---

<sup>15</sup> <http://www.fda.gov/ScienceResearch/SpecialTopics/CriticalPathInitiative/>

<sup>16</sup> <http://www.ctti-clinicaltrials.org/>

<sup>17</sup> <http://www.nimh.nih.gov/research-priorities/rdoc/index.shtml>

<sup>18</sup> <http://www.humanconnectome.org/>

<sup>19</sup> <http://www.humanconnectome.org/>

<sup>20</sup> <http://www.pcori.org/assets/2013/11/PCORI-Methodology-Report-Appendix-A.pdf>

<sup>21</sup> <http://www.nih.gov/precisionmedicine/>

<sup>22</sup> [http://en.wikipedia.org/w/index.php?title=International\\_System\\_of\\_Units&oldid=660443495](http://en.wikipedia.org/w/index.php?title=International_System_of_Units&oldid=660443495) Here is more information about derived units: [http://en.wikipedia.org/w/index.php?title=SI\\_derived\\_unit&oldid=650747120](http://en.wikipedia.org/w/index.php?title=SI_derived_unit&oldid=650747120).

- IoT scores have meaningful values of 0 that indicate no evidence for an interaction-over-time
- IoT scores appear to be well suited for mathematical modeling.
- IoT scores from two or more individuals are well suited for statistical analyses.
- IoT scores quantify how individual CAS work over time. Work has three operationally defined components.
  - Internal function as for patient diagnosis
  - Response – how individual CAS respond to their environments including treatments
  - Agency – how individual CAS affect their environments.
- This legislative guidance is about individual CAS that are patients or other people.
- Benefit and harm scores are a variant of IoT scores for evaluative investigations such as RCTs.

This legislative guidance is presented at a moderate level of detail. More information is readily available.

- Here is a 1992 peer-reviewed publication that introduces core components: <http://dataspeaks.com/resources/APA-JCCP-1992-Vol60-No2-P225-239.pdf>.
- Here are two issued software patents:
  - 6,317,700 – Computational Method and System to Perform Empirical Induction
  - 6,516,288 – Method and System to Construct Action Coordination Profiles
- This includes two demonstrations with some detail about methods: [http://dataspeaks.com/resources/bagne\\_handout.pdf](http://dataspeaks.com/resources/bagne_handout.pdf).
- [http://www.nist.gov/tip/wp/pswp/upload/136\\_from\\_genomics\\_to\\_personalized\\_medicine\\_with\\_advances2.pdf](http://www.nist.gov/tip/wp/pswp/upload/136_from_genomics_to_personalized_medicine_with_advances2.pdf)
- At least dozens of additional papers and presentations.

This legislative guidance appears to be a roadmap to achieving *The Science of Individuality* as called for on page 228 of Dr. Eric Topol's book, *The Creative Destruction of Medicine*.<sup>23</sup>

Success in advancing clinical regulatory science in accord with these legislative guidelines would be a fitting legacy to the 21<sup>st</sup> Century Cures Act.

**Contact:**

Curtis A. Bagne, Ph.D.

████████████████████  
 ████████████████████  
 ██  
 ████████████████████

---

<sup>23</sup> <http://creativestructionofmedicine.com/>

May 7, 2015

The Honorable Fred Upton  
Chairman  
House Energy and Commerce Committee  
2125 Rayburn House Office Building  
Washington, DC 20515

The Honorable Frank Pallone  
Ranking Member  
House Energy and Commerce Committee  
2322A Rayburn House Office Building  
Washington, DC 20515

Dear Chairman Upton and Ranking Member Pallone:

CVS Health appreciates the opportunity to comment on the committee's 21<sup>st</sup> Century Cures draft proposal. We commend you for this important effort to improve the delivery of health care to patients. CVS Health supports a number of the provisions included in the draft proposal. We have also included a few recommendations to help ensure patients have access to affordable medicines and services through the growing use of medical technology. Our comments center around **CMS data-sharing encouraging the use of telehealth in Medicare, creating the Medicare pharmaceutical and technology ombudsman, preventing fraud and abuse in Medicare Prescription Drug Programs (PDPs), and expanded drug exclusivity.**

We look forward to working with you, as you to advance legislation that leads to 21<sup>st</sup> Century Cures. As one of the country's largest pharmacy benefits manager, CVS Health touches more than 65 million Americans through 2,200 clients who provide health coverage. There are more than 26,000 pharmacists and more than 7,700 CVS/pharmacy retail stores within our company. CVS Health is also a leading specialty and mail service pharmacy provider. Our 900 MinuteClinic locations employ more than 2,500 combined nurse practitioners and physician assistants, who provide convenient access to routine health care services. CVS Health is also a leading provider of Medicare prescription drugs and health care services. We offer our comments on the draft legislation below.

