



April 9, 2015

VIA ELECTRONIC MAIL

cures@mail.house.gov

The Honorable Fred Upton
Chairman, Committee on Energy and Commerce
U.S. Representative for Michigan
2183 Rayburn House Office Building
Washington, DC 20515

Dear Chairman Upton,

Adventist Health System (AHS) welcomes this opportunity to comment on the *Advancing Care for Exceptional (ACE) Kids Act of 2015*. AHS is the nation's largest not-for-profit Protestant health care provider. Our organization includes 45 hospital campuses located across 10 states and comprises more than 7,700 licensed beds. Through this network of facilities, AHS provides inpatient, outpatient and emergency room care for four million patient visits each year. However, it is our children's hospital, Florida Hospital for Children that drives home the importance of this legislation for our organization. Part of our flagship hospital in Orlando, Florida, Florida Hospital for Children had more than 154,000 children visits and 57,000 discharges in 2014, and houses 120 dedicated pediatric beds (excluding neonatal).

AHS commends Congress for recognizing the magnitude of the issue of children suffering from medically complex conditions. As noted in the legislation, approximately 3 million children in the U.S. suffer from medically complex conditions. These children account for approximately 6 percent of Medicaid enrollees and 40 percent of children's Medicaid spending due to the severity of the illnesses. The movement towards care coordination is key.

Recognizing this, and in response to the needs of the Greater Orlando community, Florida Hospital for Children developed the Coordinated Care for Kids Program. This program helps provide our patients under 18 years of age with effective care management for medically complex conditions. It is important to note that this is not a revenue producing program but rather a program that is centered on providing our medically complex pediatric patients with the most effective care possible. Because of our leading team of specialists we have been able to see reductions in overall cost of care, cost avoidance by way of decreasing emergency room and inpatient stays, as well as reduction of Length of Stay (LOS) for those who do get admitted. Because Florida Hospital for Children is a part of a larger system, we can afford to have the Coordinated Care for Kids Program.

Our experience over the past two years provides us with a valuable perspective on the proposed legislation. Below, please find our comments on the *ACE Kids Act of 2015*.

Medicaid Children's Care Coordination (MCCC) Requirements

Under Section 1947, entitled "Medicaid Children's Care Coordination Programs for Children with Complex Medical Conditions," the legislation outlines MCCC program requirements and how patients will be initially assigned to a Nationally Designated Children's Hospital Network. Under Section 1947 (c)(2)(C) the legislation states that a child's assignment to a Nationally Designated Children's Hospital Network "ensures access to a medical home that is located not more than 30 miles of the primary residence of the child." Given the special needs of the children and the lack of specialists who can treat these children, the 30 mile limit is too restrictive. Our program routinely cares for children who live 150

Extending the Healing Ministry of Christ

900 Hope Way | Altamonte Springs, Florida 32714 | 407-357-1000



miles away from our facility. **AHS recommends that the 30 mile limit should be increased to 150 miles.**

Following the 90-day period, the legislation states that “the child may elect” to be assisted to a Nationally Designated Children’s Hospital Network of their choice *or* not participate in any MCCC program and receive care through enrollment in the State plan. **AHS requests clarification that this in fact is not the child who will elect to be redesigned but rather the child’s parent or guardian.**

Transition from Fee-for-Service (FFS) to Risk-Based Payment Models

Section 1947 (g)(2)(A) addresses the transition from a FFS to a risk-based payment model. The legislation proposes that for the first two years a per capita care coordination payment will be provided in addition to FFS payments.

Florida Hospital for Children Coordinated Care for Kids Program has been providing complex care management described in the legislation for two years without receiving payment for integrated care delivery. We are able to afford to provide this level of integrated care because we are part of a larger system and because we are committed to developing future models of care. However, for many hospitals (such as freestanding children’s hospitals) providing coordinated care is not sustainable in the current FFS payment model.

AHS thanks Congress for recognizing the need for a coordinated care payment and supports the introduction of per capita care coordination payment in addition to a FFS payment. The legislation proposes that during these two years of concurrent FFS and per capita care coordination payments the Secretary will analyze “verifiable Medicaid Analytic Extract data or a comparable data set” for the purpose of developing a risk-based payment methodology to be implemented in the third year of implementation of the MCCC program. **AHS requests further information on the type of data that will be analyzed and how this data will be utilized in order to develop a risk-based payment methodology.** We are concerned that the risk-based payment methodology will not be sensitive enough to account for the variations seen in local markets. A methodology based upon national data simply may not be adequate.

Additionally, the legislation proposes that the per capita care payment would apply to items and services “for which an all-inclusive payment model is more suitable than fee-for-service reimbursement.” **AHS requests further information on the criteria for types of items and services that would be deemed more suitable for an all-inclusive payment model opposed to FFS.** It is to be remembered that complex cases are being seen. The treatment needs to be tailored to the individual and not to the payment methodology. The database required to be able to move to a per capita amount, that takes into consideration the expenditures for items and services being used, will have to be very robust.

While the legislation makes significant steps towards more appropriate payment for complex care, what remains unaddressed is the lack of recognition of the scarcity of pediatric expertise. Largely, this can be attributed to the fact that Medicaid reimbursement rates are extremely low compared to cost of care. This has led some physicians to opt not to take on Medicaid patients. **If Medicaid financial data will be the basis of developing a risk-based payment model, AHS has serious concern that the new risk-based payment model will not adequately or appropriately pay for coordinated care of complex conditions.** It is critical that Medicaid reimbursement for subspecialty care accurately pays for the care provided by pediatric physicians providing complex care management. If Medicaid reimbursement rates during the first two years of the MCCC programs remains the same as now, **AHS proposes that a study**

Extending the Healing Ministry of Christ



be performed to analyze the difference in cost of service and Medicaid reimbursement rates in order to come up with a risk-based payment model that makes it possible for providers to provide

effective care. We would be willing to share our cost data based upon our actual history of treating complex cases. As we are not being reimbursed for care coordination services, our data should provide a fair assessment of what is actually needed as compared to what generates income.

Nationally Designated Children’s Hospital Network

Section 1947 (j)(5)(A) defines the Nationally Designated Children’s Hospitals Network as a “network of hospitals and health care providers” that:

- are anchored by a qualified children’s hospital or hospitals with principal governance responsibility over the hospital network;
- have the full complement of health care providers needed to provide the best care for children;
- represent the interest of physicians, other health care providers and the family of medically complex children.

AHS is concerned that such broad guidelines leave room for hospitals without a significant, and demonstrated, commitment for children’s care to participate as a Nationally Designated Children’s Hospital Network. We believe that this is not in the best interest of patient care, the children receiving this care and their families. **AHS recommends that the definition of the Qualified Children’s Hospital should be modified with the proposed criteria outlined in the section below.**

Definition of Qualified Children’s Hospital

The legislation provides a definition of a Qualified Children’s Hospital, which includes a provision on Medicaid reliance, highlighting a threshold of “at least 30 percent of the pediatric discharges or inpatient days (excluding observation days) in the hospital.” **AHS believes this threshold is too low and recommends that it be raised to 40 percent.**

It is important to note that many leading children’s hospitals, such as Florida Hospital for Children, are not freestanding and are in fact a hospital within a hospital. **AHS believes that while the legislation does not currently exclude children’s hospitals that are within a larger hospital from participation, additional criteria should be added to the legislation’s definition of a Qualified Children’s Hospital.** We suggest the following additions.

The children’s hospital must:

- have separate medical staff;
- have a separate leadership structure;
- have a pediatric department separate from adults;
- provide the majority of services in a contained area separate from adult care areas;
- have an emergency department staffed by trained pediatric staff;
- provide emergency services staffed by pediatric certified physicians;
- and demonstrate a network of pediatric sub specialists that will take Medicaid.

Additionally, the legislation does not indicate who will designate the National Designated Children’s hospital. **AHS requests that the legislation be amended to provide information on who will designate hospitals as a Qualified Children’s Hospital.**

Extending the Healing Ministry of Christ



AHS very much appreciates your individual and collective interest in children with medically complex conditions. Please do not hesitate to contact us, if you would like to discuss further.

Sincerely,



Richard E. Morrison
Vice President, Government & Public Policy
Adventist Health System
Rich.Morrison@ahss.org
407-303-1607



Deborah Spielman
Chief Operating Officer
Florida Hospital for Children
Office: 407.303.6950
Mobile: 407.832.2283

CC: U.S. Senator Chuck Grassley, U.S. Representative Joe Barton, U.S. Senator Michael Bennet and U.S. Representative Kathy Castor

Extending the Healing Ministry of Christ

900 Hope Way | Altamonte Springs, Florida 32714 | 407-357-1000



April 9, 2015

VIA ELECTRONIC MAIL

cures@mail.house.gov

The Honorable Fred Upton
Chairman, Committee on Energy and Commerce
U.S. Representative for Michigan
2183 Rayburn House Office Building
Washington, DC 20515

Re: 21st Century Cures Draft Legislation

Dear Chairman Upton,

Adventist Health System (AHS) welcomes this opportunity to comment on the *21st Century Cures* discussion draft. AHS is the nation's largest not-for-profit Protestant health care provider. Our organization includes 45 hospital campuses located across 10 states and comprises more than 7,700 licensed beds. Through this network of facilities, AHS provides inpatient, outpatient and emergency room care for four million patient visits each year.

AHS commends you and the Committee for its efforts to accelerate medical innovation through the *21st Century Cures* Initiative. We appreciate the hard work set forth in crafting this legislation and thank the Committee for engaging health care stakeholders on this important topic.

Below, please find AHS's comments.

Patient-Focused Drug Development

Section 1001 calls for the development and use of patient experience data to enhance a structured risk-benefit assessment framework in the new drug approval process. According to this section, the Food and Drug Administration (FDA) will incorporate this patient experience data into the regulatory-decision making process, including the assessment of desired benefits and tolerable risks associated with new treatments.

AHS supports the use of patient experience data by the FDA and agrees that such information regarding a disease and its treatment will bring a real-world perspective to drug development. However, we recommend that the patient experience data collected be truly representative of the selected patient population. It is generally true that people are more likely to report bad experiences than good ones. Therefore, we believe that there may be an inherent bias in the data. We recommend the Agency to address this issue by conducting a statistically valid sampling, or other statistical measure, to overcome response bias.

AHS also believes that the FDA should better integrate the proposed patient experience data with that of our European partners. In concept, we do not see a reason why the clinical experience with a pharmaceutical used in Europe could not be more efficiently applied to our domestic approval process. It is our view that the evaluation of this data will enhance the drug development process.

When overseeing the development of new drugs, AHS also encourages the FDA to consider segmenting the risk and reward of new pharmaceuticals on both a gender and an ethnic-specific basis where indicated. Women respond differently to drugs than men and certain ethnic groups also respond differently. It is our belief that the testing of clinical efficacy and risk should account for genetic factors that may affect the efficacy of a drug, the dosage or the course of administration. This is not touched upon in the legislation. Calling for a new initiative along these lines will go far towards making treatment more patient-focused and effective.

Approval of Breakthrough Therapies

Section 1041 clarifies that the FDA may approve a drug that has received a breakthrough therapy designation under Section 506(a) of the *Federal Food, Drug and Cosmetic Act* (FFDCA). This designation will be granted when early stage clinical data provides sufficient evidence under the current safety and efficacy standards.

AHS supports the above policy and commends the Committee for its inclusion in this legislation. Given the rise in drug resistant bacteria and both the health and economic consequences created by these mutations, it is imperative to establish a national policy that advances the development, testing and dissemination of new approaches to the threat of bacteriological infections.

Section 1041 includes a limitation that will allow the Secretary of the Department of Health and Human Services (HHS) to require drug sponsors to conduct a post-market assessment plan in order for a particular drug to be approved. Such a plan will be based on an agreement between the Secretary of HHS and the sponsor of the drug. **AHS supports this policy and believes that a focused post-market assessment plan will allow for a more timely withdrawal of a drug if this one is either found ineffective or has unanticipated side effects or harms.**

Cures Acceleration Network

Section 1202 authorizes additional funds to the National Center for Advancing Translational Science (NCATS) for research on repurposing drugs for new uses. The Center will award grants and contracts for the research and development of high-need cures based upon new indications for drugs and biological products that have been previously approved or licensed by the FDA.

AHS supports this provision and believes that it will facilitate a faster response to emerging research on high-need cures. It is also our view that the use of existing approved pharmacological agents can potentially provide lower cost treatment.

Building a 21st Century Data Sharing Framework

Sections 2081 to 2092 under Subtitle F establish a data sharing framework to enable patients and physicians to better identify ongoing clinical trials and improve the quality of patient care.

AHS supports the creation of such framework as long as the Secretary of HHS ensures compliance with the *Health Insurance Portability and Accountability Act of 1996* (HIPAA) and the security of patient-identifiable data. We also urge the Committee to include a limitation within these sections that will only allow the use of this data for research purposes or any other activity pertaining to medical innovation.



The same limitation should apply to Section 2201, which requires all National Institute of Health (NIH) grantees to share their data with the federal government. It is our concern that without this limitation, HHS may use this data for other purposes contrary to the goals of this legislative initiative.

AHS also appreciates the recommendation proposed under Section 2092, the promotion of bidirectional, interoperable exchange of information between Electronic Health Records (EHR) and clinical registries. We believe that it is critical for HHS to adopt and better enforce interoperability standards to ensure the seamless exchange of information between certified EHR and qualified clinical data registries.

Innovation Cures Consortium

Section 281 calls for the establishment of an Innovative Cures Consortium to foster collaboration among the Consortium, government agencies, academia and other stakeholders to advance the discovery, development and delivery of innovative cures.

AHS agrees that an Innovation Cures Consortium can help promote medical innovation. Involving as many parties as is practical in the discovery and development process of innovative cures, mirrors recent developments in the private sector. Universities, health care organizations and business interests are currently coming together to support and fund focused efforts in creating new therapeutic and diagnostic approaches. **AHS recommends the Committee to coordinate and seek partnerships with the local consortia so there are no duplicative efforts.**

Section 281B outlines the duties of the proposed Consortium and highlights interoperability as one of those duties. AHS supports the need for secure and interoperable health information and believes it to be fundamental in our new health care environment. We commend the Committee for highlighting this issue and look forward to seeing more details regarding Section 2181, the legislation's provision on interoperability.

21st Century Chronic Disease Initiative Act

Section 2241 requires the Secretary of HHS to develop a plan to carry out a longitudinal study designed to improve the outcomes of patients with chronic disease.

AHS strongly supports this provision as we find chronic disease to be a very costly problem but yet an often ignored policy. According to a study by the Milken Institute, the annual economic impact on the U.S. economy of the most common chronic diseases is more than \$1 trillion, which could increase to nearly \$6 trillion by the middle of the century. **While there is much to be learned by undertaking such proposed longitudinal study, the Committee should also consider using the Medicare database to conduct a retrospective study of chronic disease.** It is our belief that much could be learned about chronic disease progresses, the effectiveness of interventions and the timing of care and associated costs, using historical data on outpatient, inpatient and ancillary claims.

AHS also encourages the Committee to provide a clear avenue for reimbursement of remote patient monitoring for chronic disease. Under the Center for Medicare and Medicaid Services' (CMS) definition of telehealth services, remote patient monitoring is not a telehealth service, rather it is a physician service. However, only in rare cases has CMS assigned a value to the remote patient monitoring codes. We believe that it is essential that physicians are permitted to use and receive reimbursement for remote patient monitoring when cost savings and improved health care outcomes are supported by evidence.

Advancing Care for Exceptional Kids

Sections 4361 to 4362 under Subtitle R, call for the establishment of a Medicaid and Children's Health Insurance Program (CHIP) Care Coordination program for children with complex medical conditions. This language is consistent with H.R.4930, the *Advancing Care for Exceptional (ACE) Kids Act of 2015*, which AHS has provided separate comments on. AHS commends the Committee for recognizing the magnitude of the issue and addressing it in this legislation.

As noted in the *ACE Kids Act*, approximately 3 million children in the U.S. suffer from medically complex conditions. AHS wholeheartedly agrees that the movement towards care coordination is the key to solving this issue. Florida Hospital for Children, which is part of our flagship hospital in Orlando, has a Coordinated Care for Kids Program that has been operational for two years. This program helps provide our patients under 18 years of age with effective care management for medically complex conditions. With this experience in mind, we recommend the Committee to adopt the legislative changes below.

Section 1947 (c)(2)(C), explains that a child's assignment to a Nationally Designated Children's Hospital Network will ensure a child's access to a medical home located not more than 30 miles away from the primary residence of the child. Given the special needs of the children and the lack of specialists who can treat these children, we believe that the 30 mile limit is too restrictive. Our program routinely cares for children who live 150 miles away from our facility. **AHS recommends the Committee to increase the 30 mile limit to 150 miles.**

Section 1947 (j)(5)(A) provides a broad definition of a Qualified Children's Hospital and the criteria to meet that definition. It includes a provision on Medicaid reliance, highlighting a threshold of "at least 30 percent of the pediatric discharges or inpatient days (excluding observation days) in the hospital." **AHS believes that this threshold is too low and recommends that it be raised to 40 percent.** AHS believes that while the *ACE Kids Act* does not currently exclude children's hospitals that are within a larger hospital from participation, **additional criteria should be added to the legislation's definition of a Qualified Children's Hospital.**



AHS appreciates your diligent efforts to accelerate medical innovation and reduce regulatory burden. We look forward to continuing the national dialogue to maintain our nation's standing as the biomedical innovation capital of the world. Should you or your staff wish to discuss further, please do not hesitate to contact me.

Sincerely,



Richard E. Morrison
Vice President, Government & Public Policy
Adventist Health System
Rich.Morrison@ahss.org
407-303-1607

Energy and Commerce Committee 21st Century Cures Act Discussion Draft: American Diabetes Association Comments

On behalf of the nearly 30 million individuals living with diabetes and the 86 million individuals with prediabetes, the American Diabetes Association (the Association) is grateful to Chairman Fred Upton and the members of the House Energy and Commerce Committee for the opportunity to review and provide comments regarding 21st Century Cures Act discussion draft. The Association commends Chairman Upton and Representative DeGette for leading the 21st Century Cures Initiative effort, which started in 2014 to help ensure that the United States can be the worldwide leader in biomedical innovation.

For the diabetes community, there are numerous reasons for greater investments in research, literally millions and billions. In addition to the horrendous physical toll, diabetes is economically devastating to our country. A 2014 report published in *Diabetes Care* found the annual cost of diagnosed, undiagnosed, prediabetes, and gestational diabetes has skyrocketed by an astonishing 48 percent over five years to from \$322 billion.

We believe that the cycle of discovery, development, and delivery should be fostered. Supporting this cycle of innovation is in the best interest of patients and their loved ones, researchers, health care providers, our economy and society. In this spirit, we are happy to share comments on the discussion draft for your consideration. Our comments focus on four areas including: 1) Sections 1021-1024 of the discussion draft regarding the use of surrogate endpoints; 2) Section 2241 of the draft regarding the longitudinal study on outcomes of individuals with chronic disease 3) In Title IV, the need for additional resources at the National Institutes of Health (NIH) and the Centers for Disease Control and Prevention (CDC), and 4) the need for support at the NIH for study of the microbiome, which we believe should be added to Title IV of the draft. Our comments are organized in the order they appear in the discussion draft. Items 2 and 4 offer suggested language in line with our comments.

Comments and Recommended Changes to the 21st Century Cures Discussion Draft

1) Sections 1021-1024: Surrogate Endpoints

Explanation

This is a topic of great importance to the Association and individuals with diabetes. Thanks to medical advances, people can live with chronic illnesses for 20 to 60 years. It is not feasible to conduct controlled clinical trials over that timespan in order to study all potential outcomes; so surrogate endpoints are a feasible means to measure the effect of a treatment. Historically, A1C measures have been used as a clinical endpoint related to diabetes care. However, measuring A1C does not provide a measure of glycemic variability or hypoglycemia. Hypoglycemia is a condition characterized by abnormally low blood glucose, and is a key factor in developing patient glycemic goals. Therefore the Association supports use of hypoglycemia as an endpoint in all diabetes-related research.

2) Section 2241: Plan for Longitudinal study on Outcomes of Individuals with Chronic Disease

Explanation

The Association is pleased the Committee included a requirement to develop a longitudinal study designed to improve outcomes of patients with chronic disease. According to the Centers for Disease Control and Prevention, chronic diseases and conditions such as heart disease,

stroke, cancer, diabetes, obesity and arthritis are among the most common, costly, and preventable of all health problems.ⁱ Given that many people can live with chronic illnesses for many years, traditional research study outcomes – while still important - may not be of immediate concern to individuals living with a chronic illness, like diabetes. The Association believes there is a need to change the conversation in this area, shifting focus away from utilizing morbidity and deadly cardiovascular disease outcome measures exclusively and instead focus on how people with diabetes can live productively with the disease. Towards this end, we would suggest amending the discussion draft to include language that underscores the need for research and a regulatory environment which stresses the importance of patient-reported intermediate outcomes, including an individual’s productivity and quality of life.

Suggested Language

On page 215, paragraph 1, line 11, after “the,” add, “intermediate and longer- term.”

On page 216, paragraph 3, line 21, before “outcomes,” add, “intermediate and longer-term.”

3) The Need for a Significantly Increased Federal Investment at NIH and CDC

Explanation

The Association notes that Title IV of the discussion draft focuses in large part in accelerating the process of research at NIH and CDC. While the Association appreciates the Committee’s work to address these needs, we believe additional and significant increases in these valuable agencies is required to ensure this acceleration in research is successful.

The federal government is uniquely positioned to provide the leadership and financial resources to spur the discoveries through the National Institutes of Health (NIH), particularly at the National Institutes of Diabetes, Digestive and Kidney Diseases (NIDDK) necessary to tackle diabetes and other diseases. Unfortunately, while progress has been made because of the federal investment in biomedical research, the attacks on biomedical and translational research funded by the federal government continue to threaten the U.S. position in research and discovery. Federal funding for biomedical research at NIH represents less than 1% percent of overall spending. Federal funding for diabetes research represents less than one-half of 1% percent of overall spending, despite diabetes taking more lives than breast cancer and HIV/AIDS combined. If our country is to remain at the forefront of biomedical discovery, a deeper and consistent investment by the federal government is mandatory.

4) The Need for NIH Research on the Microbiome

Explanation

As the Committee continues to discuss opportunities to build upon the tremendous research being conducted and funded by the NIH, the Association urges you to consider including a focus for microbiome research. The microflora that colonize the gut of humans are diverse and numerous, and their collective genome is referred to as the microbiome. Changes in the composition of the gut microflora are associated with obesity and diabetes. Similarly, the microbiome composition of individuals at risk for type 1 diabetes is distinct from that of unaffected individuals, suggesting there may be a link between the microbiome and the development of autoimmunity. At the Association’s 2014 Scientific Sessions, our annual research conference, standardization of microbiomal research and data was raised as a major unmet need. Therefore, the Association urges the Committee to ensure this important area of

study can move forward by including language in a new section of Title IV of the discussion draft that provide federal support for this work.

Suggested Language

On page 255, insert a new paragraph and the following:

Sec. 4010: Authorization of Appropriations for NIH Microbiome Research

the Public Health Service Act is amended by adding at the end the following:

“(5) AMOUNTS.—For the purpose of carrying out this section, there is authorized to be appropriated \$10 million for each of fiscal years 2016 through 2020.

ⁱ Centers for Disease Control and Prevention, Chronic Diseases and Health Promotion, May 2014. Available at: <http://www.cdc.gov/chronicdisease/overview/#sec3>.



9312 Old Georgetown Road
Bethesda, MD 20814-1621
Tel: 301-571-9200
Fax: 301-530-2752
www.apma.org

April 10, 2015

The Honorable Fred Upton
Chairman
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

RE: 21st Century Cures Act Discussion Draft

Dear Chairman Upton and Representative DeGette:

The American Podiatric Medical Association (APMA), representing more than 12,000 doctors of podiatric medicine and millions of patients for whom they provide care, appreciates the opportunity to provide comments on the 21st Century Cures Act discussion draft released on January 27, 2015. We applaud your continued commitment to this important initiative to streamline the implementation of new medical treatments and look forward to working with you as this work moves forward.

Podiatrists, also known as podiatric physicians and surgeons or doctors of podiatric medicine (DPMs), are qualified through their education, training, and experience to furnish the same services and provide the same care as other physicians and surgeons treating the foot and ankle and related structures of the lower leg. Thus, as in Medicare and other public and private plans, care by podiatrists is covered as a vital component in the continuum of medical care.

Our members would be directly impacted by several provisions in this discussion draft. APMA would like to provide comments and recommendations on the following sections of the discussion document.

Sec. 2181. Interoperability

APMA looks forward to additional information on Section 2181 concerning interoperability and working toward the goal of an interoperable health information infrastructure. The promise of 21st Century Cures is highly correlated to the ability of physicians and patients to use technologies that support effective communication and allow for the easy transfer of information throughout our health-care delivery system. However, there are substantial barriers to making this a reality.

We are very concerned that Electronic Health Records (EHR) adoption is not achieving the goals set out by Congress, namely increased efficiency, improved health outcomes, and better access to

electronic information. We believe this is largely because the program has failed to facilitate interoperability across systems and providers. We are likewise concerned that Meaningful Use (MU) is becoming more and more of a burden on health care providers with little improvement in patient care quality or health and where the costs of the program may outweigh the program's benefits. There are several reasons for this, including: a process that produces poor results; misaligned time frames; poorly defined measures; and a lack of focus on achievable short-, mid- and long-term outcomes.

We strongly urge the Committee to consider that improving interoperability and usability of EHRs is tied to streamlining MU regulations for physicians. More effective methods to encourage EHR adoption may include:

- Removing the rigid pass-fail approach of the MU program;
- Better alignment of the Physician Quality Reporting System (PQRS) program and MU quality reporting requirements; and
- Expanded current hardship exemptions.

Sec. 4141. Disposable Medical Technologies

APMA supports the inclusion of the disposable medical technologies coverage language under Medicare. Unfortunately, current Medicare law is antiquated by not covering certain disposable technologies in the home care setting that may be more cost-effective, promote greater patient compliance, and lead to improved outcomes. These disposable technology items are commonly reimbursed by private payers, as they are easier to use, less expensive, and provide comparable outcomes. Many of these technologies are smaller and designed for single-patient use. They may last a few days, weeks, or months, but not years.

The outdated Medicare definition of DME precludes consideration of these modern technologies suited for home-based care. By providing coverage for disposable medical technologies in the home, Medicare would promote continuity of care between care settings, facilitate better outcomes, reduce costs, and enhance system efficiencies. Moreover, Medicare coverage would ensure that patients do not lose access to these technologies as they transition from private insurance at age 64 to Medicare at age 65.

Sec. 4161. Local and National Coverage Decision Reforms

We welcome the inclusion of local coverage determination (LCD) reforms. The Medicare program issues thousands of coverage determinations relating to medical efficacy each year. The overwhelming majority of such determinations issue from Medicare's insurance contractors (fiscal intermediaries and carriers); these LCDs govern only in the contractors' catchment areas. To date, Medicare contractors have issued more than 2,000 LCDs. Most LCDs are not all-or-nothing determinations and instead provide that the treatment in question will be covered only for certain populations or conditions.

As you know, in January 2014, the US Department of Health and Human Service's Office of the Inspector General (OIG) released a report focused on the variation in coverage of Part B items and services as a result of LCDs. Key findings in the OIG report include the following:

- LCDs limited coverage for Part B procedure codes differently across States;
- LCDs prohibited coverage for some procedure codes—often those for new technology—in some States and not in others;
- LCDs limited coverage for many Part B items and services in some States and few items and services in others; and
- LCDs defined similar clinical topics inconsistently.

The current LCD process can increase the burden on providers and contractors, since different rules apply depending on the location of the service provided. Moreover, LCDs have not been targeted to the most costly, highly-utilized services in a consistent way and may lead to discrepancies in access to care depending on the beneficiary's location.

Consistent with the OIG's recommendations, APMA believes that your 21st Century Cures legislation should establish a framework to evaluate new LCDs for national coverage consistent with Medicare Modernization Act requirements; put in place mechanisms to increase consistency among existing LCDs; and consider requiring MACs to jointly develop a single set of coverage policies.

Sec. 4241. Global Surgery Services Rule

APMA has serious concerns with the CMS final rule relating to transitioning and revaluing 10-day and 90-day global surgery services with 0-day global periods through a yet-to-be-determined methodology, and make these changes effective in CY 2017 (for 10-day global services) and CY 2018 (for 90-day global services). This final rule would have significant implications for doctors of podiatric medicine (DPMs), since we estimate that 10- and 90-day global surgical services account for roughly 14 percent of all Medicare payment for the podiatry specialty, based on finalized 2015 Medicare payments and 2013 utilization.

First and foremost, APMA strongly opposes any attempt to implement an artificial deadline for unbundling global surgical services. It is evident from the final rule that CMS is uncertain about whether and how this might be done in a fair and practical way. We believe that setting implementation deadlines in the absence of more information regarding methodology both precluded adequate public input.

Second, any unbundling of the 10- and 90-day global surgical services must guarantee that the values for the base procedures as 0-day global can be determined accurately and fairly and not end up disadvantaging these services compared to other services paid for under the Medicare physician fee schedule. APMA believes the jury is still out on this. In particular, we believe there

is considerable risk that the unbundling process will end up disadvantaging those who provide surgical services by reducing both direct and indirect practice expense payments to these specialties. As it is, CMS understands that direct and indirect practice expense costs are not fully reimbursed under the existing practice expense methodology, and we believe it would be important to demonstrate that any unbundling proposal would not worsen this situation for the affected specialties compared to non-surgical specialties. Unjustified reductions in practice expense payments do not miraculously eliminate real-world practice expense costs. If equipment and supplies used for post-procedure visits are not taken into account in valuing evaluation and management services for non-surgical problems, this would appear to disadvantage physicians furnishing post-procedure visits following the unbundling of 10- and 90-day globals.

APMA does see some value in unbundling in that it would allow more payment for difficult cases, where additional post-procedure visits are needed. This is not a feature of the existing global surgical definition in that the 10- and 90-day global surgical services are valued based on the typical patient, as are all other services reimbursed under the Medicare physician fee schedule. However, we also fear that, post unbundling of the 10- and 90-day globals, physicians furnishing surgical services would face inordinate scrutiny and find themselves constantly arguing with Medicare about the medical necessity of every single post-procedure visit. In the context of the current enormous backlog in Medicare appeals related to Part B claims, we see this additional potential area of disagreement as an enormous risk for physicians, beneficiaries and even the Medicare program itself. In addition, we worry that separate billing for each post-procedure service and the separate cost-sharing amounts for each such service might cause some beneficiaries to avoid medically necessary post-procedure care, even if their total cost-sharing obligations might otherwise end up being no different than they are today under the global surgical service policy. This unintended reduction in patient compliance could lead to greater postoperative morbidity and diminished outcomes, and even have professional liability implications.

In addition, there are other important issues that would need to be addressed if any unbundling initiative were to move forward. For example, it would be absolutely unfair to apply the existing multiple procedure payment reduction policy across all the newly created 0-day global surgical services. That policy was primarily justified by the fact that a single set of post-procedure visits would be furnished following multiple, same-day procedures, rather than the otherwise assumed post-procedure visits associated with each of the individual procedures. However, if and when the unbundling of 10- and 90-day global surgical services occurs, this issue would be addressed since physicians would bill separately for each visit. This multiple procedure payment issue was not addressed in the final rule but is a major consideration. If this matter is not addressed properly, it could negatively affect patient care, as physicians would become understandably hesitant to furnish multiple procedures, even if this is clinically appropriate, if they knew that the Medicare payment result would unfairly penalize them. Similarly, there are a number of payment modifiers currently in use in relation to global surgical services and it would be necessary to review all of these and determine which would no longer be needed and which would continue to

apply under certain circumstances. We also cannot rule out the possibility that the unbundling of global surgical services could impact the correct coding initiative. None of this was addressed in the final rule.

It is also true that the unbundling of 10- and 90-day global surgical services is likely to increase the volume of claims submitted to Medicare, thereby increasing the Medicare claims submission burden and claims processing costs, since physicians furnishing surgical services would probably end up submitting more than one claim for each procedure. We see this as another area of concern not acknowledged in the final rule. Are Congress and CMS prepared to increase Medicare contractor budgets to accommodate the increased number of claims that would likely be submitted and processed? Or is CMS planning to force physicians furnishing surgical services to submit only a single claim covering both the 0-day global procedure and all post-operative visits? If so, this would unfairly distinguish between evaluation and management services furnished post-procedure and those furnished during an episode of care for non-surgical problems, or distinctly different conditions. This would be unacceptable. Resulting increases in the number of claims could also affect other contractor costs related to auditing, pre- and post-payment review, and other activities.

The final rule raises concerns about the impact of current global surgical service payment policies on alternative payment models. However, it is also true that alternative payment model constructions typically rely on historic Medicare data and it is far from clear to us how this historic data would be adjusted going forward in the context of alternative payment models. Any such adjustments to historic data once again risk disadvantaging physicians who furnish surgical services under one or another alternative payment model.

Sec. 4381. Exempting From Manufacturer Transparency Reporting Certain Transfers Used for Educational Purposes

APMA strongly supports the inclusion of this provision which clarifies that peer-reviewed journals, journal reprints, journal supplements, and medical textbooks are excluded from the reporting requirement under the Sunshine Act. Physicians must have access to the most up-to-date independent medical knowledge to support their delivery of high quality patient care.

The Helping Ensure Life- and Limb-Saving Access to Podiatric Physicians (HELLPP) Act

APMA strongly urges the Committee to consider inclusion of the HELLPP Act provisions (HR 1221 / S 626) aimed at removing patient access barriers to podiatric physicians and surgeons.

Foot and ankle care provided by podiatrists is essential to any comprehensive national health-care program, especially as the Committee is seeking to modernize health programs to reflect 21st Century medicine. It is important to ensure patient access, especially Medicaid patients, to timely and early specialty medical and surgical foot and ankle care in order to prevent chronic conditions from becoming an even greater cost burden for our public health programs. Numerous studies underscore that when podiatric physicians and surgeons are providing medically necessary foot and

American Podiatric
Medical Association, Inc.

ankle care, patient outcomes are better, hospitalizations are fewer and shorter, and our health system saves billions of dollars annually.¹

DPMs are on the front line everyday identifying patients at risk for a variety of conditions, including but not limited to diabetes, peripheral arterial disease, and arthritis, as well as treating and preventing complications from these conditions.

Even though foot and ankle care is a covered benefit under the current Medicaid program, access to medical and surgical foot and ankle care *provided by a podiatrist* is considered optional and is not covered by all state plans. As a result, Medicaid patients have limited access to specialized foot and ankle medical and surgical care.

The HELLPP Act would remedy this access barrier by recognizing podiatrists as physicians, just as they are in Medicare, to ensure that Medicaid patients—who disproportionately suffer from chronic conditions—have timely access to the most appropriate and best trained providers of foot and ankle care. The Medicare program has recognized doctors of podiatric medicine as physicians since 1967. Additionally, the HELLPP Act clarifies documentation requirements for Medicare’s Therapeutic Shoe Program for persons with diabetes. This provision does not in any way expand the Therapeutic Shoe program. Rather, it would improve coordination of care for beneficiaries with diabetes and result in improved medical care and outcomes, fewer physician office visits and health-care cost savings.

Additionally, the HELLPP Act as introduced contains a budget savings provision which would strengthen Medicaid program integrity. The provision, based on a US Government Accountability Office report and recommendation (GAO-12-857), would allow for improved collection of outstanding tax debts from delinquent Medicaid providers.

APMA applauds your efforts to date on this important initiative and welcomes the opportunity to discuss these comments and to provide additional input as the Committee continues its path toward legislative action on a bill that will generate broad support and, when enacted, will speed

¹ “*The Economic Value of Specialized Lower-Extremity Medical Care by Podiatric Physicians in the Treatment of Diabetic Foot Ulcers*”, Journal of the American Podiatric Medical Association, Vol. 101, No 2, March/April, 2011;

Sloan, F.A., Feinglos, M.N. and Grossman, D.S., RESEARCH ARTICLE: *Receipt of Care and Reduction of Lower Extremity Amputations in a Nationally Representative Sample of U.S. Elderly*. Health Services Research, no. doi: 10.1111/j.1475-6773.2010.01157.x

Details of both studies accessible at: www.APMA.org/saving; “[Fact Sheet: Studies Prove Podiatrists Prevent Complications, Provide Savings](#)”; and

Skrepnek GH, Mills JL, Armstrong DG, “Foot in Wallet Syndrome: Tripped up by 'Cost-Saving' Reductions”, 73rd Scientific Sessions, American Diabetes Association, Chicago, IL, June, 2013.

American Podiatric
Medical Association, Inc.

medical progress. Please contact Scott Haag, APMA's Director of Health Policy & Practice, with any questions about our comments at 301-581-9233 or slhaag@apma.org.

Respectfully,



Phillip E. Ward, DPM
President



an international forum for cell biology

8120 woodmont avenue, suite 750 • Bethesda, Maryland 20814-2762, USA
tel: 301-347-9300 • fax: 301-347-9310 • email: ascbinfo@ascb.org • website: www.ascb.org

March 27, 2015

Representative Fred Upton
Chair
Energy and Commerce Committee
U.S. House of Representatives
Washington, DC 20515

Representative Diana DeGette
Energy and Commerce Committee
U.S. House of Representatives
Washington, DC 20515

Dear Rep. Upton and Rep. DeGette:

I am writing on behalf of the American Society for Cell Biology (ASCB), a professional scientific society of about 8,000 basic biomedical researchers in the United States and 65 other countries. A majority of our U.S. members are funded through the U.S. National Institutes of Health (NIH).

We were pleased last year when you jointly announced that the House Energy and Commerce Committee would begin a long-term, wide-ranging review of the process through which drugs are developed in the United States. We also took note that part of your mandate was to maintain the United States' position as the leader in biomedical research and innovation. Any serious investigation of the pipeline that delivers new treatments and cures without including the critical role of basic biomedical research would be flawed.

In an era of hyper-partisanship, we were equally heartened by your pledge that the work would be done in an open and bipartisan manner.

We have carefully reviewed the first Discussion Draft of the 21st Century Cures Act released in January. While a vast majority of the Discussion Draft focuses on FDA-, CDC-, and CMS-related issues, there are provisions of the bill draft that have significant bearing on the work of the NIH and the NIH community.

We are particularly pleased that the committee has paid attention to the needs of young investigators, who are so important to the continued success and vitality of American science. Efforts to foster high-risk, high-reward research and to reduce the administrative burdens facing researchers are also important to us. I will go

OFFICERS

- SHERLEY M. TILGHMAN
President
- JENNIFER LIPPINCOTT-SCHWARTZ
Past President
- PETER WALTER
President Elect
- KATHLEEN J. GREEN
Secretary
- GARY J. GORBSKY
Treasurer
- STEFANO BRUTUZZI
Executive Director

COUNCIL

- MARTIN CHALPIS
- ANTHONY A. HYMAN
- DANIEL P. KOBHART
- RUTH GELMANIN
- IAN G. MACARA
- LAURA M. MACHESKY
- IRA MULLMAN
- TOM MITTEL
- EMUSEI MONTILL
- JODI RUPNARI
- SAMARA BECK-PETERSON
- CLAIRET WALCZAK

COMMITTEE CHAIRS

- THEODORE T. HO, Co-Chair
- BRUNDA ROCHA-AZEVEDO, Co-Chair
Committee for Postdocs & Students
- SUSAN M. WICK
Education
- GARY J. GORBSKY
Finance & Audit
- YIXIAN ZHENG
International Affairs
- KATHLEEN J. GREEN
Membership
- RENATO J. AGUILERA, Co-Chair
- ANDREW G. CAMPBELL, Co-Chair
Membership Affairs

- DON W. CLIVELAND
Nominating
- JULIE A. THIRNOT
Program
- SEMON J. ATENSON
Public Information

- CYNTHIA M. LEE
Public Policy
- SANDRA K. MASLO
Women in Cell Biology

CBE – LIFE SCIENCES EDUCATION

- BRIS L. DOLAN
Editor-in-Chief

MOLECULAR BIOLOGY OF THE CELL

- DAVID C. DEPUBIN
Editor-in-Chief

into greater detail about these provisions later in this letter. I would also like to share our concerns with you about a number of the provisions but hope this is the just the start of a longer dialog with the Committee on these issues.

Title II, Subtitle O – Helping Young Emerging Scientists

Young investigators are the life's blood of the American biomedical research enterprise. They are the embodiment of the vigor and energy necessary for the field and are, in very sense of the word, the future of the American biomedical research enterprise. The future of the biomedical community is depends heavily on the continued success of these young scientists.

While we are pleased that the bill focuses on these researchers, particularly the development of a unique program to help these investigators as they start their independent careers, we do not support the funding mechanism selected by the Committee. Under the bill, support of these young researchers would be moved away from the NIH institute(s) that supports their field of study and placed in the Common Fund under the direction of the NIH Director.

The Common Fund was created as part of the 2006 NIH Reform Act with the goal of stimulating crosscutting, trans-NIH programs. Congressional authors of the Common Fund saw it as a way to stimulate collaboration among institutes that was, at the time, in short supply. While support for young investigators is certainly spread across all NIH institutes, the support for their area of research remains at the institute that funds their area of science. For that reason, we feel that support for research by emerging researchers must remain at their home institute and not with the Common Fund.

Title II, Subtitle P – Fostering High-Risk, High-Reward Science

The ASCB generally supports the goals of this provision of the bill. We do, however, strongly believe that the responsibility for determining the level of funding available through the program should rest with the individual institute director and not solely the NIH Director. The directors of the individual institutes are closest too and have the most detailed understanding of their institute's overall research portfolio and the funding necessary for that portfolio. For that reason, they should be the ones determining the portion of their portfolio directed to high-risk, high-reward science.

Title IV, Subtitle A – Section 4001 – NIH Research Strategic Investment Plan

The ASCB must strongly oppose this provision. Individual institutes and centers at the NIH already have at least one strategic plan. Some institutes have multiple plans, each focused on particular areas of research. These plans are the result of exhaustive, time-consuming reviews and include meetings with key stakeholders, including members of the scientific and patient's communities associated with the work of the institute.

In addition, the NIH, as an agency within the Department of Health and Human Services (HHS), is already required to participate in the development of the HHS performance planning and reporting requirements. The development of agency strategic plans are required, first by the Government Performance and Results Act (GPRA) of 1993 (P.L. 103-62) and later modified by the GPRA Modernization Act of 2010 (P.L. 111-352). Similar strategic reporting requirements are also part of Office of Management and Budget Circulars A-11 and A-136.

To require each institute to develop additional strategic plans, complete with regular review, would be incredible administrative burdens and a waste of scarce federal resources that could otherwise be used for support for research.

We are also deeply concerned with Use of Plan provision of this section that requires that the strategic plan be used to make both resource allocation decisions and research decisions. Not only could this conflict with the role of Congressional appropriators in allocating funding, but such plans, if too prescriptive, could seriously override the peer review and scientific review process that currently exists at the NIH.

Title IV, Subtitle A – Section 4002 – Biomedical Working Group to Reduce Administrative Burden on Researchers

Any thoughtful efforts to reduce administrative burdens on researchers would be welcomed by the biomedical research community. American biomedical researchers spend too much time away from the lab bench working on administrative issues. The NIH continues to work to improve administrative requirements that come along with being a scientist. One major change was to shorten the NIH grant application, reducing applications by as much as half of the previous length. Many of the solutions to the administrative burdens facing researchers do not demand legislative remedies, some may benefit from legislative encouragement. The ASCB would welcome the opportunity to work with you to identify these areas.

We do, however, object to including the issue of replicability in the mission of the Working Group. All too often, “reproducibility” and “duplication” have one set of meanings in the scientific community and completely different definitions to the public. It is critical to the progress of science that one set of scientific results be able to be repeated many times before being accepted by the community. The inability to replicate a particular experiment, may be the result of a variety of different scientific reasons. Too much duplication and too little replicability should not be viewed negatively.

The solutions associated with replicability are scientific, not legislative. The issue is already being addressed by the scientific community, most notable by the NIH and by scientific organizations such as the ASCB. For that reason, replicability should be removed from the Working Group’s mandate.

Title IV, Subtitle A – Section 4003 – NIH Travel

In 2010 and 2012, media reports about excessive expenditures for IRS and GSA employee travel led to the implementation, first by Office of Management and Budget (OMB) directive and later codified in several FY13 Continuing Resolutions (PL 113-6, PL 113-32, and PL 113-46) and the FY14 Omnibus funding bill (PL 113-76), of limitations on Federal employee travel. As stated in the original OMB memorandum, these limitations were to ensure “that Federal funds are used for purposes that are appropriate, cost effective, and important to the core mission of executive departments and agencies.”

These travel restrictions, and the way they are being interpreted by the U.S. National Institutes of Health (NIH), are having a significant impact on the ability of federal employee scientists at the NIH to do their jobs and participate in the scientific process.

Science does not take place in a vacuum. It is collaborative and depends heavily on the interaction and exchange of ideas and information that can only take place face-to-face at a scientific meeting. It is critical for NIH intramural researchers to share the results of their research with colleagues. It is also critical for them to learn what other research is being done in their area of expertise.

The scientific community does not desire NIH staff, such as program directors, review officers who manage and make decisions about grants, and intramural scientists to be isolated from the rest of the scientific community. These federal employees, must, in order to do their jobs effectively, stay abreast of the latest advances in their scientific areas. The current travel restrictions are equally debilitating for them too.

In short, attending scientific meetings is “important to the core mission” of the NIH and other federal scientific agencies.

A reduction in the participation of federal scientists at scientific meetings is compromising the quality of the research done at federal research labs and will, over time, foster a detached and increasingly isolated federal research program. In addition, the absence of federal researchers is having implications for scientists at universities and institutions around the United States. These scientists benefit from and depend on the scientific advances made in federal laboratories such as the NIH.

Last year, the ASCB worked closely with appropriators to amend this problem. In its Committee Mark of the FY15 Labor, HHS, and Education Appropriations bill, the Senate Appropriations Committee included language in both the bill and the report that offers smart solutions to the travel restrictions currently placed on scientists working at the NIH. We feel these are smart changes that recognize the importance of federal scientists’ participation in scientific meetings without ignoring the original intent of the regulations to limit travel by federal employees to trips that are appropriate, cost efficient and important to the mission of the agency. Similar language was included in a House Appropriations Committee draft of the FY15 Labor-HHS bill and was ultimately included in the final FY15 Omnibus bill.

Language similar to the provisions in the FY15 appropriations bill should be included in this legislation. The impact of travel restrictions on science was, most assuredly an oversight when originally crafted.

Title IV, Subtitle A – Section 4004 – Increasing Accountability at the NIH

(a) Appointment and Terms of Directors of National Research Institutes and Centers

The two provisions in this section are, in many ways, solutions in search of problems. The heads of each of the 27 institutes and centers at the NIH currently undergo regular reviews every five years. The ASCB does not oppose these types of regular review. In fact, regular administrative review is very common in the academic world and is, therefore, something our members are familiar with and supportive of. If the committee feels that existing review process is not rigorous enough, it should work with existing regulations to strengthen the current process instead of creating an additional procedure.

(b) Review of Certain Awards by Directors

We do not understand the requirement for each institute director to “personally review and approve” each R-series award made by their institute.

In fact, this is already what happens and there appears to be no need for legislation. Today, an award can only be paid if personally approved by the Institute Director, who must sign each award as the individual responsible for each award. The line of responsibility is already crystal clear and resides with the Institute Director; any other peer review body serves as advisory to the Director. At the conclusion of the two-year rigorous peer review, each successful grant application is reviewed and signed by the director of the specific institute.

(c) GAO Study on Duplication in Federal Biomedical Research

As mentioned earlier, repeating scientific results plays a central role in science. The duplication of results by one scientist serves to validate similar work already done. The ASCB strongly urges the committee to proceed cautiously in this area to make sure that legitimate efforts to do away with redundant federal activities do not also erroneously eliminate critical parts of the scientific process. We once again urge caution in the areas of duplication and replicability.

In general, we are concerned that, instead of helping expedite health research, too many of the NIH-related provisions in the first draft are designed to assign responsibility so that in the future blame can be assigned. Instead, we would urge the committee to concentrate on the pipeline through which the drug discovery process travels, beginning with the critical basic, basic research and continuing on through to translational research.

The ASCB and its members stand ready to work with the committee in whatever way possible to improve the NIH portions of the legislation.

Sincerely,



Stefano Bertuzzi, Ph.D., M.P.H.
Executive Director
American Society for Cell Biology



**Association of
American Medical Colleges**
655 K Street, N.W., Suite 100, Washington, D.C. 20001-2399
T 202 828 0400 F 202 828 1125
www.aamc.org

March 18, 2015

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

The Honorable Diana DeGette
United States House of
Representatives
Washington, DC 20515

Dear Chairman Upton and Representative DeGette:

The Association of American Medical Colleges (AAMC) is pleased to provide our preliminary thoughts on the 21st Century Cures discussion document released on January 27. 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 thanks and commends you and the Committee for convening the extensive series of hearings and roundtables, both in Washington and across the country, to explore the opportunities for and obstacles to accelerating the pace of discovery and translating this knowledge into novel therapeutics and prevention strategies for the benefit of all Americans. As you heard from the representatives of academic medicine, patient groups, industry, and the federal agencies who participated in the roundtables and hearings, this is a time of unprecedented opportunity to employ the fruits of scientific discovery to transform health care both in the United States and globally.