#### **Advancing Telehealth Opportunities in Medicare (Section 3021)**

We are pleased that the committee has a placeholder for advancing telehealth in Medicare. Medicare beneficiaries should have increased access to telehealth services as commercial patients do, and CVS Health has been working with the Alliance for Connected Care on policy solutions with this goal in mind. We look forward to working with you on this important issue and reviewing and providing comments when it is available.

#### **Medicare Pharmaceutical and Technology Ombudsman (Section 3101)**

We appreciate that the committee would like to provide an additional resource for those negatively impacted by adverse policy decisions at CMS. However, this provision creates an unlevel playing field in favor of pharmaceutical and device manufacturers within CMS where no such advocate exists for other federal partners, including payers, at FDA or CMS.

#### **Establishing a Part D Safe Pharmacy Program (Section 3151)**



CVS Health supports this section designed to address the incredibly damaging and costly scourge of drug abuse in the Medicare beneficiary population. This provision would make it more difficult for Medicare beneficiaries to use the Medicare Part D benefit to abuse drugs by utilizing different pharmacies, thus inhibiting efforts to identify and stem abuse. We encourage the committee to add language requesting that the Secretary accept stakeholder input when implementing key provisions in this section including the establishment of drug safety criteria.

The previous draft bill included an e-prescribing provision and a provision improving activities of Medicare Drug Integrity Contractors (MEDICs). While these provisions have been removed from this draft, we hope to work with the committee on these important issues in the future.

#### **Additional Exclusivity (Subtitle I Placeholder)**

We understand that the committee wishes to encourage and expedite life-saving cures for those patients most in need. We support these efforts when they are balanced appropriately with cost savings initiatives. CVS Health is concerned about expanding exclusivity and the impact that would have on the affordability of medications. We look forward to offering further comments when further language is available related to the section titled "Repurposing drugs for serious and life-threatening diseases and conditions." We want to work with the committee to identify additional tools that can help patients' access new therapies at an affordable price and help them remain adherent to their medications.

#### **Conclusion**

Given CVS Health's varied and extensive experience operating in the current health care system, we sincerely applaud the committee's work on 21<sup>st</sup> Century Cures and look forward to collaborating with the committee to further enhance the health care system.

Thank you for seeking input from stakeholders such as CVS Health on your draft legislative proposal. If you have questions on any of the policy recommendations noted above, please contact me at 202-760-0156 or at [ann.walker-jenkins@cvshealth.com](mailto:ann.walker-jenkins@cvshealth.com).

Sincerely,

Ann Walker-Jenkins  
Director, Federal Government Affairs

May 8, 2015

The Honorable Fred Upton  
Chairman  
Committee on Energy & Commerce  
2125 Rayburn House Office Building  
Washington, DC 20515

The Honorable Frank Pallone  
Ranking Member  
Committee on Energy & Commerce  
2322A Rayburn House Office Building  
Washington, DC 20515

Dear Chairman Upton and Ranking Member Pallone:

On behalf of the Coalition for Pediatric Medical Research, an alliance of our nation's leading children's hospitals and pediatric research institutions; and FightSMA, an organization of families and researchers leading the effort to find a treatment for spinal muscular atrophy, we are writing to thank you for your continued support of pediatric medical research and for including a provision to drive implementation of the National Pediatric Research Network Act (Title II of Public Law 113-55) within the latest 21<sup>st</sup> Century Cures discussion draft. We greatly appreciate your actions to date, and we urge you to retain this provision in subsequent drafts of the legislation as it moves through your committee, and to consider any opportunities to include an explicit authorization of appropriations for the provision.