We recognize that the discussion draft reflects the Committee's initial attempts to address a wide range of research-related issues that emerged during the hearings and roundtables, and we applaud the transparent and inclusive approach to this process. We are concerned, however, that the lack of a unifying vision for re-energizing the nation's medical research enterprise weakens this document. Instead, the draft presents a collection of ideas and proposals that address perceived deficiencies of varying magnitude. This piecemeal approach is at odds

with the stated needs for addressing the research enterprise as a whole and for a more strategic approach to research funding and oversight.

As the next draft is developed, we urge the Committee to ensure that:

- 1) the bill presents a comprehensive vision for the funding and regulation of medical research and is internally consistent;**
- 2) any revised oversight or regulation of research serves to facilitate the research enterprise, not tie the hands of the agencies, institutions, or researchers;**
- 3) current ongoing efforts to improve and harmonize the regulatory environment for research are encouraged and supported, not hampered;**
- 4) federal agencies working to realize the vision of 21st Century Cures are provided with sufficient funding to accomplish their goals, appropriated in a predictable and timely manner that allows for strategic planning by the agencies, institutions, and researchers;**
- 5) patients are more engaged in all aspects of the biomedical research enterprise; and**
- 6) federal policies enhance the preparation of the 21st Century biomedical research workforce.**

In this spirit, we hope the following preliminary observations are useful to you as the Committee works to revise and update the current discussion draft.

Legislative and regulatory provisions governing medical research should facilitate a 21st Century research enterprise, not hinder scientific progress or duplicate current efforts

The AAMC wholeheartedly agrees that planning, oversight, and accountability are necessary, particularly in dealing with the fiscal constraints of the past decade, but must be done in a way to incentivize innovation, not stifle scientific serendipity. Section 4001 requires the NIH to issue “a “5-year biomedical research strategic investment plan” to make funding allocation decisions, including strategic investment for each institute; have a common format; and identify strategic focus areas.

The AAMC is unconvinced that an overarching NIH strategic plan will enhance fiscal or scientific efficiency, transparency, or accountability sufficiently to merit the considerable time, effort, and resources NIH and the community would need to devote. Currently, each NIH Institute and Center produces its own 5-year strategic plan, based on extensive input from the scientific and patient communities, the groups best suited to identify and prioritize emerging scientific opportunities and compelling health needs. Because Congress appropriates annual funding to each Institute and Center, it is incumbent on each Institute and Center to identify visionary, but attainable, goals and make strategic investments to achieve these objectives. Furthermore, the Institutes and Centers vary significantly in terms of

the health needs they must address, the state of science in their relevant areas, and the range of funding mechanisms used to support their scientific mission, and the variability in individual plans appropriately reflects these differing factors.

Moreover, NIH already engages in extensive trans-institute planning, as demonstrated by its commitment to activities such as the BRAIN Initiative and the administration's initiatives on precision medicine, antimicrobial resistance, and Alzheimer's disease; this planning reflects a balance between emergent priorities and longer-term strategic objectives.

Among broader concerns about the proposed approach, the AAMC specifically objects to the discussion draft's provision 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. While the AAMC agrees with the critical importance of the NIH's mission in the support of basic research, we believe that mandating in statute a specific percentage of funding to any type of research is counter-productive and unnecessarily limits NIH's ability to respond to emerging scientific opportunities or health needs.

The draft's provisions regarding federal funding of research by NIH demonstrate the document's fragmentary approach. In some cases, the draft's proposals are internally inconsistent. For example, section 4005 of the draft calls for the Government Accountability Office (GAO) to conduct a study on NIH's Common Fund, including an analysis of how the funds "have been used and the impact of that funding on each of the areas that received funding." On the very next page, section 4007 proposes to authorize additional money for the Common Fund.

The bill should mitigate regulatory burden on researchers and institutions, rather than increasing burden through potentially duplicative provisions or efforts.

As noted by NIH Director Francis Collins, M.D., Ph.D., and various representatives from academic medicine, the regulatory burdens that are imposed on institutions and faculty continue to grow, and in many cases different agencies have different regulations on the same issue. A March 2014 report from the National Science Board on *Reducing Investigators' Administrative Workload for Federally Funded Research* stated, "The past two decades have witnessed increasing recognition that the administrative workload placed on federally funded researchers at U.S. institutions is interfering with the conduct of science in a form and to an extent substantially out of proportion to the well-justified need to ensure accountability, transparency and safety." The report also noted, "Failure to address these issues has resulted in wasted Federal research dollars. At a time of fiscal challenges and with low funding rates at many Federal agencies, it is imperative that these issues

are addressed so that researchers can refocus their efforts on scientific discovery and translation.”

The AAMC notes that in 2013, Congress charged the National Academy of Sciences (NAS) with conducting “a study on the impacts of Federal regulations and reporting requirements on institutions of higher education” (Senate Report 113-71 to accompany the FY 2014 Labor HHS Appropriation), and a designated committee was appointed to carry out this charge. The AAMC has already provided the NAS committee with information about the high cost and burden of certain regulations, including the Public Health Service regulations on financial conflicts of interest in federally funded research. The results of AAMC’s research indicate that the time and resources institutions and faculty must devote to keeping up with and maintaining compliance with such regulations is a growing burden without demonstrated value added. The AAMC urges the Committee to use the upcoming results of the NAS committee’s work to better frame any regulatory changes and to adapt the framework they suggest for addressing regulatory burden.

With regard to the proposal for clinical trials modification (sections 3001-2), the AAMC has long supported efforts to provide all human subjects with consistent and adequate protections. For example, the AAMC is working with the NIH and the research community to ensure a single Institutional Review Board (IRB) of record that ensures the protection of human research subjects while streamlining regulatory requirements and decreasing unnecessary burden on the institutions and investigators. In addition, the long-awaited proposed revision to the “Common Rule” on the oversight of federally funded research with human subjects has been drafted and is at the Office of Management and Budget (OMB) awaiting regulatory review. Given these productive efforts, we support a legislative approach that facilitates the harmonization of requirements through collaborative efforts; we worry that legislation that requires a specific approach is unnecessary and could hamper rather than encourage these ongoing efforts.

The AAMC welcomes the Committee’s interest in removing unnecessary restrictions on activities that facilitate research. The AAMC urges lifting or easing the restrictions on travel by federal employees to scientific meetings, which are essential to help build and maintain the connections within and across disciplines that do help drive research innovation. We appreciate that the discussion draft appears to recognize this need, and we look forward to reviewing the text when section 4003 is updated.

The AAMC commends the Committee’s inclusion of language in section 2221 to amend the HITECH Act to remove many of the current barriers imposed by the Health Insurance Portability and Accountability Act (HIPAA) for conducting research. The most beneficial proposed changes would maintain HIPAA’s privacy

protections, but would also: allow using health care data to be considered health care operations; let 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 allow an individual to authorize future research (currently prohibited). All of the proposed revisions would be beneficial and remove barriers to research that have no potential of protecting or benefitting patients or research subjects.

Scientific Progress Requires Sustained, Predictable Funding Growth

The AAMC is disappointed that the current draft does not include authorization levels for NIH that reflect the unprecedented scientific opportunities and pressing health needs. If we are to achieve the full potential of advances in areas such as precision medicine, neuroscience, digital health technologies, and the other emerging opportunities discussed by the Committee, it will require sustained, predictable real growth in the budget for National Institutes of Health (NIH). As you know, the NIH budget has lost nearly 25 percent of its purchasing power after adjusting for inflation since 2003.

As NIH Director Collins noted during the initial roundtable discussion last May, “Certainly from NIH’s perspective what we most desperately need in order to continue what has been the most successful story on biomedical research that the world has ever seen is a steady, predictable trajectory of support.”

The AAMC urges Congress and the Administration to work together to support sustained predictable real growth in the NIH budget. In particular, the AAMC supports the recommendation of the Ad Hoc Group for Medical Research that Congress provide at least \$32 billion for NIH in FY 2016.

In addition, while it is beyond the purview of this document and jurisdiction of the Committee, the failure to complete the annual appropriations process in a timely fashion unnecessarily impedes both planning and administering the research enterprise, both for NIH and for the institutions and scientists supported by federal funding. However, the Committee could mitigate the impact of this shortened timeframe for NIH decision making by granting NIH multi-year budget authority. Allowing NIH to carry over funding into the next fiscal year would enable more strategic management of grant funding, particularly in years when appropriations are not finalized until late in the fiscal year.

Patients should be more fully engaged in all aspects of the biomedical research enterprise

The AAMC agrees with the critical need to engage patients more fully in all aspects of the research enterprise. For example, the Committee is working on a proposal

[Title I, Subtitle H] to clarify and rationalize the rules to facilitate the responsible communication of scientific and medical developments. While the current rules are confusing and could use some clarification, the AAMC strongly encourages the Committee to develop a process that involves patients and physicians and other health providers in the formulation of these new rules, and to ensure that the new framework emphasizes the communication of evidence-based information.

The AAMC applauds and supports efforts to address the availability of educational information regarding medical products and to ensure the equitable diffusion of such information. The convening of an internal, agency-wide working group to strategize around traditional and electronic communication efforts and to identify subpopulations of import is an essential first step in ensuring equitable access to medical information and safety alerts. Additionally, the specific opportunities identified by the working group, including targeted outreach to traditionally underserved subpopulations and increasing their representation in the Food and Drug Administration (FDA) Patient Network, addressing the needs of Limited English Proficiency (LEP) populations, and leveraging the communication power of social media are all promising strategies.

We encourage federal agencies to work with hospitals, medical centers and electronic health record (EHR) developers to explore the possibility of enhancing or testing the use of automatic prompts via EHRs to alert providers, and therefore patients, to important safety and medical product information at the point of care. This or a similar strategy would assure the broadest possible dissemination of crucial information via practitioners well suited to interpret and deliver medical product alerts and updates.

Federal policies should enhance the preparation of the 21st Century biomedical research workforce

The AAMC thanks the Committee for recognizing the importance of early career scientists, and encourages Congress to keep in mind the complexity of the continuum of activities necessary to educate and train the next generation of biomedical scientists. The AAMC has been working with the NIH and other federal agencies on issues related to the biomedical research workforce, and we urge Congress to afford the agencies with the necessary flexibility to modify existing and add new training programs to meet the evolving needs of the 21st Century biomedical research workforce.

For example, the AAMC believes that NIH and other federal agencies are on the right track to recognize that a broad diversity of careers in academia, industry, and other sectors is a legitimate, valuable outcome of agency training and career development programs. AAMC also supports NIH's efforts to build a diverse research workforce. NIH's efforts to collect more data on the biomedical workforce needs will inform efforts to better understand the careers that trainees are

entering, align training with those needs, and educate trainees about these career options.

The challenge is to accelerate training and transition of these trainees to fully functional careers in science. NIH has developed several programs to help this career development and to recognize outstanding research by early career scientists. However, age at time of first award is not alone a determinative measure for how well the research system engages new scientists; increasingly, scientists train to work in teams and in collaborations on cross-disciplinary research. Training programs with team-based focus encourage interdisciplinary training and collaborations, which are necessary for the science of today and the future. The ages of “principal” investigators become less pertinent in multi-faceted team environments. Yet, other efforts are needed to continue to catalyze career transitions. Congress should allow federal agencies to continue monitoring these efforts without mandating specific data reporting. Research program leaders and their institutions are focusing strategically on how best to invest in and sustain research and research training programs, and to ensure that we are preparing the workforce for the needs of society - as a partner to federal funding agencies, private organizations, and industry in such investments.

Again, the AAMC thanks you and the Committee for the dedicated and diligent efforts to date 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.

Sincerely,



Ann Bonham, Ph.D.
Chief Scientific Officer



July 21, 2014

Chairman Fred Upton
2125 Rayburn House Office Building
Washington, DC 20515

Ranking Member Henry Waxman
2322A Rayburn House Office Building
Washington, DC 20515

Dear Chairman Upton and Ranking Member Waxman,

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 Committee's comprehensive approach to identifying methods to accelerate the pace of curing diseases in America. ASTRO is working to improve cancer care and pinpoint practices that bring us closer to a cure for cancer, including providing funding for radiation oncology research, incident learning systems and practice accreditation.

Radiation oncology research funding

ASTRO commends Congress for demonstrating an understanding of the importance of sufficient and reliable funding for the National Institutes of Health (NIH). Radiation oncology is a crucial part of cancer treatment and a focus of NIH's research programs. As a part of Congress' oversight duties and to ensure that funding levels are appropriate, it is vital for Congress to know precisely how NIH research funds are allocated. Therefore, we urge the Committee to get a clearer understanding of NIH's funding of research projects related to radiation oncology and ultimately gain more insight into NIH's priorities.

Major advances in cancer diagnosis and treatment, including radiation oncology, are happening at a faster pace than ever. As you know, Congress has demonstrated longstanding support for NIH and cancer research, and we are committed to accelerating recent advances. Our hope is that by fulfilling this request, Congress can have a better understanding of which types of research are being funded by NIH. 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 on radiation oncology research. With more than two-thirds 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 urge you to explore this disparity in funding. With federal funding diminishing, particularly in radiation oncology, promising young researchers are leaving the field.

Each year, ASTRO awards nearly \$1 million to fund research as part of the organization's overall effort to prevent, treat, and cure cancer. Specifically, ASTRO-supported research awards and grants supporting

work in radiation and cancer biology, radiation physics, comparative effectiveness research, translational research and outcomes/health services research. While this is a significant part of our budget, we cannot make up for needed federal funding.

Ensuring patient safety

In June 2014, ASTRO launched RO-ILS: Radiation Oncology Incident Learning System, a new, national patient safety initiative to facilitate safer and higher quality radiation oncology care. RO-ILS allows radiation oncology centers to provide non-patient-specific data about near-misses and safety incidents that have occurred at their facilities in a secure, non-punitive environment as outlined in the Patient Safety and Quality Improvement Act of 2005. The data collected in RO-ILS will educate the radiation oncology community about how to improve safety and patient care. This data will be analyzed to inform radiation oncology safety procedures and processes, best practices, practice guidelines and/or recommendations. RO-ILS is a key milestone in ASTRO's Target Safety Campaign, a patient protection plan to improve safety for radiation oncology. Learning from near-misses and safety incidents is a critical piece to improving patient care.

ASTRO is committed to ensuring that patients receive the best possible care by encouraging radiation oncology practices to report incidents so that we can learn from errors and improve processes of care, identify education gaps and develop needed clinical guidelines for the field. To guarantee that there is accountability in radiation therapy practices, ASTRO will launch the Accreditation Program for Excellence or APEx in early 2015. This program will hold practices accountable to meet a broad range of practice standards and highlight any variances in the delivery of radiation oncology care. We urge the Committee to investigate how to incentivize the use of such incident learning systems and practice accreditation programs to ensure that patients receive safe, high-quality care.

Thank you in advance for your work on behalf of the health of Americans. Please feel free to contact Shandi Barney at 703-839-7382 if you have any questions.

Sincerely,



Laura I. Thevenot
Chief Executive Officer



The Honorable Fred Upton
House Energy & Commerce Committee
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:

On behalf of Biogen, I would like to applaud you for your leadership on the 21st Century Cures Initiative. As one of the world's leading biotechnology companies, Biogen develops medicines that change the lives of people living with neurodegenerative diseases, hematologic conditions and autoimmune disorders. We share your goal of advancing science and accelerating the pace of cures for patients.

We appreciate the opportunity to comment on the Discussion Draft that was released by the Energy & Commerce Committee. Attached is our response to proposals of importance to Biogen.

Comments on the 21st Century Cures Discussion Draft

Title I – Putting Patients First by Incorporating their Perspectives into the Regulatory Process and Addressing Unmet Needs

Subtitle A – Patient Focused Drug Development

We support the discussion draft language that builds off the current Patient-Focused Drug Development program at FDA and seeks to establish a framework for the meaningful consideration of patient experiences and preferences in the regulatory process.

Biogen believes that understanding patient perspectives and needs and incorporating those perspectives and needs into our research and development decisions leads to more meaningful treatments, better outcomes, and improved quality of life for patients. A formalized Patient Focused Drug Development program provides a mechanism for the FDA to better understand patient experiences and preferences and to incorporate this understanding in regulatory decision making.

Recommendations:

- Clarify that Sponsor outreach to patient groups for the purpose of better understanding their perspectives on the design and conduct of a particular clinical development program does not constitute promotion or marketing of an unapproved investigational product or indication subject to enforcement. Also require FDA guidance describing the appropriate parameters and regulatory/legal safe-harbor for Sponsor engagement with patient groups during drug development.

- Add the following underlined text to the definition of ‘patient experience data’ [Section 1001 – pg. 10, line 18]
 - In this subsection, the term ‘patient experience data’ means data collected by patients, parents, caregivers, patient advocacy organizations, disease research foundations, biopharmaceutical companies, or medical researchers that is intended to provide information about the experience and preferences of patients with a disease, patients’ beliefs with respect to such benefits and risks in the management of the patient’s disease; or...
 - This change: (1) incorporates data collected by biopharmaceutical companies in this process, and (2) uses existing language from pg 12, line 19-22 to broaden the narrow definition of ‘patient experience data’, clarifying that the definition is not intended to be just a reciting of a patient’s medical experience, but to capture the collection of patient preferences as well.
- Add ‘representatives of biopharmaceutical companies’ under the definition of ‘attendees’ for ‘workshops’ that are designed to obtain input regarding methodologies for patient-reported outcomes [Section 1001 – pg. 13 line 20]
 - Representatives of biopharmaceutical companies would share a valuable perspective in all public workshops, methods development, and data collections processes, both because biopharmaceutical companies are the primary collectors of patient data during clinical trials and because they would ultimately be required to gather and apply the patient-experience data – developed under this section – to future trials. Efforts to expand the Patient Focused Drug Development Initiative should engage industry.

Subtitle B – Surrogate Endpoint Qualification and Utilization

We support the discussion draft language that would establish a predictable, transparent process for FDA’s consideration, and qualification, of surrogate endpoints and the recognition that there may be a need for qualification of biomarkers for use other than as surrogate endpoints. In fact, industry has long supported efforts to expand FDA’s acceptance of surrogate endpoints and other tools as evidenced by industry funding of these efforts in PDUFA V.¹

Recommendations:

- Expand the scope of this provision to include all Drug Development Tools (DDTs) by replacing ‘surrogate endpoint’ with ‘drug development tool’ in this section. Define DDTs as: ‘methods, materials, or measures that aid drug development and include but are not limited to biomarkers, surrogate endpoints, clinical outcome assessments, and animal models.’
- We support engaging the broader scientific and health care community, through public-private partnerships or otherwise, to develop evidentiary standards for the use of DDTs in order to assist the FDA with this undertaking and fill its resource gaps. We believe industry and academic institutions, in particular, would be valuable contributors to the

¹ <http://www.fda.gov/downloads/ForIndustry/UserFees/PrescriptionDrugUserFee/UCM270412.pdf>, page22

process because they have specialized expertise and a strong interest in advancing this science. In addition, establishing partnerships could lift resource burdens on the agency.

- Overall, a key issue with the current drug development tools (DDT) qualification process is that no timelines are associated with each stage of the review. This represents a significant challenge, because sponsors are unable to ensure that a qualification process can be completed in a timely matter to support, for example, inclusion of a clinical outcome measure as a primary endpoint for a pivotal study. We do not believe the draft discussion language is sufficient to spur FDA to advance the full spectrum of DDTs. Therefore, we support the development and implementation of predictable, evidenced-based and timely processes for the qualification of *all* DDTs, including biomarkers and patient-reported outcomes. We believe these tools will make the drug development process considerably more effective and efficient.

Subtitle H – Facilitating Responsible Communication of Scientific and Medical Developments

We support efforts to facilitate appropriate communication between drug companies and health care professions and look forward to reviewing this future proposal.

Recommendation: Consistent with FDASIA section 114, FDA should establish a mechanism to allow drug companies to communicate truthful and non-misleading information about approved uses and medically accepted alternative uses of approved products to healthcare professionals and payers.

Subtitle I – Modernizing the Regulation of Social Media

We support the discussion draft language that provides more certainty regarding communications about biopharmaceutical therapies on social media, particularly that which allows more detailed safety and efficacy information to be hyperlinked. We believe that this flexibility allows for effective communication with patients while still considering the need for appropriate patient safeguards in the ever-changing internet age.

Subtitle J – Streamlined Data Review

We support the discussion draft language that allows the FDA to accept and review data summaries rather than full data packages from companies requesting to add indications to a drug label.

Recommendation: Expand the definition of ‘qualified indication’ to ‘an indication for the detection, diagnosis, prevention, treatment or cure of any disease’ [pg 96, line 5] in order to include all diseases, not solely cancer.

Title II – Building the Foundation for 21st Century Medicine, Including Helping Young Scientists

Subtitle F – Building a 21st Century Data Sharing Framework

Part 2 – Improving Clinical Outcomes for Patients and Program Integrity Through CMS Data Sec. 2086. Empowering patient research and better outcomes through CMS data.

We support expanding the availability of Medicare data and ask that the Committee clarify the scope of the data sets to be made accessible. Such data would be helpful for research, product safety, and patient access for Biogen and the broader health care community.

Recommendation: We ask that the Committee include a provision that would ensure that sensitive and/or proprietary information is protected in the process of allowing broader access to these data.

Subtitle G – Utilize Real-World Evidence

We support the discussion draft language that would authorize the FDA to utilize real world evidence for regulatory purposes, particularly the use of evidence from observational studies and registries to expand indications and fulfill post-market requirements. Through partnerships with Universities and companies like Google, Biogen uses real world data to inform our research, and the company hopes to expand its capacity to use this data to advance science and help patients.

Subtitle H – Coverage With Evidence Development

We oppose codifying CED authority as CMS has a process in place to perform CED.

Title III – Modernizing Clinical Trials

Subtitle A – Clinical Research Modernization

We support the discussion draft language that streamlines the IRB process by eliminating the requirement that companies use local IRBs, thereby making it easier to use a centralized IRB to oversee clinical trials. We believe these measures will minimize regulatory duplication and unnecessary drug development delays.

Subtitle B – Broader Application of Bayesian Statistics and Adapted Trial Designs

We support the draft language that would establish and implement a framework that would allow companies to incorporate adaptive trial designs, Bayesian methods, or other alternative statistical methods into proposed clinical protocols. We believe that these options will shorten the time it takes to conduct clinical trials and perform subsequent data analysis.

Subtitle C – Postapproval Studies and Clinical Trials

We support the discussion draft language that would establish a process for the Secretary to periodically evaluate if a post-approval study or clinical trial is no longer scientifically warranted or if the design or timeline of the study or trial should be renegotiated. We believe that this language would help ensure that post-approval efforts are a fruitful use of time and resources.

Title IV – Accelerating the Discovery, Development, and Delivery Cycle and Continuing 21st Century Innovation at NIH, FDA, CDC and CMS

Subtitle B – Advancing Research for Neurological Diseases

We support the discussion draft language that directs the CDC to develop a surveillance system to track the epidemiology of neurological diseases. Biogen has four approved MS therapies and focuses on the treatment of neurological conditions, and we believe that having a repository of natural history data on neurological conditions would be beneficial to our researchers as well as the broader scientific community.

Subtitle H – Local and National Coverage Decision Reforms

We support requiring each Medicare administrative contractor to create a process for the development of Local Coverage Determinations (LCD) that includes public comment periods, meetings and disclosure of decisional information. We believe increasing transparency and opportunities for stakeholder feedback will improve the current LCD process.

Subtitle J – Revise IPPS New Technology Add-On Payment (NTAP) Reimbursement Amounts Sec. 4201(b):

We oppose the current proposal to replace Level II HCPCS codes for drugs and biologicals with NDC codes for the purposes of Medicare Part B coding.

Recommendation: We propose the Committee adopt BIO's proposal that would require CMS issue J-codes more frequently (i.e., at least quarterly) as this would address concerns around the current lag-time with issuing codes and patient access without requiring a complete overhaul of the current billing system which may cause further logistical and access challenges.

Thank you for your consideration, and we look forward to working with you on this important initiative.

Sincerely,

Kathleen W. Tregoning
Vice President
Public Policy & Government Affairs

CANCER LEADERSHIP COUNCIL

A PATIENT-CENTERED FORUM OF NATIONAL ADVOCACY ORGANIZATIONS
ADDRESSING PUBLIC POLICY ISSUES IN CANCER

March 27, 2015

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

The Honorable Diana DeGette
United States House of Representatives
Washington, DC 20515

Dear Chairman Upton and Representative DeGette:

The undersigned organizations represent cancer patients, physicians, pharmacists, researchers, and other health professionals who are engaged in efforts to improve cancer treatment and enhance the overall quality of cancer care. We appreciate the opportunity to comment on the January 2015 discussion draft, “21st Century Cures Act.”

Our comments will focus on the following objectives:

- Balancing the speed of regulatory review against an assurance that new cancer drugs are safe and effective;
- Preparing for review of precision medicine drugs;
- Ensuring that “patient-focused drug development efforts” are reflected in FDA programs and regulatory approaches;
- Encouraging the consideration of patient-reported outcomes data in the review process;
- Building data-collection and sharing efforts on a firm foundation of successful clinical trials data reporting;
- Defining the new roles of patient advocacy and patient research foundations in the therapeutic development process; and
- Ensuring that new commissions, panels, and reports serve the needs of patients, do not duplicate existing commissions and reporting requirements, and do not create unreasonable burdens for federal agencies.

Ensuring a Strong Regulatory Review Process

Cancer patients, physicians, and other health care providers have an interest in eliminating any inefficiencies in the regulatory review process and ensuring patients access to safe and effective drugs at the earliest possible time. However, we want to be sure that those drugs that are approved by the Food and Drug Administration (FDA) are safe and effective and will provide clinical benefit to patients.

Cancer patients and their health care teams have benefited greatly from the efforts of the Office of Hematology and Oncology Products to improve the cancer drug review process and expedite the review of cancer drugs whenever possible. The Office, within the Center for Drug Evaluation and Research (CDER), has made aggressive but appropriate use of the expedited programs for serious conditions, as defined by the Guidance for Industry dated May 2014. These expedited programs include fast track designation, breakthrough therapy designation, accelerated approval, and priority review designation.

The 2014 review record of the Office of Hematology and Oncology Products is impressive. Drugs were approved for treatment of advanced ovarian cancer, acute lymphoblastic leukemia, three types of blood cancer, metastatic non-small cell lung cancer, and melanoma. This drug review and approval record was accomplished through use of the expedited development and review pathways; Prescription Drug User Fee Act (PDUFA) goals were met in almost all 2014 drug reviews and most drugs were approved on the first review cycle.

In light of the record of Office of Hematology and Oncology Products, we offer cautions about two proposals that are included in the draft. First, we are not persuaded that confirmatory trial requirements should be eliminated for those drugs that are subject to accelerated approval. Those requirements should remain in place for those drugs approved on the basis of surrogate endpoints. Second, we are concerned about a suggestion that supplemental approvals might be based on summaries of data, without a requirement of submissions of the underlying data. FDA has a proven track record for efficient review of cancer drugs, and changing the amount of data necessary for an application is neither necessary nor advisable.

Instead of eliminating post-approval study requirements or changing data requirements for approval, we encourage evaluation and replication of the work of the Office of Hematology and Oncology Products. That effort will identify effective ways to utilize current expedited review mechanisms.

Preparing for Review of Precision Medicine Drugs

Although we are pleased with the performance of the cancer review office to date and applaud the willingness of the office staff to collaborate with patient advocacy groups and professional societies on issues ranging from clinical trial design to identification of surrogate endpoints, we see significant challenges for the office and for all of FDA in the future.

As we move more completely into the age of precision medicine, the office will need assurance that all personnel possess the skills for review of targeted therapies. In addition, FDA reviewers need more flexibility to attend and participate in scientific and medical meetings. These meetings are an opportunity for continuing medical education and for staying current on developments related to precision medicines, and these opportunities should be available to review staff.

We note that the committee has left in its discussion draft a “placeholder” for FDA personnel issues. We urge that this placeholder be replaced by revisions to FDA authority that will streamline hiring processes. In addition, travel and ethics rules should be addressed – if necessary, in legislative language – to guarantee FDA staff the ability to attend important meetings in their field.

Patient-Focused Drug Development Efforts

The Food and Drug Administration Safety and Innovation Act (FDASIA) included a number of important patient-focused drug development efforts. The patient-focused drug development meetings have been of special interest to patient advocates. We appreciate that the agency recognizes the importance of involving patients in drug development issues consistent with FDASIA requirements. Although we are concerned about adding responsibilities to the portfolios of review teams, which should be primarily focused on new product review, we would like to see more engagement of reviewers in the planning and execution of the patient-focused drug development meetings. This is the most efficient means of ensuring that the patient-focused meetings undertaken by the agency are integrated into the operations and inform the thinking of the agency.

Patient-Reported Outcomes in the Regulatory Review Process

The initial section of the discussion draft encourages the use of patient experience data to inform the risk-benefit assessment. We are pleased that the draft seems to encourage serious consideration of patient-reported outcomes in the regulatory process, but we recommend more specific definitions be included in this section of the bill. If patient-reported outcome data are to be utilized in a data-driven regulatory process, the standards for those data must be well-defined. It will not benefit patients if the agency is encouraged to consider patient anecdotes that do not meet reasonable data standards.

The committee should consider setting goals for approval of patient-reported outcome tools by the agency and encouraging reference by the agency to the information provided through those validated tools.

Building Successful Data-Collection and Data-Sharing Initiatives

We are strong supporters of a movement toward “big data” collection and sharing to fuel strong cancer drug development and clinical care improvement. In fact, a number of our organizations have developed data registries that track the treatment and outcomes of our patients. We urge that any federal involvement in data collection and sharing efforts be built on a strong foundation. To that end, we encourage that recent findings of limited compliance with the

reporting requirements of www.clinicaltrials.gov be considered by the committee. These findings should inform efforts to strengthen clinical trials reporting. In addition, a stronger trials results reporting system might serve as a foundation for other data collection efforts.

Defining the Roles and Responsibilities of New Commissions and Panels

A review of the discussion draft raises some concerns related to the number of new commissions, consortia, and reporting requirements that are authorized. Our reservations are two. First, we are concerned that some of the new research and regulatory efforts and initiatives may be redundant of existing research and regulatory programs. For example, has the National Center for Advancing Translational Sciences been evaluated to determine if parallel clinical research programs are necessary? Has the regulatory science collaboration between the National Institutes of Health (NIH) and FDA been reviewed? What are the results of the Critical Path Initiative?

Second, we are concerned that the new consortia, commissions, and reports will be accompanied by significant costs that cannot easily be absorbed by NIH and FDA and that additional resources for these responsibilities will not be available.

Understanding the Roles of Nonprofit Research Foundations

If the 21st Century Cures Consortium and the Medical Products Innovation Advisory Commission are retained after the committee considers any possible overlap with existing programs and the cost associated with new commissions, we recommend that membership of both groups be redefined to include more members drawn from patient advocacy organizations and robust representation from non-profit, patient-driven research foundations. The Cures Consortium would number 22 members, including 8 representatives of the biopharmaceutical and medical device industries and 9 who shall be “representatives of academic researchers, patients, health care providers, and health care plans and insurers, to be appointed by the Comptroller General of the United States, after soliciting nominations.” The Medical Products Innovation Advisory Commission would include 17 members, and the discussion draft does not indicate that any will be patient advocates or representatives of non-profit research foundations.

We believe that the membership categories for both of these panels should be redrafted to ensure strong representation of patient advocates and inclusion of individuals from non-profit research foundations. Patients can speak to unmet medical needs, and those representing research foundations may also bring extensive experience and expertise about research and development of new treatments. Over the last decade, there has been nothing short of a revolution in the operation of patient-driven research foundations. These groups have refined the manner in which they invest their resources, expanding beyond investigator-initiated grants to therapy development programs. In addition, many of them have been innovators in clinical trial design and recruitment and are pioneering data collection and sharing efforts. Their expertise must be reflected in the deliberations of these commissions, and that can be accomplished by guaranteeing robust membership from their ranks.

Ensuring Access to New Therapies

We note that the discussion draft focuses primarily on the development and regulatory review of new therapies, and we have confined our comments to those topics. However, it is critical that cancer care delivery systems ensure patients access to the treatments of the 21st century. We are actively involved in payment and delivery reform efforts that will ensure access to quality, affordable, and sustainable cancer care.

Thank you for the opportunity to participate in the process of developing legislation to encourage development of new treatments for the new century.

Sincerely,

Cancer Leadership Council

Association for Molecular Pathology
CancerCare
Cancer Support Community
Fight Colorectal Cancer
Hematology/Oncology Pharmacy Association
International Myeloma Foundation
Kidney Cancer Association
The Leukemia & Lymphoma Society
LIVESTRONG Foundation
Lymphoma Research Foundation
Multiple Myeloma Research Foundation
National Coalition for Cancer Survivorship
National Patient Advocate Foundation
Ovarian Cancer National Alliance
Pancreatic Cancer Action Network
Prevent Cancer Foundation
Sarcoma Foundation of America
Us TOO International Prostate Cancer Education and Support Network



March 27, 2015

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

The Honorable Frank Pallone, Jr
U.S. House of Representatives
Ranking Member, Energy & Commerce
Committee
2322A Rayburn House Office Building
Washington, DC 20515

Dear Chairman Upton and Ranking Member Pallone,

The Coalition for Clinical and Translational Science (CCTS) thanks you and your colleagues for your ongoing efforts to advance medical research and improve patient care through the *21st Century Cures Initiative*. We appreciate this opportunity to provide general feedback on the initial discussion draft and we hope to engage the Committee in a constructive dialogue with more nuanced recommendations as specific provisions begin to crystalize.

As the unified voice of the clinical and translational medical research community, the advocacy priorities of CCTS align with the legislative and policy goals of the *21st Century Cures Initiative*. Particularly, our interest in assuring that the full spectrum of federal medical research activities is supported to ensure breakthroughs in basic science are translated into innovative therapies and diagnostic tools falls in line with the Initiative's focus on the *discovery, development, delivery* cycle. We also place a strong emphasis on adequate support for training activities and opportunities for young investigators to ensure this nation maintains a robust career development pipeline for the next generation of medical researchers.

We are pleased to see the *21st Century Cures* discussion draft acknowledges many of the systemic barriers to meaningful and timely progress commonly associated with the research valley of death and ongoing attrition in the research training and career development pipeline. In the interest of continuing to advance this important effort, we would like to lend our perspective on critical overarching items that need to be addressed as the initiative moves forward.

At this time, CCTS concerns with the *21st Century Cures* draft fall into two primary areas:

- 1) Adequate annual funding for federal agencies, particularly the National Institutes of Health (NIH), which currently have extremely limited resources and would need to take on new programs and activities.
- 2) The questionable merits, unintended outcomes, and unanswered questions associated with some of the provisions related to medical research.

Funding Concerns

Recognizing that federal investment in biomedical and healthcare research has fallen increasingly behind previous inflation-adjusted funding levels, the substantial mandates of this draft bill, without substantial new funding, would take away from already insufficient funds supporting current efforts, which would have the unintended consequence of undermining rather than facilitating research and medical progress.

As a result of the leveling of its annual budget coupled with the specter of sequestration, NIH has already become extremely efficient with available resources. In recent years, pay lines have suffered greatly and many meritorious research projects have gone unfunded despite receiving exceptional peer-review scores. The current financial situation is directly tied to the loss of young investigators in the medical research field. As talented young researchers gain an appreciation of just how difficult it is to secure federal funding for research projects, they inevitably entertain alternative career opportunities. Still others opt out of the field altogether after weighing the significant educational commitment against the low probability of submitting a NIH grant application that ultimately receives funding.

There is tremendous promise offered through the *21st Century Cures Initiative*. Please consider this vehicle an opportunity to authorize a new funding paradigm or supplemental funding mechanisms for federal medical research programs. Uncertainty in the annual budget process has consistently undermined critical research projects. We hope this effort can advance a strategy to provide stable, predictable, and meaningful funding increases that move away from the current process of annual discretionary resources. Further, NIH pay lines are presently around 12% and any new effort should be ambitious enough in its scope and support to ensure that pay lines of around 30% can be maintained.

Provisions of Concern

We are supportive of many provisions in the discussion draft as they are potentially beneficial to patient care and the full spectrum of medical research. However, some provisions appear deleterious and notable changes and additional information will be required moving forward.

- CCTS echoes the concern regarding patient safety that the outgoing Food and Drug Administration (FDA) Commissioner, Margaret Hamburg, raised during her recent testimony before the Senate Health, Education, Labor, and Pensions Committee. Asking the FDA to accept “real world evidence” is particularly alarming and could significantly harm patients by placing regulatory speed ahead of overall safety. We would like clarification of this provision, but we are unlikely to support any language that would relax standards for evidence and could be adverse to patient safety.
- The National Center for Advancing Translational Sciences (NCATS) is leading NIIH efforts to build a clinical and translational research infrastructure at academic health centers across the country. NCATS primary mechanism for advancing this important effort is the Clinical and Translational Science Awards (CTSA) program, which the Institute of Medicine positively reviewed in 2013. The mandates for NCATS to have

flexibility in funding is particularly troubling as it could be interpreted more broadly than applying to just the Cures Acceleration Network. Such a broad interpretation could jeopardize financial support for CTSAs at a critical juncture when the program should be prioritized and expanded to help facilitate the goals and interests of the *21st Century Cures Initiative*.

- As members of the medical research community, we agree with the concerns previously raised by the Federated American Societies for Experimental Biology. Particularly, sections requesting more thorough review of grant funding and additional strategic planning at NIH Institutes and Centers would place an unnecessary administrative burden on researchers. Additional mandates on researchers would do little to improve oversight, but would have a significant negative impact on the ability to conduct research.
- Many clinical and translational researchers provide healthcare to patients and interface with the Medicare and Medicaid programs. CCTS is concerned that new provisions requiring payments by Medicare and Medicaid represent an undue intrusion into payment policies and could undermine important aspects of the nation's largest health insurance system.

We understand that the Committee is currently working to prepare a second discussion draft on *21st Century Cures* that would begin to narrow and focus the overall effort. We look forward to reviewing the upcoming bipartisan proposal and we hope to provide additional guidance and input moving forward. In the meantime, please consider us a resource on issues related to adequately supporting the full spectrum of medical research, including therapy and treatment development activities.

Sincerely,



Michael Lichtenstein, M.D., M.Sc
President
Association for Clinical and
Translational Science



Herb Pardes, MD
Co-Chair
Clinical Research Forum



Harry Selker, M.D., MSPH
Co-Chair
Clinical Research Forum



Coalition for Excellence in Medication Monitoring

March 16, 2015

Delivered by Electronic Submission

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

Re: Section 4161 of the 21st Century Cures Act Discussion Draft –Improvements in the Medicare Local Coverage Determination (LCD) Process

Dear Chairman Upton:

The Coalition for Excellence in Medication Monitoring (CEMM)¹ appreciates the opportunity to provide comments on the discussion draft of the 21st Century Cures Act released by the Committee on Energy and Commerce (the Committee) on January 27, 2015. CEMM is a coalition of the nation's leading medication monitoring clinical laboratories dedicated to providing high-quality laboratory tests as part of the standard of care for treatment of the millions of Americans, who suffer from debilitating chronic pain. Through laboratory-based tests, CEMM is working to ensure that physicians, health care professionals, and patients have the best available clinical data to make informed decisions about pain medication utilization and patient management.

Specifically, CEMM is submitting the following comments regarding Section 4161 of the discussion draft, a provision entitled "Improvements in the Medicare Local Coverage Determination (LCD) Process." We strongly support this provision and urge the Committee to strengthen the LCD process and protect opportunities for meaningful stakeholder involvement. As LCD determinations dictate coverage for a significant number of Medicare services, it is vital that the process be fair and inclusive to ensure that determinations are made based on the best available medical knowledge. We are concerned that the current LCD process has led to coverage determinations that curtail valuable stakeholder input, limit physicians' ability to make individualized medical treatment decisions, and harm patients' access to care.

Further, we urge Congress to act now to protect the integrity and transparency of the LCD process and prevent any Agency initiatives that would curtail the existing process. Recent activity at the Centers for Medicare and Medicaid Services (CMS) has indicated that the Agency

¹ CEMM members include Aegis Sciences Corporation, Alere, Ameritox Ltd., Calloway Laboratories, Dominion Diagnostics, and DRUGSCAN.

may consider reducing the opportunities for stakeholder involvement in the LCD process.² We strongly believe that the LCD process should be strengthened to provide for more opportunities for public engagement, not less. CEMM strongly opposed that CMS regulatory proposal, and we urge the Committee to act to statutorily protect and strengthen the LCD process.

I. SECTION 4161 IS NEEDED TO PROTECT STAKEHOLDER ENGAGEMENT AS AN ESSENTIAL RESOURCE IN LCD DEVELOPMENT, AND THERE ARE OPPORTUNITIES TO FURTHER STRENGTHEN THE PROVISION TO ENSURE MORE MEANINGFUL PUBLIC INVOLVEMENT IN THE PROCESS

Public engagement serves as a vital role in helping contractors develop clinically appropriate LCDs and gives stakeholders an opportunity to provide input during the development process. The current LCD process requires Medicare contractors to present draft LCDs to Carrier Advisory Committees (CACs), hold open meetings for other stakeholders to provide input, and allow stakeholders to submit written comments on proposed LCDs.³ The CAC meetings, open public meetings, and written comment period all provide distinct benefits to the MACs and lead to better quality LCDs that detail current clinical protocols with less need for frequent revisions and future appeals.

CEMM strongly supports Section 4161 for formalizing these requirements in statute. As past CMS proposals have considered restricting these processes, we salute the Committee for providing protection for this important part of the coverage determination process. We also urge the Committee to further strengthen these provisions to ensure that stakeholders have a genuine opportunity for substantive engagement.

a. Both the CAC and Open Public Meetings Serve a Vital Purpose, and Should Be Protected and Enhanced Through Statute

Both sets of in-person meetings are critically important to the LCD process, as they allow contractors the opportunity to actively engage in discussions with stakeholders and explore the perspectives of a variety of experts and impacted parties. CEMM strongly supports Section 4161 for making these requirements statutorily mandated.

The contractor's CAC or CACs are pre-arranged committees comprised of physician representatives (with no more than one per specialty or provider type), a beneficiary representative, and representatives of certain other medical organizations. CAC meetings are typically scheduled at certain intervals during the year, and meetings address a number of

² Medicare Program; Revisions to Payment Policies Under the Physician Fee Schedule, Clinical Laboratory Fee Schedule, Access to Identifiable Data for the Center for Medicare and Medicaid Innovation Models & Other Revisions to Part B for CY 2015 79 Fed. Reg. 40318 (July 11, 2014).

³ 79 Fed. Reg. at 40378-40379; Centers for Medicare and Medicaid Services, Medicare Program Integrity Manual, Chapter 13 – Local Coverage Determinations, Sec. 13.7.

proposed LCDs. These primarily physician-oriented meetings allow contractors to discuss proposed policy changes with state physicians and gain information about local standards of care and the potential clinical impacts of proposed LCDs.

Given CAC's restricted membership and heavy workload with limited time, open stakeholder meetings are also critical to ensuring that contractors have an opportunity to engage more substantively with additional stakeholders and gain insight from a variety of perspectives. Contractors must invite potentially impacted groups to participate in the meetings, which makes it more likely that the contractors can benefit from the input of a variety of stakeholders. These meetings provide a dynamic forum for multi-party conversations as well as question-and-answer discussions. Also, interested parties may make presentations of information related to draft policies. These open meetings provide an opportunity to educate the impacted community about upcoming changes in coverage policies.

Currently, contractors often schedule meetings with little notice to the impacted community, and the meetings are often held in remote locations, making them difficult to attend. The Committee should require contractors to make these meetings more accessible to stakeholders, such as requiring advanced notice and by requiring that there be opportunities for remote participation.

Further, it is important that clinicians who serve Medicare beneficiaries have the opportunity to relay appropriate clinical care decisions to contractors, as well as third-party stakeholders such as laboratories that serve a critical function in the clinical process. We ask that the Committee consider including additional stakeholders in the CAC process, as this will better inform contractors during the LCD development process.

b. In-Person Meetings Are Critical, as Written Comments Alone Would Not Serve the Purpose of Meaningful Public Engagement

CEMM strongly supports the Committee's efforts to formalize the LCD process in statute, as recent CMS proposals have considered limiting the opportunity for stakeholder involvement solely to submission of written comments.⁴ We believe that any such proposal to limit engagement to submission of written comments would be misguided, and Section 4161 is needed to protect the existing process.

While written comments are an important part of the LCD process, there should continue to be a requirement that MACs engage in in-person meetings with impacted parties. The CAC meeting and open stakeholder meeting serve separate and vital purposes that differentiate them from the comment period, which only allows stakeholders to submit written comments to the MACs. The advent of modern technology has not materially changed the nature of the written comment period, and stakeholders' opportunity for engagement through email submission remains much the same as when comments were submitted by hand-delivery. Written comments

⁴ 79 Fed. Reg. at 40378-40379.

do not allow for open discussions among multiple parties and do not provide an opportunity for dynamic give-and-take discussions.

While MACs have the opportunity to address follow-up questions to individual commenters, there is no requirement that they do so. Nor is there a requirement that they meet with stakeholders for follow-up discussions regarding written comments. In practice, contractors often fail to reply to specific stakeholder questions and refuse requests for meetings or conference calls. While contractors are required to prepare a written summary of comments and the reasoning behind the LCD, these documents are often cursory and do not provide meaningful perspective or an assurance that public comments have truly been considered.

Robust public discussions are a vital part of the LCD process, and there must be a meaningful dialogue following the release of a draft LCD. Limiting the opportunity for public engagement to merely a written comment period and a discretionary CAC meeting, as CMS has previously proposed, would significantly limit stakeholder engagement in the LCD process, increase the likelihood that resulting LCDs will be clinically inappropriate, and result in a rise of LCD reconsideration requests and appeals, which will ultimately slow down the LCD process. CEMM supports the Committee's efforts to protect the LCD process by formalizing it in statute.

II. SECTION 4161 SHOULD SPECIFY THAT LCDs CONTINUE TO BE REQUIRED TO HAVE A NOTICE PERIOD WITH AN EFFECTIVE DATE NO EARLIER THAN 90 DAYS AFTER PUBLICATION OF A FINAL LCD TO ALLOW PHYSICIANS AND OTHER STAKEHOLDERS SUFFICIENT TIME TO MODIFY CLINICAL PRACTICES AND ADJUST VARIOUS SYSTEMS

The current LCD process requires contractors to provide a 45-day notice period on all final LCDs, with effective dates no earlier than the 46th day after a final LCD is published.⁵ CMS has previously proposed curtailing this notice period and allowing LCDs to go into effect more rapidly.⁶ This notice period is critically important to physicians and to impacted health care companies, as final LCDs often necessitate changes in clinical protocols, documentation requirements, and business practices. Further, impacted companies need time to implement changes to their billing and other information systems, including time to conducting testing prior to implementation to ensure that their claims submission process is accurate and compliant with the new requirements included in any final LCD. Frequently, Final LCDs can have substantial difference from earlier draft LCDs, and providers and other stakeholders need time to implement those changes. We urge the Committee to further strengthen Section 4161 by explicitly requiring that all LCDs have a minimum implementation period of 90 days.

⁵ Centers for Medicare and Medicaid Services, Medicare Program Integrity Manual, Chapter 13 – Local Coverage Determinations, Sec. 13.7.4.3.

⁶ 79 Fed. Reg. at 40378-40379.

Final LCDs often differ substantially from the proposed draft LCDs, so stakeholders do not know the details of all the requirements until the final LCD is published. They must then act quickly to make necessary adjustments and educate other impacted parties about the changes within the 45-day notice period. Impacted parties may need to develop, order, and disseminate new paperwork, educate physicians about changes, and adjust billing protocols. Physicians may need to establish new internal clinical routines and educate patients about the changes.

The notice period is particularly crucial in providing time to educate physicians about the changes. It can also be challenging for physicians with busy practices to monitor all LCD activity, identify relevant changes, and make necessary changes to their clinical practice. It is particularly difficult for physicians in *small practices* and in *rural areas* to promptly learn of all relevant changes to coverage policies.

The 45-day notice period is already too short of a window to ensure that all impacted parties are aware of the changes and can make all necessary adjustments. Any further limitation on the notice period requirement would simply be impracticable for the majority of LCDs and would make it nearly impossible for physicians and companies to be in full compliance from day one. CEMM urges the Committee to include a 90-day notice period before LCD implementation as part of Section 4161.

III. WE APPLAUD SECTION 4161 FOR REQUIRING MACS TO COMPLETE THE FULL LCD PROCESS BEFORE ADOPTING A COVERAGE DECISION FROM ANOTHER JURISDICTION

CEMM strongly supports the Committee's decision to include a requirement that MACs complete the full process required for all new LCDs before adopting an LCD that has been established in another jurisdiction. Particularly in the clinical laboratory testing space, there has been heightened concern that various MACs may simply adopt LCDs identical to those developed in another jurisdiction with a limited process that lacks the required opportunities for public input. Such a practice would create a "de-facto" National Coverage Determination (NCD) without having been subject to the rigorous scientific and medical review process required for an NCD.