As you know, the NPRNA enjoyed tremendous bipartisan support as it moved through Congress, and was enacted into law in late 2013. The NPRNA builds upon more than a decade of pediatric research and stakeholder input to develop a meaningful and cost-effective mechanism to strengthen and enhance the National Institutes of Health's commitment to pediatric research. Modeled upon the Comprehensive Cancer Centers and other successful networked research initiatives, the NPRNA at its core seeks to encourage researcher collaboration and coordination by supporting shared core research infrastructure necessary to discharge a robust 21<sup>st</sup> century pediatric research agenda.

Each center or consortia would be comprised of multiple institutions, reflecting the reality that most of pediatric health research involves investigation into rare diseases and disorders, thus necessitating such a collaborative approach to research, particularly clinical studies. In addition to supporting shared core research technologies, NIH funding would also support vital training and development slots for early-career clinician/researchers seeking to focus in pediatrics.

Thanks to your leadership, Congress enacted the NPRNA into law 18 months ago. The time is now to implement this law, and Sec. 1081 of the latest Cures draft will help us achieve this aim.

Thank you, again, for your leadership and support. If you have any questions or would like additional support from the Coalition or FightSMA, please contact Nick Manetto at 202.312.7499 or [nicholas.manetto@faegrebd.com](mailto:nicholas.manetto@faegrebd.com), or Steve Eichenauer at 202-783-2596 or [seichenauer@psw-inc.com](mailto:seichenauer@psw-inc.com).

Sincerely,

Nick Manetto  
For the Coalition for Pediatric Medical Research

Steve Eichenauer  
For FightSMA



House Committee on Energy and Commerce  
2125 Rayburn House Office Building  
Washington, D.C. 20515

The Honorable Fred Upton  
Chairman

The Honorable Diana DeGette  
Committee Member  
Co-chair 21st Century Cures Initiative

Via email: [cures@mail.house.gov](mailto:cures@mail.house.gov)

May 13, 2015

Dear Representative Upton and Representative DeGette:

As the nation's leading private lupus research organizations founded and supported by patients and their families to fund novel research, the Lupus Research Institute (LRI) and the Alliance for Lupus Research (ALR) applaud you on your yearlong effort to explore how to accelerate the discovery, development, and delivery cycle of life-changing medical treatments and the recent release of the second draft of the 21st Century Cures Act.

On behalf of the LRI, the ALR and our lupus patients and their families nationwide, we would like to once again offer our deep appreciation for the attention you are devoting to the critical importance of engaging the patient perspective in the regulatory process, to modernizing clinical trials, and to enhancing the ultimate reach of biomedical research.

We have previously expressed priority areas that we feel could help to transform medical care for the potentially 1.5 million patients with lupus to you and other members of the Energy and Commerce Committee. Lupus is difficult to diagnose and usually requires extensive treatment, including heavy drug therapies and specialty medical care, so the possible benefits as a result of this legislation for the patients in our community is considerable. We are pleased to provide feedback on the second 21st Century Cures draft provisions that fall within priority areas for LRI and ALR.

### **Strengthen NIH biomedical research programs**

Since the founding of LRI and ALR in 2000, the driving missions of both organizations have been to invest in pioneering research searching for better, safer treatments and for the cause and

cure by raising funds in the private sector. Our research programs have been highly successful, and our substantial investments have provided academic-based investigators with the ability to initiate studies, and publish groundbreaking breakthroughs. However, the private sector cannot accomplish all the work that remains to be done alone. That is why we would like to thank you for the inclusion of **Sections 1001 and 1002** which would authorize larger amounts for NIH through 2018, and establish a NIH Innovation Fund which would provide another \$2 billion per year over the next five years to priority research. Further, we are pleased that NIH will continue to evaluate and identify valuable research opportunities through a research strategic plan (**Section 1021**).

A great deal of time and energy is spent on the granting process by researchers, and the LRI and ALR feel that their time is best served discovering and developing the next innovative treatments and cures for diseases like lupus. LRI and ALR support **Section 1023** which would create a biomedical research working group that would provide recommendations on how to reduce administrative burdens of researchers funded by NIH. We also support **Section 1024** which would exempt certain NIH research activities from the Paperwork Reduction Act.