Further, LCDs are intended to account for local variation in clinical practice, community needs and standards of care. If a MAC adopts another jurisdiction's LCD without being subject to the full process for LCD development, it is unable to receive this critical input specific to its locality. We strongly support this provision in Section 4161 and urge the Committee to retain it in any future drafts.

IV. CONCLUSION

CEMM applauds the Committee's leadership and appreciates this opportunity to comment on these important provisions of the 21st Century Cures Act discussion draft legislation. We strongly believe that a fair and open LCD process is critical to ensuring that

*Comment Letter Regarding the 21st Century Cures Act Discussion Draft
Submitted by the Coalition For Excellence in Medication Monitoring (CEMM)
March 16, 2015*

Medicare beneficiaries have access to the care they require and that physicians are free to make clinical decisions about the needs of their patients. CEMM's members would welcome the opportunity to meet with you to discuss these matters further. If you have additional questions, please feel free to contact Caitlin McCormick with Akin Gump at 202-887-4208 or cmccormick@akingump.com.

Thank you,

Coalition for Excellence in Medication Monitoring

On behalf of
Aegis Sciences Corporation
Alere
Ameritox Ltd.
Calloway Laboratories
Dominion Diagnostics
DRUGSCAN

cc: Representative Brett Guthrie



April 9, 2015

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

The Honorable Diana DeGette
U.S. House of Representatives
Energy & Commerce Committee
2322A Rayburn House Office Building
Washington, DC 20515

Dear Chairman Upton and Ranking Member DeGette:

The Federation of American Societies for Experimental Biology (FASEB) thanks the Energy and Commerce Committee for its efforts to engage stakeholders in crafting the 21st Century Cures legislation. FASEB is comprised of 27 scientific societies which collectively represent over 120,000 biological and biomedical researchers. We previously submitted [comments](#) on the initial draft; the recommendations provided herein would significantly improve the proposed bill as well as address concerns that have been raised by our colleagues in the biomedical research community. Our suggestions focus on provisions concerning the National Institutes of Health (NIH) and address three basic themes: redundancy with existing regulations; micromanagement that could hinder future progress; and omission of key sections.

There is extensive redundancy with existing laws, policies, and agency activities

FASEB identified a number of provisions that duplicate existing regulations and practices; other sections propose creating new research and advisory entities whose missions, operations and functions parallel those of existing programs. Overlapping regulations increase the cost of—and decrease the time spent—conducting research. Similarly, funding multiple enterprises with comparable goals increases the burden on taxpayers and could lead to redundancy in effort. **The following sections of the bill are duplicative with existing regulations or ongoing efforts and should be deleted.**

Section and Title	Effort or Regulation Duplicated
Section 2001. Innovative Cures Consortium	<p>A number of institutions are currently engaged in work that mirrors the mission and operations of the proposed Consortium. They are:</p> <ul style="list-style-type: none"> • The National Center for Advancing Translational Sciences (NCATS) was created specifically to speed the delivery of cures to patients. NCATS partners with small businesses through its Small Business Innovation Research and Small Business Technology Transfer programs. NCATS' Clinical and Translational Science Awards program promotes collaboration among medical research institutions, and the Cures Acceleration Network provides NCATS with flexible funding mechanisms

	<ul style="list-style-type: none"> • The Critical Path Institute is a public-private partnership (PPP) focused on developing drugs for numerous high-profile diseases • The Accelerating Medicines Partnership is a PPP working to identify and qualify biomarkers to increase the availability of diagnostics and therapies for patients
Section 2021. Medical Product Innovation Advisory Committee	<p>The review of medical product innovation described in Sec. 2021 has already been done:</p> <ul style="list-style-type: none"> • The 2011 Institute of Medicine report “Medical Devices and the Public Health” provided an extensive review of FDA review and approval processes, as well as recommendations for facilitating innovation in the medical device industry • The 2014 RAND Corporation report “Healing Medical Product Innovation” presents ten areas of focus for increasing medical product development and innovation <p>Furthermore, the Medical Device Innovation Consortium is a nonprofit PPP created in 2012 with the goal of improving patient access to cutting edge medical technologies by better managing regulations impacting the medical device industry—nearly the same goal as the proposed Advisory Committee.</p>
Section 2081. Standardization of data in clinical trial registry data bank on eligibility for clinical trials	<p>Section 801 of Food and Drug Administration Amendments Act of 2007 expanded the National Library of Medicine’s clinical trials registry and results database, www.ClinicalTrials.gov. The data fields described in Sec. 2081 were incorporated into www.ClinicalTrials.gov through that mandate. Additionally, recent proposed guidelines from HHS and NIH, if enacted, would further expand the types of data reported and the types of trials required to register with the database.</p>
Section 2082. Clinical trial data system	<p>NIH and Agency for Healthcare Research and Quality are supporting research and development of systems that would allow approved groups to perform statistical analyses on a database without having access to the raw data (e.g. http://srdr.ahrq.gov/ and https://www.phenxtoolkit.org/index.php).</p>
Section 2201. Sharing of data generated through NIH-funded research	<p>As a result of the 2013 Office of Science and Technology Policy (OSTP) memorandum “Increasing Access to the Results of Federally Funded Scientific Research,” agencies, including NIH, have already started developing procedures and techniques to</p>

	facilitate greater access to data.
Section 2262. Report on the trends in age of recipients of NIH-funded major research grants	The 2012 Biomedical Research Workforce Working Group Report presented detailed analyses of this issue. Implementation activities of recommendations presented in the report are ongoing. Current information on new investigators is provided in the NIH Data Book .
Section 4002. Biomedical research working group to reduce administrative burden on researchers	<p>Several studies have already documented this problem:</p> <ul style="list-style-type: none"> • Federal Demonstration Partnership Faculty Surveys (2007, 2012) • 2014 National Science Board report “Reducing Investigators Administrative Workload for Federally Funded Research” <p>The 2014 National Academy of Sciences Committee on Federal Research Regulations and Reporting Requirements is currently examining the issue, as are several federal agencies (USDA, NSF) and multiple NIH groups (Advisory Committee to the Director, Center for Scientific Review, and Scientific Management Review Board).</p> <p>In addition, Rep. Comstock (R-VA) sponsored legislation (H.R. 1119) to establish an OSTP working group to recommend how to streamline regulations and reduce reporting burden for all federally-funded investigators, including NIH.</p>

Many provisions would micromanage NIH and could interfere with decision making based on scientific merit

The draft bill is overly prescriptive—yet concurrently overly simplistic—regarding how its goals should be accomplished. While some provisions may lead to short-term benefits, they would ultimately limit NIH’s ability to adapt to future research challenges.

The plight of early career scientists has received national media attention, but as a group they fare nearly as well as more experienced investigators in terms of grant success rates. The underlying problem is that too few research project grants are being awarded as a result of decreases in funding and spending power for NIH. **Section 2261, which increases funding specifically for new investigators, should be eliminated.** Increased funding solely for early career researchers, without concomitant increases for all applicants, will only shift the burden to other career stages.

Similarly, the call to fund more high-risk, high-reward science by NIH institutes and centers (I/Cs) in Section 2281 without allocating new monies to do so means that other essential research will suffer. Moreover, the Common Fund already has four dedicated high-risk, high-reward programs that support research in areas of interest to all I/Cs. **Unless additional funds are provided, FASEB recommends deleting Section 2281.**

The NIH-wide, five-year strategic investment plan proposed in Section 4001 is unnecessary. The vast majority, over 94 percent, of the NIH budget is allocated to I/Cs, which already develop their own strategic plans. Research sponsored by I/Cs lays the foundation for advances that will affect hundreds of diseases and multiple demographic groups. With scientific knowledge and opportunities expanding rapidly, there is enormous potential for breakthrough discoveries with wide-ranging benefits. However, the narrow parameters set for determining funding priorities in the draft legislation will politicize the funding process and hinder scientific progress by constraining inquiry. **Section 4001 is misguided and should be removed.**

Despite the section's title, provisions in Section 4004 directed at NIH I/C directors will do little to improve accountability. Subsection (a) would establish four-year term limits. With the exception of the National Cancer Institute, all I/C heads are appointed by and serve at the pleasure of the NIH director. Setting arbitrary term limits for directors will not improve I/Cs' productivity, but will likely impede future recruitment for these positions. Subsection (b) requires I/C directors to personally review and ensure that all new R-series grants are in the public interest and worth the investment. I/C directors already give final authorization for grants after they have undergone multiple rounds of review by experts in the field who scrutinize their scientific merit, innovation, and feasibility. However, while the social and economic value of a broad portfolio of research can be demonstrated, the benefits that will arise from any given research project cannot always be assessed in advance. This concept is a fundamental aspect of basic research, and it cannot be overstated that pursuing knowledge for the sake of knowledge, without expectation of benefit or reward, is the driving force behind some of the most important advances in health and medicine. These provisions suggest an underlying mistrust of the peer review process that is viewed as the gold standard for evaluating research and which other nations have strived to copy; therefore, **FASEB recommends removing subsections (a) and (b) from Section 4004.**

Several important sections are missing from the bill text

Finally, the discussion draft left blank several sections which could significantly impact the progress and success of the research enterprise. To ensure that the intent of the bill is achieved, FASEB would like to see the committee incorporate the following suggestions into the next draft.

It is our understanding that Section 2161, under Title II, Subtitle J – Modernizing Regulation of Diagnostics, will address regulation of laboratory developed tests (LDTs). The oversight of LDTs is a complex topic that affects many different endeavors. Discussions on the proper mechanism for regulating

LDTs have been divisive across the healthcare and research communities and have spanned decades. **The committee should consult the [comments](#) submitted to FDA on this guidance to gauge the sentiments of the healthcare ecosystem in which LDTs are developed and administered before finalizing any language on their regulation.**

Travel restrictions imposed on federal workers as a result of Executive Order 13589 and subsequent Office of Management and Budget memorandum M-12-12 have had unintended but dramatic consequences for researchers and clinicians at federal agencies. The limits on conference budgets and attendance at scientific meetings have led to a substantial decrease in participation by federal researchers, some of whom are missing out on continuing medical education credits they need to maintain licensure. **FASEB recommends that federal researchers and clinicians be exempt from these restrictions, and that language be added to Sections 4003 and 4101, on NIH and FDA travel, respectively, to reflect this.** Such language was included, for example, in the original text of the Senate Labor-HHS Appropriations Bill for fiscal year 2015:

“SEC. 526. (a) None of the funds in this Act may be available for agencies, or in the case of an agency with multiple bureaus, each bureau (or operating division) to support: (1) More than 50 agency employees on official travel away from their duty station to attend a particular conference; or (2) More than \$1,000,000 for sponsoring a conference. (b) This section shall not apply to conferences that are scientific in nature or scope.”

Sustained and predictable funding is essential to maintain a highly productive research enterprise, but the bill does not address this critical problem. Furthermore, a long-term plan for increasing federal investment in research and development is necessary to restore the constant dollar losses in funding that have reduced the NIH budget by over 20 percent since 2003. **FASEB recommends granting multi-year budget authority to NIH through the 21st Century Cures Act, along with a commitment to increases in appropriations of at least five percent annually for the next five years.** This would enable thoughtful planning and efficient use of funding, and parallels suggestions from the American Academy of Arts and Sciences in its 2014 report “Restoring the Foundation: The Vital Role of Research in Preserving the American Dream.” Sample text should read:

SEC. 4010. AUTHORIZATION OF APPROPRIATIONS.

(a) Funding.— 402a(1) of the Public Health Service Act (42 U.S.C. 282a(1)) is amended to read as follows:

``SEC. 402A. AUTHORIZATION OF APPROPRIATIONS.

``(a) In General.--For the purpose of carrying out this title, there are authorized to be appropriated--

``(1) \$32,000,000,000 for fiscal year 2016, to remain available until September 30, 2017; and

``(2) such sums as may be necessary for fiscal year 2017, to remain available until September 30, 2018.

``(3) such sums as may be necessary for fiscal year 2018, to remain available until September 30, 2019.

``(4) such sums as may be necessary for fiscal year 2019, to remain available until September 30, 2020.

``(5) such sums as may be necessary for fiscal year 2020, to remain available until September 30, 2021.

FASEB appreciates the Energy and Commerce Committee's concern for the future of biomedical research. The opportunities for progress have never been greater, but we must move forward in a way that stimulates and encourages innovation. We encourage the committee to thoughtfully consider how best to incorporate these suggestions into the next draft of the 21st Century Cures Act.

Sincerely,



Joseph R. Haywood, PhD
FASEB President

Institute for Advanced Application
Geisinger Health System
100 N. Academy Ave., MC 44-00
Danville, PA 17822
Telephone: 570-214-1463
Fax: 570-214-9541

Gregory J. Moore MD, PhD
Director
gjmoore@geisinger.edu

GEISINGER
HEALTH SYSTEM

March 9, 2015

The Honorable Fred Upton, Chairman
The Honorable Diana DeGette
Energy & Commerce Committee
United States House of Representatives
2125 Rayburn House Office Building
Washington, DC 20515

Subject: 21st Century Cures Initiative

Dear Chairman Upton & Congresswoman DeGette:

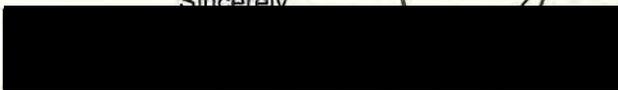
I am writing to express my enthusiastic support for the 21st Century Cures Initiative and to thank you for your leadership in advancing this important legislation. Specifically, I want to express my support of the provision adding "research" to the definition of "healthcare operations". This change will provide physicians and scientists with access to critical data needed for medical research and quality assessment, including the ability to assess the effectiveness of new treatments and interventions in the patients we desire to heal. The vast majority of this valuable data is currently siloed or locked away and not available to researchers due to an impractical regulatory process which requires that physicians and scientists obtain a specific individual consent for every single use of healthcare records for research purposes.

While I am a strong advocate for patient privacy, I am also equally passionate as a physician and scientist to serve my patient's best interests by using the highest quality of evidence available to alleviate their suffering, to diagnose and cure their disease, improve the quality of their care and minimize the possibility of errors. Enabling efficient and practical access to the data contained in these clinical records will provide much needed insight to physicians and scientists seeking to understand some of the most significant healthcare questions of our time.

My patients, colleagues and neighbors are nearly unanimous in their altruistic desire and willingness to have their clinical data used to help their fellow human beings suffering from illness and disease. As physicians and scientists it is our solemn responsibility to use this data responsibly and with a sense of urgency to bring healing to humankind. As lawmakers, you are in a pivotal position to enable practical access to healthcare data which will allow biomedical researchers and physicians the opportunity to fulfill their missions of discovery and healing.

Once again thank you for your leadership in advancing this important initiative. Please feel free to contact me directly at (570) 214-1463 or at gjmoore@geisinger.edu if you have any questions or would like to follow-up on any specific items. I am at your service in any way you or your staff might find helpful in advancing this legislation.

Sincerely,



Gregory J. Moore, MD, PhD
Chief Emerging Technology and Informatics Officer
Director, Institute for Advanced Application
Geisinger Health System

<http://geisinger.iaa.org>

WWW.GEISINGER.ORG



April 13, 2015

The Honorable Fred Upton (R-MI)
Chairman
Energy & Commerce Committee
2125 Rayburn House Office Building
Washington, DC 20501

The Honorable Frank Pallone (D-NJ)
Ranking Member
Energy & Commerce Committee
2322A Rayburn House Office Building
Washington, DC 20501

Dear Chairman Upton and Ranking Member Pallone:

I am writing on behalf of the Healthcare Supply Chain Association (HSCA) concerning the 21st Century Cures discussion draft. We appreciate your efforts aimed at accelerating the discovery, development, and delivery of new treatments and cures for patients.

HSCA is a broad-based trade association that represents 14 group purchasing organizations (GPOs), including for-profit and not-for-profit corporations, purchasing groups, associations, multi-hospital systems and healthcare provider alliances. HSCA's mission is to advocate on behalf of healthcare group purchasing associations to provide educational opportunities designed to improve efficiencies in the purchase, sale and utilization of all goods and services within the health industry and to promote meaningful dialogue between GPOs and their provider customers.

Clinical data is the key to improving efficiencies. As the Committee prepares a second draft proposal, HSCA encourages policymakers to help standardize data requirements. The use of unique device identifiers is one in a series of steps that will help modernize product supply. If integrated in interoperable electronic health records (EHRs), then additional supply chain efficiencies can be further unlocked generating \$16 billion in savings annually.

Health Information Technology (HIT) is necessary for comprehensive care. Many HIT systems are "locked" within their data silos, hindering their ability to connect and exchange information with other EHR systems or mechanisms critical for patient care. One way to ensure data interoperability is to deploy common data architecture that requires open-source standards, such as application programming interfaces (API) that enable secure and innovative applications to provide the interoperability necessary to quickly, easily and affordably integrate data with other systems. It is, of course, important to ensure the functionality of APIs within practical workflow scenarios before tying such policies to meaningful use requirements for currently certified EHR technologies.

As the Committee strives to improve the healthcare supply chain, we encourage a private public partnership established in collaboration with the leadership of the federal government's Office of National Coordinator for Health Information Technology (ONC). This will help drive the

development of a nationwide framework on critical matters such as patient identifiers, clinical queries, security and certified methodologies. We also believe that the partnership can aid in leveraging (GSI U.S.) standards. This includes Global Location Numbers (GLNs) and Global Trade Item Numbers (GTINs). Used together, they can identify each product item to be scanned into information systems such as EHRs.

As healthcare shifts from volume- to value-based federal payment systems, and as negotiations move from price-driven to quality and performance measurements, evolutionary technological changes in the supply chain will help provide meaningful data that can lead to more patient-centered care. Advances in HIT and increasing adoption of global standards will help connect the supply chain so that healthcare providers, suppliers and manufacturers can more efficiently collaborate and effectively treat patients. HSCA is working to bring about a world in which EHRs connect all aspects of the supply chain. Standardizing data protocols and leveraging U.S. standards in a global market is central to the delivery of promising new treatments and sustaining medical innovation.

Thank you again for spearheading this initiative. HSCA's member companies are on the front lines in the quest for common data sets as innovate technologies continue to be introduced in the health care marketplace. We look forward to sharing additional policy perspectives – legislatively and administratively – as you and your colleagues work to enhance the safety of patients, and the larger health care provider community.


Signature


Curtis Rooney
President
Healthcare Supply Chain Association

cc: The Honorable Diana DeGette

04-17-15

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, appreciate this opportunity to submit comments on the immunization-related provisions of the discussion draft of the 21st Century Cures Act, as released by the House Energy and Commerce Committee majority on January 27, 2015.

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 recommendations along with safety and efficacy data and guidelines for use are published in the Morbidity and Mortality Weekly Report (MMWR). Key health provider associations, including the American Academy of Pediatrics, the American Academy of Family Physicians, the American College of Nurse-Midwives, 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 would like to express our concerns about provisions of the discussion draft that could disrupt this balance by imposing rigid requirements and deadlines for action. It is 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 discussion draft would require 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 directs that if the ACIP does not make a recommendation within 120 days of licensure, a manufacturer may submit a request that would then require the ACIP to draft and vote on a recommendation within 60 days of receipt of that request.

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 votes on vaccine recommendations 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 “standard timelines” 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 in turn 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

We are concerned that Section 4042 of the discussion draft, “Review of Transparency and Consistency of ACIP Recommendation Process,” could have unintended consequences for

important aspects of the ACIP review process with regard to both transparency and consistency of recommendations.

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, 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. Work groups often examine confidential business information provided by industry, and removing that confidentiality could lead to less information provided to ACIP, which in turn may delay new vaccine recommendations. Key information is released publicly at predictable junctures, and votes take place solely at open meetings. We are concerned that Section 4042 could disrupt this careful balance by introducing new opportunities for either the appearance or actual exercise of undue influence.

Section 4042 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

Section 4044 of the discussion draft, “Meetings Between CDC and Vaccine Developers,” would require that CDC meet with vaccine industry officials within certain timeframes, 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.”

This section 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 could divert precious resources from other, more urgent public health needs. The mandate for CDC to provide specific, detailed information to industry officials 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.

In conclusion, we deeply appreciate this opportunity to express our views regarding the immunization provisions of the discussion draft of the 21st 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.

Sincerely,

American Academy of Family Physicians
American Academy of Pediatrics
American College Health Association
American College of Nurse-Midwives
American College of Physicians
American Congress of Obstetricians and Gynecologists
American Osteopathic Association
Every Child By Two - Carter/Bumpers Champions for Immunization
First Focus
March of Dimes
National Association of County and City Health Officials
National Association of Pediatric Nurse Practitioners
National Foundation for Infectious Diseases
Voices for Vaccines



MITA[®]
MEDICAL IMAGING
& TECHNOLOGY ALLIANCE
A DIVISION OF **NEMA**[®]

1300 North 17th Street • Suite 900
Arlington, Virginia 22209
Tel: 703.841.3200
Fax: 703.841.3392
www.medicalimaging.org

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

March 20, 2015

Re: 21st Century Cures Discussion Draft

Dear Chairman Upton:

As Executive Director of the Medical Imaging and Technology Alliance (MITA), I applaud you for your 21st Century Cures Initiative aimed at accelerating the discovery, development and delivery of promising new treatments and cures for patients. We appreciate your inclusion of industry stakeholders in this process.

MITA commends you on the continued bipartisan efforts to promote substantive and positive changes in FDA's regulation of medical devices. 21st Century Cures puts patients first; enhancing American's access to safe and effective advancements in healthcare. As the committee has often noted, the evolution of regulatory frameworks has fallen behind the pace of innovation and we believe legislative intervention can appropriately address a number of challenges the Agency has struggled to resolve in recent years.

Among other major points, we believe establishing a prioritized process for approving breakthrough devices will improve regulatory efficiency, advance lifesaving technologies, and heighten the quality of patient care. We also strongly support efforts to enhance FDA's use of third party review and certification of manufacturing processes and changes. Third party certification represents a substantial opportunity for improving regulatory efficiency and the allocation of FDA resources. We believe similar third party certification could extend to an even broader group of device modification to include all those which do not impact the clinical performance or risk profile of a device.

As you may know, MITA submitted a proposal for 21st Century Cures that was not included in this initial draft. One significant obstacle to faster diagnoses and staging of diseases is persistent difficulty gaining Medicare coverage. Specifically, imaging technologies are being held to an unreasonable standard to achieve coverage and provide access to life-saving technologies. Given the different applications of medical technology in diagnostic and therapeutic settings, the federal government logically should use different endpoint metrics when making coverage determinations.

MITA has identified two policy options to advance this goal, and submitted the following for your consideration.

1. Direct CMS to create an "endpoint" standard for coverage determinations that would differentiate between diagnostic imaging and therapeutic technologies.

Proposed amendment to the Social Security Act:

Section 1862(l)(1) of the Social Security Act (42 U.S.C. 1395y(l)(1)) is amended by inserting the following after the first sentence:

“No later than January 1, 2018, the Secretary shall make available to the public a guidance document describing standards for the clinical endpoints to be used to support national and local coverage determinations that differentiate diagnostic imaging technologies from therapeutic technologies. This standard shall recognize that intermediate endpoints, such as a change in management of a patient’s illness, are appropriate for diagnostic imaging technologies. Further, this standard shall prohibit burdensome requirements which are more suited to coverage of a therapeutic, such as endpoints and designs for coverage with evidence development which include Data Safety Monitoring Boards and long-term follow-up of patients beyond the therapeutic decision, for example”.

2. Require the GAO to conduct a study to assess how the CMS coverage process related to diagnostic imaging meets the needs of the Medicare population. The disease states included would not be limited to, but would potentially include, Alzheimer’s, cancer, and other conditions where emerging diagnostics could be impactful from a patient management and health economics perspective.

Proposed legislative language:

(a) GAO STUDY OF MEDICARE NATIONAL COVERAGE DETERMINATIONS FOR DIAGNOSTIC IMAGING.—

(1) STUDY.—The Comptroller General of the United States shall conduct a study to assess whether the process for developing national coverage determinations related to diagnostic imaging meets the needs of the Medicare population. Such study shall include—

- (A) an assessment of access to diagnostic imaging services following issuance of national coverage determinations on such services in the past 10 years;
- (B) the evidentiary standards used in national coverage determinations for diagnostic imaging, variations in those standards across diagnostic imaging national coverage determinations, and comparison of those standards to the standards applied to therapeutic technologies;
- (C) variations in evidentiary standards applied to diagnostic imaging for different disease states, such as Alzheimer’s Disease and cancer; and
- (D) potential effects of the evidentiary standards used in the national coverage determination process on development of and access to diagnostic imaging for Alzheimer’s Disease, cancer, and other conditions where emerging diagnostic imaging technologies could have an impact on patient management and health economics.

(2) CONSULTATION WITH STAKEHOLDERS.—In conducting the study described in subsection (1), the Comptroller General shall interview relevant stakeholders, including diagnostic imaging providers, patient groups, and manufacturers of diagnostic imaging technology, about the topics identified in paragraphs (A) through (D) of subsection (1).

(3) REPORT.—Not later than 18 months after the date of the enactment of this Act, the Comptroller General shall submit to Congress a report on the study conducted under subsection (1), together with recommendations for such legislation and administrative action as the Comptroller General determines appropriate.

With additional insights from various stakeholders and a reasoned approach to crafting robust legislative language, we believe this bill represents a potential watershed in the modernization of regulation to accommodate the increasing velocity of change and innovation in medical treatment options. MITA would like to provide some additional comments on particular items in the discussion draft to positively progress the overall intent of the committee.

Title I - Putting Patients First by Incorporating Their Perspectives into the Regulatory Process and Addressing Unmet Needs

Subtitle B – Surrogate Endpoint Qualification and Utilization

Surrogate end points are currently used for a variety of devices, both low and high risk. MITA would encourage and support CDRH’s continued use and acceptance of surrogate endpoints. The current discussion draft language would amend Section 507 of the FDCA for prescription drugs, but MITA believes unnecessarily references medical devices in two places (p. 18, L1-7; p. 26, L5-8).

Title 2 – Building the Foundation for 21st Century Medicine, Including Helping Young Scientists

Subtitle E – Sensible Oversight for Technology Which Advances Regulatory Efficiency (SOFTWARE)

The Software Act provides sound concepts which can be bolstered through effective editing during the drafting process. The language is vendor and source neutral—addressing a longstanding concern regarding medical software. At present, medical device manufacturers are often required to submit 510(k)s for certain software products, while technology companies outside the traditional medical device industry have been able to introduce similar software products without the need for FDA review.

This bill clarifies that it is the content of the software and its correlated use cases, not the platform, which determines the need for regulatory oversight. Software should be regulated based on intended use, regardless of regulatory path.

Regarding the definition of ‘medical software’: 201(ss)(2)(C)(i) defines medical software as recommending a treatment to a healthcare professional “without the need for such professional to perform additional interpretation of, or to independently confirm the means for, such recommendation,” while 201(ss)(2)(C)(ii) conversely states the software is “for the purpose of informing or influencing health care decisions...” The first statement implies the information is provided without the need or means for additional thought, while the second statement infers the health practitioner independently reaches a medical decision. MITA recommends clarifying this language based on whether the committee intends decision-making to lie within the software or the healthcare provider for the purposes of this classification.

We are also concerned the current language of the bill would deregulate complex software capable of calculating essential aspects of a medical procedure without further input or review by the attending physician (black box software). Some examples include radiation dosage calculators, radiation therapy treatment planning software, and robotic surgical planning and control. We strongly suggest the committee include risk analysis language designed to reserve certain high-risk devices from the categories defined here. The bill should specifically exclude software in the planning or execution of high-risk medical procedures. It is important to note that health software would be excluded entirely from FDA regulation, including quality systems requirements.¹

While more difficult to implement and conceptually inelegant, the provisions of this bill should provide a risk criteria excluding high risk software from this legislation where regulation as a medical device or accessory remains more appropriate. Software can be used for a large variety of medical purposes. In that respect the arguments do not differ from those used for other medical devices. Standalone software can directly control an apparatus (e.g. radiotherapy treatment), provide immediate decision triggering information (e.g. blood glucose meters), or provide support for healthcare professionals (e.g. ECG interpretation). Owing to this broad spectrum in functionality, some nuance around risk is essential for meeting the safety needs of patients while creating a rationale regulatory alternative to software oversight.

The International Medical Device Regulators Forum, in which FDA participates, has previously produced a well-reasoned document entitled *Software as a Medical Device: Possible Framework for Risk Categorization and Corresponding Considerations*. This document outlines important factors to consider when reviewing software's characterization as a potential medical device. We encourage the committee to review the four categories outline by IMDRF and the associated risk criteria. Understanding the principles outlined by the IMDRF would help the committee craft appropriate mechanisms for maintaining regulation of certain high-risk software.²

Subtitle H – Coverage with Evidence Development

We applaud the Committee's inclusion of improved health outcomes in informing CED decisions. MITA requests that in addition to the criteria currently outlined in the discussion draft, patient experience data also be included in the assessments. We have included prospective legislative language and how it fits in the current draft.

SEC. 2121. AUTHORITY FOR COVERAGE WITH EVIDENCE DEVELOPMENT FOR MEDICAL DEVICES UNDER THE MEDICARE PROGRAM

(a) EXCEPTION TO REASONABLE AND NECESSARY REQUIREMENT.—Section 1862(a)(1)(A) of the Social Security Act (42 U.S.C. 1395y(a)(1)(A)) is amended by inserting “or a CED item or service (as described in section 1861(iii))” after “(as described in section 1861(ddd)(1))”.

¹ Proposed section 3 modifying 21 U.S.C 351 et seq. at proposed Sec. 542b(b)

² [IMDRF/SaMD WG/N12FINAL:2014](http://www.imdrf.org/docs/imdrf/final/technical/imdrf-tech-140918-samd-framework-risk-categorization-141013.pdf), viewed at <http://www.imdrf.org/docs/imdrf/final/technical/imdrf-tech-140918-samd-framework-risk-categorization-141013.pdf>

(b) DEFINITION OF CED ITEM OR SERVICE.—Section 1861 of the Social Security Act (42 U.S.C. 1395x) is amended by adding at the end the following new subsection:

‘(iii) CED ITEM OR SERVICE.—

“(1) IN GENERAL.—The term ‘CED item or service’ means an item or service that is for coverage with evidence development (as described in paragraph (2)).

‘(2) COVERAGE WITH EVIDENCE DEVELOPMENT.—For purposes of paragraph (1), an item or service is for coverage with evidence development if—

“(A) the item or service is furnished to individuals as part of a clinical study performed to determine whether the furnishing of such item or service improves the health outcomes of such individuals, as determined under paragraph (3); and

“(B) the furnishing of the item or service to the individual is determined by the Secretary to be reasonable and necessary to the carrying out of such clinical study.

‘(3) DETERMINATION OF IMPROVED HEALTH OUTCOMES.—For purposes of paragraph (2)(A), a determination of whether the furnishing to individuals of items or services improves the health outcomes of such individuals shall be determined by assessing whether the furnishing of such items or services improves the—

‘(A) evaluation of the patient problem list to achieve diagnosis and staging of, or treatment planning for, illnesses or injuries...

(B) or treatment of illnesses or injuries of such individuals (as compared to the diagnosis or treatment of illnesses or injuries of comparable individuals who are not so furnished such items or services);

“(B) functioning of malformed body members of such individuals (as compared to the functioning of malformed body members of comparable individuals who are not so furnished such items or services), or

(C) ability of patients, caregivers, or treating physicians to develop more appropriate care plans, as determined by approved patient experience data.

(4) DEVELOPMENT AND USE OF PATIENT EXPERIENCE DATA TO ENHANCE THE CED DETERMINATION FRAMEWORK.—

‘(A) IN GENERAL.—Not later than two years after the date of the enactment of this subsection, the Secretary shall establish and implement processes under which—

“(a) an entity seeking to develop patient experience data may submit to the Secretary—

“(i) initial research concepts for feedback from the Secretary; and

“(ii) with respect to patient experience data collected by the entity, draft guidance documents, completed data, and summaries and analyses of such data;

“(B) the Secretary may request such an entity to submit such documents and summaries; and

“(C) patient experience data may be developed and used to enhance the improved outcomes determination framework under subsection (3).

“(5) PATIENT EXPERIENCE DATA.—In this subsection, the term ‘patient experience data’ means data collected by patients, parents, caregivers, patient advocacy organizations, disease research foundations, or medical researchers that is intended to provide information about the experience of patients with a disease, or the impact a disease and management of the disease has on the lives of patients or their caregivers.”

(6) COVERAGE – services and items provided under the CED framework shall be covered in a manner consistent with the indications for use in the proposed coverage policy.”

Title IV – Accelerating the Discovery, Development, and Delivery Cycle and Continuing 21st Century Innovation at NIH, FDA, CDC, and CMS

Subtitle I - Telemedicine

We approve of including store-and-forward technology in the definition of “telehealth services,” understanding the committee intends to include technologies which capture, store, and electronically transmit digital diagnostic medical images and/or diagnostics imaging reports.

The committee asked how store-and-forward should be defined. We propose the following: *computer mediated data storage and transmission system(s) utilized for review or consultation of medical or patient information previously captured for the purpose of diagnosis, prognosis, treatment, or disease management through secure digital communication.*

Subtitle P—Medicare Pharmaceutical and Technology Ombudsman

The establishment of a Medicare Pharmaceutical and Technological Ombudsman to make Medicare more responsive and open to concerns over its sometimes arbitrary policies is a step forward, as it will streamline communications between CMS and industry stakeholders on coverage decisions. We would recommend that the legislation include more specifics on the role of the ombudsman, particularly his or her responsibilities.

Title V – Modernizing Medical Product Regulation

Subtitle D – Medical Device Reforms

Section 5061— Third-party quality systems assessment

Allowing FDA to rely on third party accredited bodies to certify technology or manufacturing changes would reduce the overall number of premarket submissions, lessen unnecessary documentation requirements, and improve the use of FDA resources. MITA encourages the committee to consider expanding this provision to third-party certification of any modification which has no clinical impact on the safety or performance of the device.

Section 5062 – Valid Scientific Evidence

We support, under sub-clause III, the inclusion of data gathered outside the United States as valid scientific evidence. This data, in our opinion, is appropriate for scientific use when it meets the criteria defined in sub-clause I.

Section 5063 – Training and Oversight in Least Burdensome Means Concept

Proper training of FDA reviewers is vital to a properly function regulatory process. In addition to the proposed language in the discussion draft, MITA suggests including language to emphasize the proper use of FDA guidance by review staff.

There is a significant distinction between guidance and regulatory requirements. Guidance is merely a suggestion based on current FDA knowledge regarding a certain pharmaceutical or device. It is entirely possible for a given manufacturer to devise a less-burdensome and more appropriate pre-market submission package than that outlined in an official guidance. This is especially true as a guidance document ages over time and a technology matures. In our experience however, guidance documents are often strongly enforced as requirements even as they become outdated.

Reviewers should also receive specific instruction on appropriately using draft guidance. There is often a period of several months between the release of a draft guidance document, the comment period, and release of a final document. During the interim period, inexperienced reviewers are often tempted to rely on draft guidance as precedent. Draft guidance is often changed significantly based on the comments and insights of various policy and technical experts familiar with the costs and feasibility of the proposed guidance. Due to these changes, guidance requirements will have been unevenly applied between the draft and final stages. A reviewer may also rely on a draft guidance which becomes rescinded or delayed. Through inexperience, they may continue to rely on the invalidated guidance despite its removal from drafting process. To avoid these undesirable scenarios, it's important for reviewers to understand the drafting process and the importance of public notice and commenting.

Section 5064 – Recognition of Standards

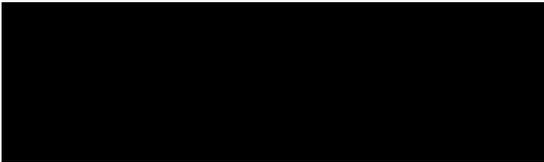
MITA agrees that vetted, recognized standards play an important role in medical device manufacturing and, when certified against, offer an efficient alternative to traditional regulatory oversight. Expanded and further informed use of national and international standards is a shared goal of many in the device world seeking regulatory alignment. While the current legislative language would require FDA to transparently review and resolve their disposition on new or updated standards, it may result in the agency becoming inundated with proposed standards of varying quality. There are many 'national' (both U.S. and non-U.S.) and international standards development organizations. We suggest limiting this to US national standards developing organizations (SDOs) and certain international SDOs such as IEC

and ISO. Requiring a detailed public notice of FDA's opinion on too broad a swath of 'national' or international standards would likely prove unsustainable. Further, we must advise against the default recognition of standards in the absence of Agency action. The volume and pace of standards development far surpasses any one entities ability to review even a majority of new medical device standards.

To accomplish the intent of this provision, the legislation should require the Secretary of Health and Human Services to create a process for identifying and adopting best-in-class standards from national (US) and international SDOs recognized as having expertise in developing consensus standards for device manufacturing, safety, and performance. Any process should define how FDA is notified when a new standard becomes available, and how promising standards are selected for review and possible recognition.

Thank you for the opportunity to participate in the 21st Century Cures Initiative process. We appreciate the committee's leadership on these important issues, and look forward to working with you to advance these proposals.

Sincerely,



Gail Rodriguez
Executive Director



April 8, 2015

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

The Honorable Frank Pallone
Ranking Member
House Committee on Energy and
Commerce
U.S. House of Representatives
Washington, D.C. 20515

Dear Chairman Upton and Congressman Pallone:

On behalf of the nation's Medicaid Directors, we appreciate the opportunity to provide the Energy and Commerce Committee with comments on the 21st Century Cures Initiative and the January 27, 2015, discussion draft proposal.

NAMD is a bipartisan organization which represents Medicaid Directors in the fifty states, the District of Columbia and the territories. The Association was created in part to develop consensus among Directors on critical issues, specifically those that have national policy implications. In recent years, Directors have coalesced around emerging trends shaping access to and expenditures for outpatient prescription drugs, devices and related pharmaceutical therapies.

The 21st Century Cures and related Senate initiative could result in the delivery of meaningful access for all U.S. citizens over the longer term to high-quality, cutting-edge pharmaceuticals. We remain concerned, however, that the Committee has focused almost exclusively on the development and access components for stimulating innovation, and has not dedicated sufficient attention to equally important issues that impact payers and the safety of patients.

As you know, under federal statute pharmacy services are an optional benefit for most Medicaid-eligible populations. However, states have historically recognized that prescription drugs and devices are integral in prevention, treatment and maintenance of health and well-being for most individuals. Currently all states include pharmacy in their benefit Medicaid packages, and as of 2013, Medicaid expenditures on outpatient prescription drugs topped \$40 billion.



Over two decades ago, as part of the Omnibus Reconciliation Act of 1990 (OBRA '90), Congress established the Medicaid Drug Rebate Program (MDRP) to provide expenditure offsets for covered outpatient drugs utilized by Medicaid populations. The MDRP requires that the Medicaid program cover all drugs approved by the Food and Drug Administration (FDA) where the manufacturer has signed a federal rebate agreement. In some situations, states have secured additional drug expenditure offsets by establishing state supplemental drug rebate programs.

As compared to new, innovative, “curative” drugs that have unit cost pricing in the thousands of dollars, the original MDRP was designed to provide offsets for drugs where the unit cost was several magnitudes lower. Additionally, state Medicaid programs are finding that legacy pharmacy market cost containment and utilization strategies are proving ineffective and potentially creating barriers or inequities or both for patient access to new novel “curative” therapies.

Modifications to the FDA’s drug and device approval pathways and continued breakthroughs in science innovations and medical technologies coupled with the growth in the Medicaid enrollment, require a comprehensive review of the current MDRP incentives as they apply to Medicaid and drug manufacturers. This is particularly relevant to ensure states can advance value-based purchasing, risk-sharing and proven quality outcomes instead of the current no-risk sharing, discounted payment model which is driven by rebate agreements with fixed Average Manufacturer Price-based discounts.

Any meaningful discussion to improve process and incentives for the development and approval and access of pharmaceuticals for the U.S. market requires careful consideration by Congress. Federal policymakers must assess the impact to U.S. prescription drug budgets, insurance premiums and costs borne by the state Medicaid programs, taxpayers and patients.

Specifically, we believe Congress should carefully examine the existing legacy payment and reimbursement regulatory frameworks to ensure that the innovations proposed in the 21st Century Cures or similar proposals are appropriately balanced with equitable pharmaceutical pricing and payment strategies that create a sustainable and fiscally responsible competitive market. Further, Congress must assess the full spectrum of patient-related issues. New pathways and incentives should include appropriate protections for vulnerable patients, particularly those enrolled in the Medicaid program, to ensure they are not inadvertently subject to adverse consequences.

The key issue of U.S. pricing and expenditure offsets for new high-touch and high-cost curative pharmaceuticals remains a high priority for states. Enclosed we provide additional comments on the discussion draft. We also refer you to the NAMD letter transmitted to congressional



leaders on October 28, 2014, which discussed many of these issues in the context of hepatitis C therapies.

We appreciate your consideration of our comments. We remain committed to informing federal policy discussions and potential changes to federal statute that may impact the Medicaid program.

Sincerely,



Thomas J. Betlach
Arizona Health Care Cost
Containment System Director
State of Arizona
President, NAMD



John B. McCarthy
Director
Ohio Department of Medicaid
State of Ohio
Vice-President, NAMD

Cc:

Congressman Tim Murphy, Chairman, Subcommittee on Oversight and Investigations, House
Committee on Energy and Commerce
Congresswoman Diana DeGette, Ranking Member, Subcommittee on Oversight and
Investigations, House Committee on Energy and Commerce



Spurring Innovation in Pharmaceutical Development and Access: A Medicaid Perspective

The following comments from the National Association of Medicaid Directors (NAMD) address the January 27, 2015, discussion draft as posted on the House Energy and Commerce Committee website. Our overarching comments focus on the likely impact to state Medicaid program policies and budgets as well as safety considerations for the Medicaid-eligible population. We also offer comments on specific provisions of the discussion draft.

General Feedback

- The provisions of the discussion draft do not take into account ramifications for payers, both public and private, particularly with regards to the extended exclusivity periods for new or modified therapies.
- Congress should evaluate the Medicaid drug rebate program (MDRP) in the context of the emerging market for high-touch, high-cost curative pharmaceuticals. The MDRP was designed to provide offsets for drugs where the unit cost was several magnitudes lower than recent breakthrough therapies. Additionally, legacy pharmacy market cost containment and utilization strategies are proving ineffective and potentially creating barriers for patient access to novel curative therapies. Medicaid pharmacy program reforms will be required to address benefit design flexibility and value-based payment models to support Medicaid beneficiary access to these therapies.
- Lowering the evidentiary standard for drug and device approvals without granting coverage flexibility to the Medicaid program makes the Medicaid population a captive market for these products and potentially puts vulnerable Medicaid populations at risk. Other payers may choose not to cover these therapies, but current law requires Medicaid to do so.
- Medicare coverage and policy decisions will have downstream impacts and costs for the Medicaid program, both on the Medicaid-Medicare dually eligible population and the potential need for states to support providers to comply with new Medicare policies.

Section-by-Section Feedback

Proposal Provisions	NAMD Comments/Questions
<p><i>Sec. 1041 – Approval of Breakthrough Therapies</i></p> <ul style="list-style-type: none"> • <i>Allows the FDA to approve a drug, for a disease/condition with unmet medical need, that has received “breakthrough therapy” designation based on early stage clinical safety/effectiveness data that provides sufficient evidence under current safety and efficacy standards</i> • <i>Allows HHS to require post-market assessment of the drug, with ability to withdraw approval if assessment is not conducted, drug is found unsafe and/or ineffective, or manufacturer uses false or misleading marketing</i> 	<ul style="list-style-type: none"> • This provision appears to lower the evidentiary standards for this drug class. • Shorter FDA review times combined with increased FDA authority to require further studies <i>after</i> approval, rather than settling safety issues <i>before</i> approval, may contribute to increased rates of patient safety risks, drug withdrawals, and black box warnings. • Patient education is needed about any new approval pathway and the potential risks of fast-tracked therapies. • The Medicaid population is significantly different from the privately insured population. This raises concerns about the quality of evidence for drug approval as it pertains to a drug’s effects on Medicaid’s generally frailer, sicker population. • Medicaid is statutorily required to cover any FDA-approved drug in exchange for mandatory rebates. No other payer is under this same obligation. <u>In effect, the lowered evidentiary standards of this provision makes the Medicaid population a captive market for any potential adverse effects not discovered in the accelerated approval pathway. Further, the current framework would make the Medicaid program responsible for managing the short and long-term costs associated with such outcomes.</u> • If this provision is enacted, policymakers should consider a corresponding policy for periodic surveillance reports on adverse events, outcomes, etc. for Medicaid and other vulnerable populations. • <u>Policymakers should consider the benefits of providing new Medicaid flexibility in at least two ways:</u> <ul style="list-style-type: none"> ○ Flexibility for state Medicaid programs to <i>not cover</i> drugs approved under this provision until sufficient post-approval studies have been conducted. ○ Flexibility for state Medicaid programs to enter into value-based payment arrangements for drugs approved under this

	<p>provision, with payment contingent on drug efficacy and safety.</p>
<p><i>Sec. 1063 – Election to Convey a Portion of Extended Exclusivity Period Applicable to Qualified Infectious Disease Products</i></p> <ul style="list-style-type: none"> <i>Extends exclusivity periods for “qualified infectious disease products” by 5 years</i> <i>Allows manufacturers to apply up to one year of this extended exclusivity to one or more drugs, in exchange for a commensurate reduction in exclusivity for the designated infectious disease product</i> <i>Manufacturers are required to make a donation of profits to the NIH and a patient assistance programs.</i> 	<ul style="list-style-type: none"> This provision may delay the introduction of new generic antibiotics and delay generics for entirely separate drug categories. As proposed, manufacturers could extend exclusivity for particularly high-cost drugs longer than they otherwise would. The variable exclusivity arrangement also removes predictability for state planning purposes, and could potentially impact Medicaid’s ability to negotiate supplemental rebates for comparable treatment options. Policymakers may wish to consider enhanced Medicaid rebates, value-based purchasing flexibility for Medicaid programs, or similar policies that reflect the burden of extended exclusivity periods to mitigate these concerns. The potential effect of high-cost therapies with extended exclusivity periods on state budgets, even if there were a potentially enhanced rebate, should be taken into consideration when crafting such a policy. If policymakers were to allow a manufacturer to extend an exclusivity period for a “qualified infectious disease product,” they may wish to consider tying this to the availability of a generic or lower-priced drug belonging to the same therapeutic category.
<p><i>Sec. 1064 – Encouraging the Development and Use of New Antimicrobial Drugs</i></p> <ul style="list-style-type: none"> <i>Adds supplemental Medicare payment to hospital discharges which use new antimicrobial drugs.</i> 	<ul style="list-style-type: none"> Further analysis is needed to ensure this provision does not create an incentive for overutilization of new antimicrobial drugs. Policymakers should consider that, to the extent that such new drugs are utilized, Medicaid cost-sharing for Medicare-Medicaid enrollees will increase. Further analysis is needed to assess other potential impacts on the Medicaid program. If policymakers include a supplemental Medicare payment they should also consider making this contingent upon other factors, such as the general ineffectiveness of previous antimicrobial drugs.