From the very beginning, innovation has been our philosophy, and we focus on cutting-edge, novel research with the intention to deliver new treatments, prevention and a cure, and to stop disease progression and damage in the very near term. LRI and ALR also support **Section 1028** which would direct the directors of each institute at NIH to establish programs, and set aside funding for such programs, that would conduct or support high-risk research that addresses contemporary challenges in the biomedical field. Lupus patients only recently celebrated the first FDA approval of a new drug to treat their disease, and many more are needed. It is this type of research that we believe will change the treatment landscape for lupus patients.

### **Support measures to enhance the numbers and conduct of clinical trials**

Because lupus has only had one new drug approved for treatment of this debilitating disease in over fifty years, the streamlining of clinical trials is essential to ensuring that innovative breakthroughs reach patients quickly. Further, FDA needs flexibility to innovate and deal with complex diseases like lupus. It is imperative that we get safe and effective treatment options to lupus patients as soon as possible. For these reasons, LRI and ALR support:

- **Section 2022** which would facilitate early interactions and agreement between sponsors and FDA on designing studies to generate evidence for purposes of accelerated approval;
- **Section 2061** which would require FDA to hold a public meeting and issue guidance documents that would assist sponsors in incorporating adaptive designs and Bayesian statistical modeling into their clinical protocols and new drug applications;
- **Section 2062** which would require FDA to establish a program to evaluate the potential use of evidence from clinical experience to help support the approval of a new indication for a drug and to help support or satisfy post-approval study requirements;
- **Section 2063** which would require FDA to establish a streamlined data review program that would make use of submitted clinical data summaries to support the approval or

licensure of specified new indications of drugs and biologics if certain qualifying criteria are met.

We also commend the inclusion of **Section 1141** which would create the Council for 21st Century Cures. A holistic approach for evaluating how to accelerate the discovery, development, and delivery of innovative cures, treatments, and preventive measures for patients is greatly needed. We thank the Committee for ensuring that three patients will be included on this Council.

However, LRI and ALR have several concerns. The draft bill asks FDA to take on a considerable amount of new work. We hope that the Committee is considering whether FDA has the additional resources necessary to successfully implement these new programs. Secondly, we hope that the safety of patients is one of the highest priorities when finalizing language on this bill. We are very concerned that **Section 2241** would substantially weaken human subject protections. This section would exempt clinical trials that are subject to the comprehensive human subject regulations under the Federal Food Drug and Cosmetic Act (FFDCA) from requirements under the Common Rule.

NIH also needs the capacity to enhance its clinical trials and biomedical research programs generally. LRI and ALR endorse **Sections 1026 and 1027** which would enhance the abilities of the National Center for Advancing Translational Science (NCATS) at NIH to develop life-altering treatments and cures. Additionally, access to data is an important component to ensuring that the best research and potential therapies can be fully developed. We endorse **Section 1101** which would give the NIH Director the authority to require as a condition of the award or grant that the researchers share data generated through the research. We also support **Section 1121** which would instruct the FDA and NIH to enter into a collaborative Clinical Trial Data System Agreement with other entities to implement a system to make de-identified clinical trial data from qualified clinical trials available for purposes of conducting further research. LRI and ALR also believe that the partnership and studies that would result from **Section 1123** would greatly support the innovative research being done on lupus.

LRI's and ALR's investments in the research community have led to the identification of over 20 new lupus biomarkers for future investigation. These biomarkers will be instrumental in the ability of researchers and physicians to diagnose and monitor lupus. Further, these biomarkers will allow physicians to intervene earlier by giving them the ability to predict flares in lupus patients before a patient's organs are severely compromised and extensive treatments are needed. FDA's qualification of lupus biomarkers is a necessary and important step in advancing clinical research on medical treatments. We generally support **Section 2021** which would establish a codified process for qualification of biomarkers and other drug development tools. We hope that the Committee continues to work with FDA to ensure that FDA's work to develop biomarkers continues to expand to provide researchers with these vital tools.