<p>Sec. 1081 – Priority Review for Breakthrough Devices</p> <ul style="list-style-type: none"> Creates a priority review program for breakthrough devices which have no approved alternative or offer significant advances over existing devices <p>Sec. 1082 – CMS Coverage of Breakthrough Devices [currently a placeholder]</p> <p>Sec. 1101 – Accelerated Approval for Breakthrough Devices</p> <ul style="list-style-type: none"> Allows HHS to approve breakthrough devices based on surrogate endpoints that are reasonably likely to predict clinical benefit Such approval may be subject to post-approval studies 	<ul style="list-style-type: none"> The forthcoming placeholder section could impact Medicaid coverage policies. Policymakers should consider the policy, clinical and budgetary impact to Medicaid. The accelerated approval provision could lower the evidentiary standard for device approval. This reduction will make it more difficult for Medicaid programs to utilize evidence-based assessments for coverage decisions. <u>The Medicaid population is significantly different from the privately insured population. This raises concerns about the quality of evidence for device approval as it pertains to the device’s effects on Medicaid’s generally frailer, sicker population.</u> If enacted, periodic surveillance reports on adverse events, outcomes, etc. for Medicaid and other vulnerable populations should be considered. Post-marketing surveillance/reporting must be robust and timely, with swift action for any identified issues. Further analysis is needed to determine what type of devices are permitted through this pathway. (Example: Implantable devices may need to be handled outside this process or have additional scrutiny before they are surgically implanted.)
<p>Sec. 1121 – Expanded Access Policy as Condition of Expedited Approval</p> <ul style="list-style-type: none"> Requires manufacturers who receive a “covered investigational drug” designation to make their patient access policies to said investigational drug 	<ul style="list-style-type: none"> A “covered investigational drug” should be treated as an “investigational drug” for purposes of the Medicaid outpatient drug exclusion for investigational drugs. <u>States should retain the flexibility to decide their Medicaid coverage policies for such drugs.</u> If federal policymakers require states to cover a “covered investigational drug,” policymakers should consider the budgetary impact to the Medicaid program. An enhanced rebate for coverage of such treatments or value-based purchasing flexibility are potential ways to address these

<p><i>publicly available within 30 days of such designation.</i></p> <p>Sec. 1124 – Expanded Access Task Force</p> <ul style="list-style-type: none"> • <i>Establishes an Expanded Access Task Force to make one-time recommendations to Congress.</i> 	<p>concerns. Further, such a decision should consider the potential for states being held liable for punitive damages for adverse drug events on Medicaid beneficiaries.</p> <ul style="list-style-type: none"> • Requiring Medicaid to cover these drugs may conflict with existing state laws, rules and policies on coverage of investigational drugs, requiring substantial time and resources at the state level to come into compliance. • The interaction of this provision with the ability for manufacturers to charge patients and payers for using investigational drugs must be considered. For example, policymakers should consider the potential impacts on patients and payers and limitations on these access costs.
<p>Subtitle L, Sec. 1221 – Dormant Therapies</p> <ul style="list-style-type: none"> • <i>Creates a “Dormant Therapies” class with 15-year exclusivity for drugs that address one or more unmet medical needs, as determined by HHS</i> 	<ul style="list-style-type: none"> • A 15-year exclusivity period for potentially high-cost drugs could disrupt pharmaceutical market dynamics and place significant strain on Medicaid programs. • Medicaid relies on competition in drug classes to secure supplemental rebates and ensure access to appropriate therapies. Lengthy exclusivity periods make these objectives more difficult to achieve. • Dormant therapies approved under this provision should either be considered “investigational drugs” for Medicaid purposes, or else be eligible for enhanced Medicaid rebates, value-based purchasing flexibility, or some other alternative payment model. • Policymakers need to clarify the types of therapies intended to be captured by this provision. It is not clear whether this provision precludes other competitor brands for the same indication or if this is meant to only prohibit generics for dormant brands. • It is not clear if it is possible for a manufacturer to provide a new application for a therapy that has been off the market for a period of time and receive dormant therapy approval. If so, this may have unintended consequences and requires further analysis. • It is not clear whether this provision includes traditional drugs through NDA approval and biologics assigned to CDER (Center

	<p>for Drug Evaluation and Research) and CBER (Center for Biologics Evaluation and Research).</p>
<p>Sec. 1241 – Extended Exclusivity Period for Certain New Drug Applications and Abbreviated New Drug Applications</p> <ul style="list-style-type: none"> • <i>Extends exclusivity by 2 years for drugs which make “significant improvements” to existing molecules</i> • <i>Includes new indications, enhanced patient adherence, reduced public health risks, reduced side effects/adverse events</i> 	<ul style="list-style-type: none"> • Allowing an additional 2 years of exclusivity for drugs may delay the introduction of generics into the market and reduce overall competition, which inhibits states’ abilities to negotiate supplemental rebates. <u>This in turn has a direct impact on both federal and state Medicaid expenditures (within the pharmacy budget) and access to appropriate therapies.</u> • Consideration should be given to the number of continuous extensions granted for the same drug being manufactured with “significant improvements,” such that these extensions do not create an excessive or monopolistic exclusivity period. • This provision appears to provide additional incentives for actions and practices already underway. Line extensions of existing drugs are already common occurrences in drug development. • It is not clear how this provision interacts with the additional rebates Medicaid receives for pharmaceutical line extensions under the ACA. • It is not clear who will determine whether the drug makes a “significant improvement” – the FDA or the manufacturer? The language of this section suggests it is the latter, which would require further analysis. • It is not clear whether a drug given this status would later lose the designation if post- marketing studies show that the drug does not represent a significant improvement. If so, policymakers should consider the implications for the Medicaid (required coverage, financial impact, etc.).
<p>Sec. 1261 – Extension of Exclusivity Periods for a Drug Approved for a New Indication for a Rare Disease or Condition</p> <ul style="list-style-type: none"> • <i>Extends exclusivity by 6 months for orphan drugs</i> 	<ul style="list-style-type: none"> • Allowing an additional 6 months of exclusivity for drugs may delay the introduction of generics into the market and reduce overall competition. This has a direct impact on federal and state Medicaid expenditures, as Medicaid disproportionately covers the sickest and frailest populations. • Congress may consider modifying the 340B Drug Discount Program as it relates to orphan drugs for the ACA’s newly covered 340B entities. There is confusion as to whether 340B pricing applies to orphan drugs purchased by these entities for

	<p>treating a non-orphan condition. The resulting confusion makes it difficult for Medicaid agencies to accurately determine which drugs are eligible for Medicaid drug rebates and which are not (due to receiving the 340B price, which makes a claim ineligible for Medicaid rebates – the “duplicate discounts” or “double-dipping” issue).</p> <ul style="list-style-type: none"> • <u>Any action taken on 340B must not further complicate the program’s administration for state Medicaid agencies. We urge federal policymakers to refer to a forthcoming NAMD paper which details existing conflicts and challenges with the intersection of the Medicaid and 340B programs. This paper will also make recommendations to resolve or mitigate these issues.</u>
<p><i>Sec. 2001 – Innovative Cures Consortium</i></p> <ul style="list-style-type: none"> • <i>Creates a public-private partnership to accelerate drug discovery and development. Sunsets on September 30, 2021.</i> • <i>Membership includes NIH, FDA, CMS, 22 appointed members – 5 federal agency representatives; 8 biomedical representatives; 9 academia/research, patient, provider, health plan representatives</i> • <i>Consortium will award grants and contracts to small businesses and nonprofits to accelerate drug and device discovery, development, and delivery</i> 	<ul style="list-style-type: none"> • Legislative language should provide for a state Medicaid representative on the consortium to ensure the entity considers issues of cost and access from the state perspective. • The consortium’s grant and contract program should reflect considerations and issues unique to the Medicaid program, particularly in the delivery components of the grants and contracts. • The grant program should consider certain criteria, such as Good Manufacturing Practices (GMP), previous violations, and other factors when awarding grants to small businesses and nonprofits.

<p><i>Sec. 2021 – Medical Product Innovation Commission</i></p> <ul style="list-style-type: none"> • <i>Creates a new Commission, structured similarly to MACPAC and MedPAC, to make recommendations to Congress on drug development.</i> 	<ul style="list-style-type: none"> • Legislative language should provide for a state Medicaid representative as part of this body to address issues of cost and access from the state perspective.
<p><i>Sec. 2085 – Expanding Availability of Medicare Data</i></p> <ul style="list-style-type: none"> • <i>Sec. 2085(b)(1)(B)(ii) grants the HHS Secretary discretion to share Medicaid and/or CHIP claims data (to supplement Medicare data) with clinical data registries to support outcomes and patient safety research.</i> 	<ul style="list-style-type: none"> • Medicaid data can be variable and dependent on state program and population contexts. An insufficient understanding of the nuances of state Medicaid data can produce an inaccurate picture of a state’s Medicaid program. • States should have the opportunity to provide context for data requests made through this provision. • CMS should share part of the data collection fees under this provision with the states to support any state administrative costs in fulfilling data requests.
<p><i>Sec. 2121 – Authority for Coverage with Evidence Development for Medical Devices under the Medicare Program</i></p> <ul style="list-style-type: none"> • <i>Allows Medicare to pay for medical devices used by patients in clinical trials.</i> 	<ul style="list-style-type: none"> • Policymakers should consider the budgetary impact of this policy on Medicaid cost sharing for dually eligible beneficiaries. • This provision could require Medicaid to pay, in part, for an intervention without sufficient evidence for normal coverage under the Medicaid program.
<p><i>Sec. 2141 – Regulation of Combination Products by FDA</i></p>	<ul style="list-style-type: none"> • Combination products potentially pose a difficult reimbursement issue for Medicaid programs. For example, the device component of the product may not need to be replaced as often as the drug component needs to be refilled, but a product which packages these components together requires

<ul style="list-style-type: none"> • <i>Requires the FDA to issue additional guidance on the review process for products that combine drugs and devices.</i> 	<p>states to replenish both. This scenario does not comport with Medicaid’s statutory mission to operate with efficiency and economy.</p> <ul style="list-style-type: none"> • Combination products also pose potential challenges for coordination across Medicaid medical and pharmacy benefits. • It would be helpful to clarify whether FDA will approve combination drug/device products as a drug vs a device. These situations have different implications for Medicaid expenditures and state budgets. FDA’s approval pathway may also impact coverage determinations for “drug-only” programs, such as AIDS Drug Assistance Programs (ADAP).
<p><i>Sec. 4181 – Advancing Telehealth Opportunities in Medicare</i></p> <ul style="list-style-type: none"> • <i>Requires HHS to develop, within 4 years, a Medicare coverage and payment methodology for telemedicine services that is equivalent to face-to-face service coverage and reimbursement.</i> • <i>Applicable services will be selected by the HHS.</i> • <i>HHS may waive originating site, geographic, and/or health provider limitations in this methodology.</i> 	<ul style="list-style-type: none"> • Policymakers should consider the impact on Medicaid’s provision of cost-sharing for Medicare-Medicaid enrollees for these services.
<p><i>Sec. 4281 – Establishing PDP Safety Program to Prevent Fraud and Abuse in Medicare Prescription Drug Plans</i></p> <ul style="list-style-type: none"> • <i>Creates a pharmacy lock-in program for Part D</i> 	<ul style="list-style-type: none"> • States are supportive of the Medicare Part D lock-in provision. Most states already have some type of lock-in program for Medicaid beneficiaries prescribed controlled substances or where there may be other patient safety or program integrity concerns. A comparable requirement on the Part D side could help bring consistency across the programs particularly as it pertains to Medicare-Medicaid enrollees, improve patient care

<p><i>beneficiaries prescribed controlled substances.</i></p> <ul style="list-style-type: none"> • <i>Allows Part D plans to suspend pharmacy payments pending investigation of credible allegations of fraud.</i> <p><i>Sec. 4284</i></p> <ul style="list-style-type: none"> • <i>Requires e-prescribing of covered controlled substances.</i> 	<p>and safety, and prevent inappropriate use. Lock-in programs are helpful for clinical coordination even in the absence of fraud and abuse. Provisions to enhance coordination between the proposed Part D lock-in program and existing Medicaid lock-in programs should be considered.</p> <ul style="list-style-type: none"> • Policymakers should consider the distinction between the federal definitions of controlled substances versus state definitions, the latter of which may be stricter. • Provider and pharmacy readiness to meet the e-prescribing provision must be considered. As there is substantial overlap between Medicare and Medicaid providers and pharmacies, Medicaid will be impacted by this requirement and may have to provide education and support to comply with it. Policymakers should consider how to support states in this work, including incorporating state prescription drug monitoring programs (PDMPs) into the e-prescribing requirement. • Policymakers should consider what occurs if pharmacies are unable or unwilling to accept e-prescriptions. Pharmacies are the primary bearers of transaction costs in an e-prescribing environment. Non-participation could seriously disrupt access to medications for Medicare-Medicaid enrollees, which are among Medicaid’s most vulnerable populations.
<p><i>Sec. 5001 – Extension of Exclusivity Period for American-Manufactured Generic Drugs and Biosimilars</i></p> <ul style="list-style-type: none"> • <i>Placeholder section will define “American manufactured drug” for purposes of exclusivity</i> • <i>Provides designated “American-manufactured” generics or biosimilars an as-yet-unspecified exclusivity extension.</i> 	<ul style="list-style-type: none"> • Though currently vague, this provision could delay introduction of additional generics and biosimilars to the market, which may otherwise help to maximize Medicaid expenditures. • Policymakers should consider additional Medicaid rebates, enhanced FMAP or other policy solutions to address the financial impact to the Medicaid program from extended exclusivity.





NATIONAL COALITION
FOR CANCER SURVIVORSHIP

The power of survivorship. The promise of quality care.

March 27, 2015

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

The Honorable Diana DeGette
United States House of Representatives
Washington, DC 20515

Dear Chairman Upton and Representative DeGette:

The National Coalition for Cancer Survivorship is dedicated to improving the quality of care and quality of life for survivors of all forms of cancer. We focus our public policy on activities that will encourage the delivery of the right treatment to the right person at the right time.

We have evaluated the 21st Century Cures discussion draft dated January 2015 for its potential impact on the delivery of patient-centered care, and we offer recommendations based on that review.

Precision Medicine

In the Energy & Commerce Committee fact-finding process and in the discussion draft, there is a great emphasis on targeted therapies, or precision medicine. We understand and generally support efforts to make the biomedical research and therapeutic development program of this country and the health care delivery system ready for the precision medicine revolution. However, we also urge that the 21st Century Cures effort reflect a goal of ensuring delivery of appropriate treatment to each patient, based on a shared decision-making process and even if the treatment is not targeted according to the patient's genetic profile.

Food and Drug Administration Review

We understand the desire to accelerate Food and Drug Administration (FDA) review so that patients receive promising new treatments at the earliest possible time. Cancer patients who have no viable treatment options remaining or who had few options at the time of diagnosis certainly hope for FDA review that eliminates all inefficiencies. However, patients also need the reassurance that drugs approved by FDA are in fact safe and effective. Speed of review is not meaningful if the drugs that are approved do not provide a meaningful benefit to patients.

Balancing speed of review and the quality and quantity of data required for approval is difficult, and we commend the committee for giving serious thought to this issue. We recommend that the committee evaluate the work of the Office of Hematology and Oncology Products for insights into the effective new of the expedited review programs, including breakthrough therapy designation, fast track, priority review, and accelerated approval. Through discriminating use of these programs, the cancer drug review office has achieved an impressive level of efficiency that might be replicated by other review offices. Because we have observed the

accomplishments related to cancer drug review – including approval of many products well in advance of their user fee dates – we are not persuaded that fundamental revisions of review processes or changes in evidence required for approval are necessary.

We do not favor the elimination of confirmatory trial requirements for those products that receive accelerated approval. Neither do we support approvals – even supplemental new drug approvals – on the basis of data summaries. Efficient review of cancer drugs is being accomplished through solid utilization of the expedited review processes, and reducing the amount of data necessary to support approval is neither necessary nor in the interest of patients who should be able to trust the safety and efficacy of new products and to have adequate data about the drugs to support informed decision-making about their treatments.

Challenges of Reviewing Drugs of the 21st Century

We anticipate that FDA will soon require more reviewers and reviewers who are well-trained to consider genetically targeted therapies. Part of the training of personnel is the ability to attend scientific and medical meetings sponsored by a wide range of organizations, including academic institutions, professional societies, research foundations, patient advocacy organizations, and regulated industries.

A staff of adequate size that is appropriately trained will be achieved only with some changes in personnel, training, and travel and meeting attendance rules. We recommend simplification of personnel procedures to reduce the length of time required to hire new reviewers. We also urge an evaluation of conflict of interest rules to ensure they protect against inappropriate conflicts but do not unreasonably prevent FDA staffers from participation in science meetings. There should also be adequate FDA resources to support necessary travel to science meetings. We stress the interaction of FDA reviewers with the scientific leaders in their field, as we consider that a critical part of continuing medical education for reviewers who will be evaluating targeted, or precision, medicines and all other products submitted to the agency.

Improving Health Care Payment and Delivery to Ensure Quality Cancer Care

We appreciate that the committee focused much of its attention on the research and development of new therapies. We recommend additional efforts to ensure that patients of the 21st century have access to new treatments in a health care system that is affordable, sustainable, and patient-centered.

To achieve the goal of the right medicine for the right patient at the right time in an age of targeted therapies, we strongly recommend that the cancer care experience begin with a cancer care planning encounter between patient and physician. The cancer care plan should facilitate and encourage shared decision-making. These elements of care will be especially critical in an age of precision medicine, when appropriate diagnosis, including genetic profiling, will be necessary to match patient and drug. In addition, patients need complete information about the benefits and risks, including treatment side effects and late and long-term effects, of all treatment options.

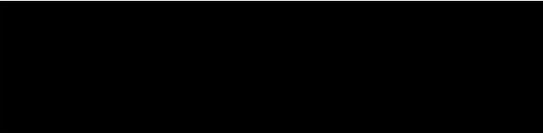
NCCS has consistently recommended that payment systems, whether fee-for-service or alternative systems like the proposed Oncology Care Model, provide appropriate reimbursement for a cancer care planning/shared decision-making service provided by cancer care professionals. We also recommend continuing medical education for health professionals to improve their communication skills around the topic of treatment decisions and to enhance their interactions with their patients.

One of the goals of the 21st Century Cures effort has been eliminating barriers to treatments of the 21st century. We urge that this include eliminating all barriers to full and open communication with patients about their treatment options. If patients participate with their health care providers in the consideration of all treatment options, evaluation of the benefits and risks associated with all treatments, and evaluation of their own genetic profile and the appropriateness of targeted therapies, they will have made significant progress toward an assurance that they will receive the right treatment at the right time.

These important patient goals will be achieved by payment systems that value the interaction between patient and physician to properly target treatment and that foster the coordination of active treatment and symptom management.

We appreciate the opportunity to comment on the 21st Century Cures initiative. We will continue to monitor the work of the committee and comment on additional questions and issues you pose for public comment.

Sincerely,



Shelley Fuld Nasso
Chief Executive Officer



**Office of the Senior
Vice President for
Research**

3181 S.W. Sam Jackson Park
Road
Mail code L335
Portland, OR 97239-3098
tel 503 494-1085
fax 503 494-1099
www.ohsu.edu/research

Daniel M. Dorsa, Ph.D.
Senior Vice President for
Research
dorsad@ohsu.edu

Dana Director, MS
Assistant Vice President for
Research Administration
director@ohsu.edu

March 25, 2015

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

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

Sent via e-mail: cures@mail.house.gov

RE: Comments on the 21st Century Cures Discussion Document

Dear Chairman Upton and Representative DeGette:

Thank you for allowing Oregon Health & Science University (OHSU) to provide comments on the discussion document and its summary distributed by the Chairman on January 27, 2015 under the 21st Century Cures Initiative. OHSU has very much appreciated the opportunity to engage in past roundtables and dialogue with committee staff. We continue to applaud the committee's interest in speeding the delivery of lifesaving treatments to patients and making the pipeline of discovery more efficient. OHSU shares this important goal.

As Oregon's only academic health center, OHSU provides an uncommon array of services from providing the state's most comprehensive health care, to educating the next generation of clinicians and biomedical researchers, to achieving breakthroughs and innovations. Its hospitals and clinics serve more than a quarter of a million patients every year with innovative care and treatment models based on the latest knowledge available. OHSU's breakthrough research leads to new cures, new standards of care, and a better understanding of the basic science that drives biomedical discovery. Of the \$355.88 million in research funding received in fiscal year 2014, OHSU received \$231.8 million from the National Institutes of Health (NIH).

Given the proposal is in draft form, we hope the proposal's final provisions will be consistent with the frame of the initiative - "Discovery, Development and Delivery" - and continue to touch on all phases of the research and development pipeline - from basic and applied research, to Food and Drug Administration (FDA) review, to coverage and access. Attached please find OHSU's specific comments organized by the discussion document's section by section summary.

Many of the draft's provisions attempt to bridge knowledge and encourage collaboration between federal health agencies, particularly the NIH and the FDA. We support efforts to break down silos that impede progress. In particular, OHSU appreciates the committee's recognition of the importance of reducing the administrative burden on researchers, including streamlining the institutional

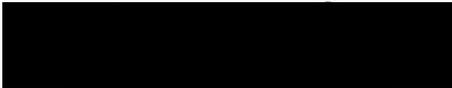
review board process for clinical trials conducted at multiple sites and streamlining the grant process for researchers. OHSU urges the Committee to coordinate these activities with those taking place by the NIH and the National Academy of Science, who has a current committee studying the impacts of Federal regulations and reporting requirements on institutions of higher education.

OHSU also commends the committee for its provisions to: foster data sharing; reinvigorate the pipeline for young investigators; and explore strategies to accelerate the pace in which therapeutics are approved. As the legislation provides expedited pathways for approval of devices, therapeutics, etc., FDA oversight of these products, including meaningful post market safety monitoring will be critical.

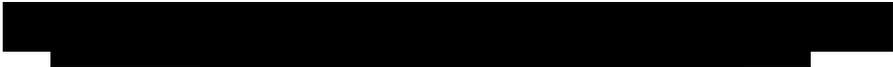
Last, as this bill adds responsibilities to the NIH and the FDA, we recommend that these agencies receive the additional resources necessary to carry out such tasks and responsibilities. OHSU strongly believes NIH funding for the bill's added responsibilities should not come at the expense of other NIH research programs. While we understand the committee is not responsible for NIH and FDA appropriations, the committee can authorize additional funding for these agencies. OHSU urges the committee to address authorization levels that reflect the unprecedented scientific opportunities and pressing health needs. If we are to achieve the full potential of advances in areas such as precision medicine, neuroscience, digital health technologies, and the other emerging opportunities discussed by the Committee, it will require sustained, predictable real growth in the budget for the NIH. Only stable and robust funding for the NIH, including for discovery and the development of basic science, can fill the pipeline with ideas to translate or demonstrate in human populations. This is a crucial issue for academia and biomedical research stakeholders across the research enterprise.

We look forward to working with the 21st Century Cures team, along with patient groups, academia and industry, to boost our nation's commitment to groundbreaking research and innovation. Please don't hesitate to contact Lynne Boyle, Director, OHSU Federal Relations, at boylel@ohsu.edu or 202-256-5070 should you need additional information or have questions regarding our comments.

Sincerely,



Daniel M. Dorsa, PhD
Senior Vice President for Research



Mark A. Richardson, MD, MScB, MBA
Dean, School of Medicine

TITLE I—PUTTING PATIENTS FIRST BY INCORPORATING THEIR PERSPECTIVES INTO THE REGULATORY PROCESS AND ADDRESSING UNMET NEEDS

SUBTITLE A—PATIENT FOCUSED DRUG DEVELOPMENT

This provision (Section 1001), led by Health Subcommittee Chairman Joe Pitts (R-PA) and Rep. Cathy McMorris Rodgers (R-WA), would build off of the Patient Focused Drug Development program at the Food and Drug Administration (FDA). Because no one understands a particular condition or disease better than patients living with it, FDA would be required to establish a structured framework for the meaningful incorporation of patient experience data into the regulatory decision-making process, including the assessment of desired benefits and tolerable risks associated with new treatments.

OHSU Comment: OHSU appreciates a more flexible approach to the rigid guidelines now in place. A more flexible approach could include allowing the development of personalized treatments based on solid biological evidence without having complete clinical trials support. This will become increasingly important as we attempt to combine multiple drugs based on information about individual tumors. While incorporating patient experience data is an important goal, OHSU cautions that such a framework be created without a lot of additional red tape, documentation requirements and bureaucracy.

SUBTITLE B—SURROGATE ENDPOINT QUALIFICATION AND UTILIZATION

This provision (Sections 1021-1024), led by Rep. Cathy McMorris Rodgers (R-WA), would establish a predictable, transparent process for FDA's consideration, and possible qualification, of surrogate endpoints. The provision also would allow FDA to use private-public partnerships to qualify other types of biomarkers.

OHSU Comment: OHSU is supportive of this direction and contends that information supporting the validity of biomarkers be made available for independent evaluation and refinement.

SUBTITLE C—APPROVAL OF BREAKTHROUGH THERAPIES

Section 1041, led by Rep. Michael C. Burgess, M.D. (R-TX), would clarify that FDA may approve a drug that has received a breakthrough therapy designation under Section 506(a) of the Federal Food, Drug, and Cosmetic Act (FDCA) when early stage clinical data provides sufficient evidence under the current safety and efficacy standards, considering the risks and benefits of the drug and the risks associated with the disease or condition for which unmet medical needs exist.

OHSU Comment: The committee should consider FDA approval under this category based on clear benefit in appropriate animal models of the disease, not only after early state clinical data. In addition, OHSU would encourage that post-market safety studies of these therapies be closely monitored.

SUBTITLE E—PRIORITY REVIEW FOR BREAKTHROUGH DEVICES

This provision (Sections 1081-1082), led by Health Subcommittee Chairman Joe Pitts (R-PA), 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. If FDA designates a medical device as such under Section 1161 and approves/clears it, Section 1162 would translate into Medicare and Medicaid transitional coverage benefits. As this policy is still under development, Section 1162 currently contains a placeholder.

OHSU Comment: OHSU is supportive of efforts that allow rapid approval of drugs and devices in ways that include careful post-market safety monitoring. We suggest that post-market data be made available for independent assessment and ensure the Secretary would still have the authority to protect the public once a clinical protocol has been agreed to.

SUBTITLE H—FACILITATING RESPONSIBLE COMMUNICATION OF SCIENTIFIC AND MEDICAL DEVELOPMENTS

FDA’s current rules and policies governing what drug and device developers may say about their own products were designed decades ago. Since then, the way that medicine is practiced and delivered and the way that information is communicated have fundamentally changed. Section 1141 includes placeholder language because the committee is working on a proposal that would clarify and rationalize these rules of the road so that scientific and medical developments can be shared with physicians, insurers, and researchers, with appropriate safeguards, in order to optimize patient care.