**Integrate greater -- and meaningful -- patient participation at all stages of the research and drug approval processes, including representative levels of women and minorities**

We commend the Committee on the inclusion of a patient engagement provision (**Section 2001**) which directs FDA to use patient experience to create a structured risk-benefit assessment framework in the new drug approval process. This work will build upon the work already being

done by FDA on this important issue. No one knows better than the patient what medication side effects are tolerable and what tradeoffs they are willing to accept in a new treatment. Any risk-benefit analysis must include the patient perspective. Providing for a mechanism for all diseases to have input into the regulatory process and not just those selected through the FDA's patient focused drug development initiative would be a major advancement. We are also pleased to see that **Section 2021** would establish a process to develop and qualify patient-reported outcomes as tools to support the approval or licensure of a drug or biologic.

The recruitment of patients in clinical trial continues to be a limiting factor in how fast a medical treatment can be approved. LRI and ALR commend the attempts made to enhance the ability of patients and their caretakers to find the most applicable clinical trials through the use of Clinicaltrials.gov (**Section 1102**). We also recognize that NIH may have concerns that the standardization of certain criteria may limit the usefulness of some information in the clinical trial registry. LRI and ALR encourage the Committee to continue working with NIH to find the appropriate balance between the ease of use of Clinicaltrials.gov by patients and the burden to researchers.

There are many patients not enrolled in a clinical trial who could benefit from a medical treatment. However, patients and the physicians often have great difficulty obtaining the information needed to gain access to these investigational therapies. We support the inclusion of **Sections 2082** which would provide transparency on the requirements of certain drug and contact information regarding their expanded access programs.

**Provide incentives to foster more clinical research, networks and clinical trials; accelerate drug discovery; and allow innovation to develop safe and effective drugs**

We are concerned that legislative language has not been shared with the public on sections that could potentially affect lupus drug development. In particular, LRI and ALR are eager to evaluate the language on (**SUBTITLE I**) repurposing drugs for serious and life-threatening diseases and conditions. We hope that the Committee continues to engage in an open and transparent process to develop this section before finalizing the 21st Century Cures Act.

Prioritizing research on a national level is also an important way to incentivize and foster more clinical research, networks, and clinical trials to accelerate drug discovery. We are looking forward to the many advances that will be made in medicine that stem from the work that will be done as part of the Precision Medicine Initiative. We commend the involvement of FDA in this important Initiative and hope that **Section 2041** will help FDA prepare for the evaluation of treatments for subpopulations of patients.

Thank you for the tremendous amount of effort that went into the development of this 21st Century Cures discussion document. We look forward to continuing our work with you as you move toward developing and implementing legislation to enhance the lives of lupus patients.

Sincerely,



Margaret G. Dowd  
President and CEO  
Lupus Research Institute



Kenneth M. Farber  
President  
Alliance for Lupus Research

**Steven Prince**  
Chairman  
Board of Directors

**John Leonard, MD**  
Chair  
Scientific Advisory Board

**Meghan Gutierrez**  
Chief Executive Officer

**National Headquarters**  
115 Broadway  
Suite 1301  
New York, NY 10006  
212-349-2910  
212-349-2886 (Fax)  
LRF@lymphoma.org  
lymphoma.org

**LRF Helpline**  
800-500-9976  
Helpline@lymphoma.org

**Chapters and Offices**  
Arizona  
California  
District of Columbia  
Florida  
Georgia  
Illinois  
Massachusetts  
Michigan  
Minnesota  
New Jersey  
New York  
Oregon  
Pennsylvania  
Texas  
Washington

May 21, 2015

The Honorable Fred Upton  
Energy and Commerce Committee  
U.S. House of Representatives  
2125 Rayburn House Office Building  
Washington, DC 20515

The Honorable Diana DeGette  
Energy and Commerce Committee  
U.S. House of Representatives  
2368 Rayburn House Office Building  
Washington, DC 20515

**Re: Comments on H.R. 6 – 21<sup>st</sup> Century Cures Act**

Dear Chairman Upton and Representative DeGette:

The Lymphoma Research Foundation (LRF) appreciates the opportunity to provide comments in response to H.R. 6 - -21<sup>st</sup> Century Cures Act ("the Act"). LRF is the nation's largest non-profit organization devoted exclusively to funding innovative lymphoma research and providing people with lymphoma and healthcare professionals with up-to-date information about this type of cancer. LRF's mission is to eradicate lymphoma and to serve those touched by this disease. Please find our written comments below and we look forward to working with you as this legislation moves forward.

**Title I: Discovery**

**Funding for NIH, FDA and CMS**

We applaud the committee for including a funding proposal for the National Institutes of Health (NIH) in the Act. Federally-funded research has played an important role in the medical advances made in lymphoma. Now is the time to seize the opportunity and rededicate our nation's support for medical research. While we are encouraged by the committee's commitment, there are still several factors that have yet to be addressed. The Act requires over 70 provisions including new programs and functions for NIH, the Food and Drug Administration (FDA) and the Centers for Medicare and Medicaid Services (CMS) to implement; yet it only provides increased funding for NIH. We are concerned about the lack of funding increases and the effect it could have on the already stretched resources of these agencies. Furthermore, language should be included to ensure that any new funding will not come at the expense of existing programs. It is necessary to safeguard the redirection of valuable but limited resources.

**Subtitle F: Advancement of National Institutes of Health Research and Data Access**

**Subtitle G: Facilitating Collaborative Research**

Accelerating the discovery, development and delivery of new treatments is a commendable objective, and one which LRF supports. Privacy concerns should not prevent patients from participating in clinical research and so revision to several provisions in the Health Insurance Portability and Accountability Act (HIPAA) Privacy Rule must be undertaken carefully. Patients must be encouraged to be active participants in their own healthcare and must feel in control of their own personal and medical information. It is necessary to demonstrate to

the patient community how these two concepts can co-exist. Patients should receive written, easily understood notification of how their health information is used and when their health information is disclosed to third parties. The legislation directs that the Secretary of Health and Human Services may make the necessary changes to HIPPA. We recommend that the Secretary allow for guidance and comments from the patient community on the best way to ensure that they are active participants in this process.

#### **Subtitle H: Council for 21<sup>st</sup> Century Cures**

The public-private partnership established in this section offers the potential to accelerate the discovery, development, and delivery of innovative treatments and preventive interventions for patients. Patient advocacy organizations play a unique role in the patient experience. They strive to change the future for everyone whose life has been affected by a specific diagnosis and that gives them unparalleled insights and knowledge about the particular needs of patients. Advocacy organizations have experience in nominating and referring interested and qualified patients to serve as representatives on a host of federal commissions, panels and advisory committees. We believe that patient advocacy organizations could be effective in ensuring the patient perspective is similarly represented on the Council.

#### **Title II – Development**

##### **Subtitle B: Qualification and Use of Drug Development Tools**

A lymphoma patient faces a difficult and complex process as it relates to their diagnosis and treatment. Lymphoma is unique in that there are more than 67 subtypes of the disease, each considered to be a rare diagnosis which is notorious for recurrence.

The use of biomarkers is becoming an increasing focus of lymphoma research. Biomarkers have the potential to support diagnostics, and prognostic and therapeutic decision-making. To this end, biomarkers offer the hope of early detection as well as for tracking of the disease progression and recurrence. Early detection could improve survival, identify tracking disease progression and biomarkers could potentially allow for patient-specific selection of therapeutic agents that are likely to be effective and less toxic. As outlined in the legislation, which we support, surrogate endpoint qualification and utilization would be streamlined to address the qualification of biomarkers in order to accelerate product approval and FDA would be tasked with publishing guidance documents concerning biomarker qualification.

##### **Subtitle C: FDA Advancement of Precision Medicine**

This section would require FDA to issue, and periodically update guidance documents intended to help the advance the clinical development of genetically targeted treatments by clarifying how investigations could be designed to answer specific questions about narrow subpopulations of patients. Precision medicine could aid in the identifying and analyzing a patient's specific abnormality and assist in determining the best therapy to treat them specifically. Precision medicine holds great promise for lymphoma patients because it could ensure that treatments are precisely targeted to the unique molecular and genetic characteristics of an individual's disease. It could allow for better treatment outcomes and fewer adverse effects compared to other approaches.

We commend the committee on their efforts and appreciate the opportunity to submit comments for consideration.



Robin Roland Levy  
Director, Public Policy and Advocacy