OHSU Comment: OHSU looks forward to reviewing this section when it is complete. It is true that the practice of medicine and the flow of information has fundamentally changed and this evolution is continuing, regardless of how information is communicated; however, it is critical that the content be accurate and unbiased and drug and device developers be held to the same standards as they are today regarding communications to the physicians and the public about their products.

SUBTITLE J—STREAMLINED DATA REVIEW

The provision (Section 1181) led by Rep. Michael C. Burgess, M.D. (R-TX), would streamline the review process for adding indications to a drug label by allowing FDA to accept and review data summaries rather than full data packages.

OHSU Comment: OHSU appreciates the desire to streamline FDA review of data and to make it easier for companies to submit data for review. However, this should not come at the expense of ensuring that data submitted is accurate and inclusive. Allowing submissions of data summaries assumes that the company preparing the submitting the data summaries are not providing a selective summary and not omitting in the summaries important information. Some safeguard against “data cherry picking” will be necessary. This could be accomplished by requiring a review of full data packages on a subset of the data included in the data summaries or releasing to qualified independent analysts the data upon its submission. The FDA still needs to be able to access to all the data so as to allow independent evaluation if desired.

SUBTITLE K—CURES ACCELERATION NETWORK

Section 1201 would provide the National Center for Advancing Translational Science (NCATS) of the National Institutes of Health (NIH) with more flexibility on the use and funding of Other Transaction Authority (OTA) so it can operate even more like the Defense Advanced Research Projects Agency (DARPA).

Section 1202 would authorize additional funds for research on repurposing drugs for new uses. One of NCATS' projects involves finding new uses for old drugs (i.e., using a drug for cancer for a rare disease). Because these old drugs have no more patent life and generics have entered the market, there is little economic reason for a brand or generic manufacturer to conduct this research. To advance the science around repurposed drugs, this provision would authorize additional funding for NCATS.

OHSU Comment: The repurposing of old drugs for new indications is a great idea. However, the funding required to test old drugs in clinical trials is substantial. We appreciate that the committee recognizes the importance of authorizing additional funding. OHSU believes that funding this section should not come at the expense of other research programs at NIH. In addition, in order for this program to be successful, quality control and peer review will be essential elements. OHSU recommends that a vetting/ranking process be described. Putting emphasis on cures rather than trials would be a significant step forward.

SUBTITLE M—NEW THERAPEUTIC ENTITIES

The New Therapeutic Entities Act (Section 1241), led by Rep. Gus Bilirakis (R-FL), would extend exclusivity for two years for significant improvements to existing molecules under Section 505(b)(2) of the FDCA. These improvements could include developing new delivery systems, new drug combinations, and new formulations that lead to less adverse events and increase patient benefits and adherence.

OHSU Comment: Rewarding therapies that demonstrate improvements over existing therapies is a good incentive.

SUBTITLE N—ORPHAN PRODUCT EXTENSIONS NOW

This Orphan Drug Extension Act (Section 1261), led by Reps. Gus Bilirakis (R-FL) and G.K. Butterfield (D-NC), would provide six months of additional market exclusivity for a drug if the company establishes that the drug treats a rare disease and receives a rare disease indication from the FDA on its label.

OHSU Comment—Section 1261 seems to remove the ability of the Secretary to revoke such a designation except if there is an untrue statement. One might want to allow Secretary to retain authority to review and revoke designations should they no longer meet certain criteria (ex. should disease definitions change, better treatments come along, new data shows that the data was not a good idea) not just for false statements. A note of caution about this section: as we get better at defining the characteristics of individual disease states, it could be likely that ALL diseases will be orphans.

TITLE II—BUILDING THE FOUNDATION FOR 21ST CENTURY MEDICINE, INCLUDING HELPING YOUNG SCIENTISTS

SUBTITLE A—21ST CENTURY CURES CONSORTIUM ACT

This provision (Section 2001), led by Rep. Cathy McMorris Rodgers (R-WA), would 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. It would be led by a board composed of government leaders from NIH, FDA, and CMS and leaders from medical device companies, pharmaceutical companies, academic research institutions, patient groups, health plans, and others. While this Consortium is broader in scope, it is based on the success of the European Union's Innovative Medicines Initiative.

OHSU Comment: OHSU believes public input will be critical to the authority, goals and accomplishments of such a consortium.

SUBTITLE B—MEDICAL PRODUCT INNOVATION ADVISORY COMMISSION

This provision (Section 2021) would create the Medical Product Innovation Advisory Commission. This Commission, which is based on MedPAC, would advise Congress on issues related to the discovery-development-delivery cycle.

OHSU Comment: OHSU is supportive of this concept.

SUBTITLE C—REGENERATIVE MEDICINE

This provision (Section 2041) would require FDA to update its guidance on surrogate and intermediate endpoints for the accelerated approval of regenerative medicine products.

OHSU Comment: OHSU believes this would be a very important boost for gene therapy as currently any change in vector design, however minor, necessitates a completely new review process, increasing cost and delaying the therapy.

SUBTITLE D – GENETICALLY TARGETED PLATFORM TECHNOLOGIES FOR RARE DISEASES

This provision (Section 2051) would clarify the accelerated approval pathway to enable FDA to rely on data from products that utilize similar genetically targeted platform technology.

OHSU Comment: OHSU is supportive of this concept.

SUBTITLE E—SENSIBLE OVERSIGHT FOR TECHNOLOGY WHICH ADVANCES REGULATORY EFFICIENCY (SOFTWARE)

This provision (Sections 2061-2063), includes language from the recently released discussion draft based on H.R. 3303, the SOFTWARE Act, which was introduced by Full Committee Vice Chair Marsha Blackburn (R-TN), Health Subcommittee Ranking Member Gene Green (D-TX) and Reps. Greg Walden (R-OR), Diana DeGette (D-CO), and G.K. Butterfield (D-NC). The language would help provide regulatory certainty for those developing apps and health information technologies.

OHSU Comment: OHSU is concerned that there could be some opportunity for abuse of such health software designation and supports continued oversight of this activity.

SUBTITLE F—BUILDING A 21ST CENTURY DATA SHARING FRAMEWORK

These sections (Sections 2081, 2082, 2085, 2086, 2087, 2088, 2091, and 2092), led by Reps. Morgan Griffith (R-VA), Leonard Lance (R-NJ), and Larry Bucshon, M.D. (R-IN), would establish a data sharing framework to enable (1) patients and physicians to better identify ongoing clinical trials, thereby increasing opportunities for patients in need of a treatment, (2) researchers and developers to use Medicare data for the purposes of improving the quality of patient care, and (3) a process for Congress to address other issues identified by the President’s Council of Advisors on Science and Technology so that data can continue to fuel all areas of the 21st Century Cures cycle.

OHSU Comment: OHSU very much appreciates the goals of this section. Improving patient participation in clinical trials is key to advancing discoveries. OHSU would like to encourage the development of national clinical metadata standards, which is currently lacking.

Regarding section 2085, one might take into account qualified family members and dependents.

Regarding provisions to strengthen privacy and security of health data used for research, it is likely that researchers could discover genomic or other defects that might be medically important to the individual patient. There may need to be some way to alert the physicians involved in a de-identified cohort that some members may have actionable abnormalities.

SUBTITLE G—UTILIZING REAL-WORLD EVIDENCE

This provision (Section 2101), led by Rep. Michael C. Burgess, M.D. (R-TX), would authorize FDA to utilize real world evidence and require FDA to issue guidance on collecting such evidence.

OHSU Comment: OHSU believes this activity would need careful oversight.

SUBTITLE J—MODERNIZING REGULATION OF DIAGNOSTICS

This provision (Section 2161) includes placeholder language.

OHSU Comment: OHSU looks forward to reviewing the language as OHSU is currently is concerned with the FDA’s approach to regulate laboratory-developed tests.

SUBTITLE K—INTEROPERABILITY

This provision (Section 2181) includes placeholder language as Rep. Michael C. Burgess, M.D. (R-TX) continues to work toward the goal of a national interoperable health information infrastructure.

OHSU Comment: OHSU looks forward to reviewing the language in this section when it becomes available. Interoperability is critical to improving patient care and for advancing research. Currently, scientists cannot easily create a database using data from the various commercial electronic medical

records (EMR) systems and there is little incentive for the companies making EMR to make it easy to transfer data from their system to any other system. Currently, EMRs are difficult to search, both within systems and between them. For example, EPIC, OHSU's EMR and dominant in the U.S., has limited search capability and would require extensive programmer time to create the means of searching EPIC for relevant data. So the data on patients being collected via EMR is literally locked away. EMRs should be required to make it easier to extract a range of data, with appropriate patient privacy contingencies.

SUBTITLE L—NIH – FEDERAL DATA SHARING

This provision (Section 2201), led by Health Subcommittee Chairman Joe Pitts (R-PA), would require those receiving NIH grants to share their data, subject to confidentiality and trade secret protections.

OHSU Comment: OHSU supports this idea but only if accompanied by funding to make the data accessible (otherwise such a requirement would be an additional cost or unfunded mandate to institutions.) The bill language does not address how long the institution would need to make the data available and such a determination would most certainly impact the costs tied to this provision. In addition, doing this correctly would require substantial work to get the data into a form where it is shareable. As mentioned above, OHSU suggests that provisions be included to stimulate the development of national clinical metadata standards. Without such standards, Section 2201 would not be useful.

SUBTITLE M—ACCESSING, SHARING, AND USING HEALTH DATA FOR RESEARCH PURPOSES

Section 2221 would unlock the research potential of data siloed in health care facilities across the country and enable patients who want to play a more proactive role in finding better treatments or a cure for their disease to do so in a responsible manner that continues to protect their privacy.

OHSU Comment: OHSU is supportive of the clarification of the definition of “Health Care Operations” to include research activity and the change in the ability to share with other entities with changes in 45 CFR 164.506(c)(4). This will ease and speed the process of data sharing for research purposes particularly when large databases include patient information from multiple entities.

The other changes in the section also will ease the burden of performing research while still protecting patient confidentiality by using the IRB/Privacy Board process.

SUBTITLE N—21ST CENTURY CHRONIC DISEASE INITIATIVE ACT

This provision (Section 2241) would require the Secretary of Health and Human Services (HHS) to develop a plan to carry out a longitudinal study designed to improve the outcomes of patients with chronic disease.

OHSU Comment: OHSU believes this is an extremely important issue as chronic diseases affect millions of people and are a major driving factor in the cost of health care. There are many successful models available for providing longitudinal care for a variety of chronic diseases. In addition, our health care system still financially rewards the provision of high-tech acute care, but not excellent longitudinal care

of chronic illnesses. We encourage the committee to expand reimbursement policy in this area by investigating models of care that already exist for chronic illnesses and find a way to provide coverage for these systems of care.

SUBTITLE O—HELPING YOUNG EMERGING SCIENTISTS

These sections (2261-2262), authored by Rep. Andy Harris (R-MD), would establish a program at NIH to help young emerging scientists.

OHSU Comment: We appreciate that the committee recognizes the importance of developing the pipeline of young emerging scientists. The timing of this provision is key in order to prevent a shortage of scientists in 10 to 30 years when senior scientists retire. Current NIH programs to help early career scientists, such as NIH career development programs (K99, K01, K08 and K23) are critical to launching the careers of young scientists and OHSU encourages increased funding for those programs and allowing for increased salary levels. Because young scientists have difficulty obtaining their first R01 and a second R01, OHSU supports increased funding levels for R01s, which remain at historic lows. Declining research funding, the limited number of faculty positions, and the increasing length of training all contribute to the instability of the biomedical workforce and the rising median age at which investigators receive their first major research grant. Increased R01 funding would also benefit mid-career investigators, who having already invested in science as a career but are encountering funding challenges. We would urge the committee to consider strategies to ensure successful careers of scientists from start to finish. Last, the NIH has issued analyses of the workforce and early career scientists, raising the question as to whether Section 2262's report is necessary.

SUBTITLE P—FOSTERING HIGH-RISK, HIGH-REWARD SCIENCE

This provision (Section 2281), led by Rep. Andy Harris (R-MD), would require NIH to support projects that pursue innovative approaches to major challenges in biomedical research that are high-risk, but have the potential to lead to breakthroughs.

OHSU Comment: This is an important issue as NIH funding decision-making and methods tend to indirectly reward low-risk research because high-risk projects receive poorer scores in study sections as reviewers seek to fulfill their role of being careful stewards of research funds. Fostering high-risk, high-reward science will require funding opportunities outside of the current R-programs and reviewers that can evaluate this type of research. The NIH should seek input from successful senior scientists who have performed high-risk, high-reward research, as well as other private funding entities who focus on this model, and obtain advice about how to accomplish what this provision seeks to accomplish.

SUBTITLE Q – PRECISION MEDICINE

This provision (Section 2301) includes placeholder language.

OHSU Comment: OHSU looks forward to reviewing this section when it becomes available as OHSU is supportive of the President’s Precision Medicine initiatives, which include providing additional funds to the NIH and the FDA.

TITLE III—MODERNIZING CLINICAL TRIALS

SUBTITLE A—CLINICAL RESEARCH MODERNIZATION ACT

This provision (Section 3001-3002), led by Reps. Cathy McMorris Rodgers (R-WA) and Diana DeGette (D-CO), would help streamline the institutional review board (IRB) process, particularly for clinical trials conducted at multiple sites, by minimizing regulatory duplication and unnecessary delays.

OHSU Comment: OHSU encourages that the streamlining of IRBs, particularly for clinical trials conducted at multiple sites. OHSU strongly urges that such clinical trial modernizations be done in coordination with current efforts at the NIH, including efforts being facilitated through NIH’s NCATS and its Clinical and Translational Science Award (CTSA) program. Currently the network of CTSA institutions (of which OHSU is one) are creating: strong inter-institutional reliance agreements for IRBs; an informatics infrastructure to allow expedited patient discovery within clinical populations for research across the entire CTSA network; and expedited contracting for trials in the consortium. In addition, NIH will be providing new funding available to CTSA in order to participate in recruitment innovation centers and trial innovation centers to work as part of the CTSA network to create efficient procedures to deal with the barriers that exist in these areas.

In addition, the long-awaited proposed revision to the “Common Rule” on the oversight of federally funded research with human subjects has been drafted and is at the Office of Management and Budget (OMB) awaiting regulatory review. Given these productive efforts, we support a legislative approach that facilitates the harmonization of requirements through collaborative efforts.

SUBTITLE B—BROADER APPLICATION OF BAYESIAN STATISTICS AND ADAPTIVE TRIAL DESIGNS

This provision (Section 3021), led by Rep. Chris Collins (R-NY), would encourage the broader application of Bayesian statistics and adaptive trial designs.

OHSU Comment: This provision could be a constructive way to test new drugs for efficacy but it may have the unintended consequence of moving away from the development of databases that currently allow development or validation of predictive biomarkers.

SUBTITLE C—POST-APPROVAL STUDIES AND CLINICAL TRIALS

This provision (Section 3031), sponsored by Rep. Chris Collins (R-NY), would ensure that FDA and sponsors periodically evaluate whether post-approval studies remain scientifically warranted.

OHSU Comment: Post-approval studies aimed at assessing side effects, long term risks and long term efficacy of new drugs and devices should be not only encouraged but mandated by the FDA; these are

too often not done by the sponsors unless it is mandated by the FDA.

SUBTITLE D—PEDIATRIC RESEARCH NETWORK IMPROVEMENT

This provision (Section 3041), led by Rep. Cathy McMorris Rodgers (R-WA), would require NIH to implement the National Pediatric Research Network Act, which was established as part of the PREMIE Reauthorization Act (P.L. 113-55).

OHSU Comment: OHSU supports.

SUBTITLE E—GLOBAL PEDIATRIC CLINICAL TRIAL

This provision (Section 3061), led by Health Subcommittee Chairman Joe Pitts (R-PA), would set forth a Sense of Congress that NIH and FDA should work with European Union, industry, and others to establish a global pediatric clinical trial network.

OHSU Comment: OHSU is supportive of this concept.

TITLE IV—ACCELERATING THE DISCOVERY, DEVELOPMENT, AND DELIVERY CYCLE AND CONTINUING 21ST CENTURY INNOVATION AT NIH, FDA, CDC, AND CMS

SUBTITLE A—NATIONAL INSTITUTES OF HEALTH

Section 4001 – NIH research strategic investment plan

Section 4001, based on the work of Rep. Andy Harris (R-MD), would require NIH to issue a strategic plan.

OHSU Comment: OHSU is supportive of this concept. However, we do have concerns with statutory mandates that specify a specific percentage of funding to as this could limit NIH's ability to respond to emerging scientific opportunities or health needs.

Section 4002 – Biomedical research working group to reduce administrative burden on researchers

Section 4002, led by Rep. Andy Harris (R-MD), would establish a working group composed of NIH and stakeholders to provide recommendations on how to streamline the grant process for researchers.

OHSU Comment: OHSU applauds the committee for recognizing the importance of reducing the administrative burden on researchers. A 2012 survey by the Federal Demonstration Partnership (FDP) found that federally funded researchers spend an average of 42 percent of their research time on administrative activities such as regulatory compliance. Several federal advisory groups, including the National Science Board and the National Academies of Sciences (NAS), however, are currently addressing this issue. In 2013, Congress charged the National Academy of Sciences (NAS) with conducting “a study on the impacts of Federal regulations and reporting requirements on institutions of higher education” (Senate Report 113-71 to accompany the FY 2014 Labor HHS Appropriation), and a designated committee was appointed to carry out this charge. OHSU urges

the Committee to work in coordination with the NAS and its committee's work to better frame any regulatory changes and to adapt the framework they suggest for addressing regulatory burden. OHSU leadership currently serves on a NAS "Committee on Federal Research Regulations and Reporting Requirements: A New Framework for Research Universities in the 21st Century," we would be pleased to continue dialogue with you on this important subject.

In regard to the proposal's working group is also directed to provide recommendations on restructuring, streamlining, and simplifying grant proposal submission at NIH. Currently, NIH has three separate groups examining this very issue (the Scientific Management Review Board, the Center for Scientific Review, and the Advisory Committee to the Director). OHSU recommends the committee coordinate this section with NIH's activities.

Section 4007 – Additional Funding for NIH Common Fund

Section 4007 would authorize additional funding for the NIH Common Fund.

OHSU comment: OHSU supports an increase in authorized funding for the Common Fund, but would like to ensure that this funding take place outside of the institutes and centers' contribution to the Common Fund, i.e. an authorized increase in appropriations should not be done in budget-neutral way.

Section 4008 – Additional Funding for NIH Brain Research

Section 4008, based on the work of Rep. Tim Murphy (R-PA), would authorize funding for the NIH's BRAIN initiative.

OHSU Comment: OHSU supports the BRAIN Initiative and welcomes additional support for that initiative.

SUBTITLE B—ADVANCING RESEARCH FOR NEUROLOGICAL DISEASES

This provision (Section 4021), led by Reps. Michael C. Burgess, M.D. (R-TX) and Chris Van Hollen (D-MD), would require the Centers for Disease Control and Prevention (CDC) to set up a surveillance system for neurological diseases.

OHSU Comment: OHSU is supportive of this section as we have incomplete data on the burden of neurological diseases in the U.S.

SUBTITLE I—TELEMEDICINE

This provision (Section 4181), led by Health Subcommittee Chairman Joe Pitts (R-PA), Full Committee Ranking Member Frank Pallone (D-NJ) and Reps. Gregg Harper (R-MS), Doris Matsui (D-CA), Bill Johnson (R-OH), Peter Welch (D-VT), Greg Walden (R-OR), and Bob Latta (R-OH), would advance opportunities for telemedicine and new technologies to improve the delivery of quality health care services to Medicare beneficiaries.

OHSU Comment: We appreciate the committee’s interest in expanding telemedicine. Telemedicine and other new technologies to deliver care are now technologically feasible and affordable. OHSU would like to associate itself with the comments already submitted by the American Hospital Association. Specifically, OHSU supports removing geographic barriers (the old rural vs. urban distinction); expanding Medicare reimbursement for in-home visits (not to be confused with in-home monitoring, which CMS just approved for reimbursement.); store and forward reimbursement would be very good; and broadening the set of services covered.

SUBTITLE R—ADVANCING CARE FOR EXCEPTIONAL KIDS

This provision (Sections 4361-4362), led by Chairman Emeritus Joe Barton (R-TX) and Rep. Kathy Castor (D-FL), would establish a Medicaid and CHIP Care Coordination program for children with medically complex conditions.

OHSU Comment: OHSU supports Rep. Barton and Castor’s legislation and is working with the Children’s Hospital Association to promote such legislation.

SUBTITLE S—CONTINUING MEDICAL EDUCATION SUNSHINE EXEMPTION

This provision (Section 4381), based on H.R. 293, which was introduced by Reps. Michael C. Burgess, M.D. (R-TX) and Peter DeFazio (D-OR) would clarify that peer-reviewed journals, journal reprints, journal supplements, and medical textbooks are excluded from the reporting requirement under the Sunshine Act.

OHSU Comment: OHSU supports.

April 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
2415 7Rayburn House Office Building
Washington, DC 20515

Dear Chairman Upton and Ranking Member Pallone:

The undersigned organizations collectively advocate on behalf of millions of men and women whose chronic health conditions (cancers, Crohn's disease, bowel disorders, chronic obstructive pulmonary disease, HIV, lung conditions, multiple sclerosis, seizures, schizophrenia) and rare disorders are treated effectively with medicines that may never have been approved without Risk Evaluation and Mitigation Strategies (REMS) to ensure safe use.

We are greatly concerned about legislative proposals that are being considered that may force the sale of medicines carrying serious risks to generic marketers for clinical (bioequivalence) testing without what we feel are sufficient safeguards to prevent harmful exposure. Although our organizations recognize the value of generic drugs to patients and the medical community, we are also aware that medicines subject to REMS can cause terrible birth defects, organ damage and even death when not handled and administered with utmost care, and we believe that any generic version of these drugs should have the same rigorous safeguards as those employed by the brand name to ensure safe use.

REMS, first authorized under the Food and Drug Administration Amendments Act of 2007 (FDAAA), gave FDA authority to require REMS from manufacturers as a condition of drug approval or post-approval to mitigate risk through certain actions. Through the FDA Safety Improvement Act of 2012 (FDASIA), Congress reaffirmed the need for a rigorous REMS program to prevent life-threatening complications, severe allergic reactions and serious infections resulting from the inappropriate use or handling of higher risk drugs.

Recognizing that REMS drugs are a unique set of important medicines, FDA streamlined the REMS program to concentrate on mitigating the risks of only the most potentially dangerous drugs. The result is that today, REMS programs are rare and only authorized when necessary to protect patients from potentially severe adverse events. Currently, only 71 medications have authorized unique REMS programs in place, while six more products exist in shared REMS systems. Less than half of these medicines (34) are subject to the more restrictive "Elements to Assure Safe Use" (ETASU) and an even smaller number require restricted distribution systems to meet the terms of these REMS programs.

Based on the current REMS safety protocols, nearly a dozen medicines subject to REMS have gone generic, including nine subject to more strict ETASU provisions. Moreover, a growing number of "abbreviated new drug applications" for new generic medicines have been filed with FDA resulting from bioequivalence testing of drugs subject to REMS. This is due to established procedures

whereby FDA permits an innovator company to sell samples of a REMS drug for bioequivalence testing after receiving documentation from the generic manufacturer that the drug will be handled, dispensed and administered safely. With a view towards accelerating this process, FDA issued draft guidance in December 2014 clarifying the process by which a generic manufacturer may obtain a letter from FDA stating the safety protections proposed for the clinical study are comparable to those in the innovator company's REMS program.

Today, the REMS program envisioned by Congress and implemented by FDA has become an essential tool to advance patient safety, protect public health, and provide access to innovative medicines that would otherwise not be available. Therefore, it is critically important for policymakers to ensure the drug safety protections REMS makes possible are guarded closely and modified only after the most careful consideration with patient safety in mind. Accordingly, policies that would allow the forced sale of drugs known to carry high risks without required safeguards to ensure these medicines are handled and administered safely are not in the public interest and should not be implemented.

As Congress considers legislation relating to FDA matters, it is critical that REMS programs and its Elements to Assure Safe Use (ETASU), including restricted distribution systems, are considered essential drug safety mechanisms, which should not be weakened.

We appreciate your consideration of the issues raised in this letter, and look forward to working closely with the Committee on this important matter.

Sincerely,
Society for Women's Health Research (SWHR®)

Alliance for the Adoption of Innovations in Medicine
American Autoimmune Related Diseases Association
American Chronic Pain Foundation
American College of Nurse-Midwives
American College of Obstetricians and Gynecologists
American Gastroenterological Association
Aplastic Anemia & MDS International Foundation
Association of Community Cancer Centers
Association of Women's Health, Obstetric and Neonatal Nurses
Center for Lawful Access and Abuse Deterrence
Crohn's and Colitis Foundation of America
Cutaneous Lymphoma Foundation
Dupuytren Foundation
Genetic Alliance
Global Genes
HealthyWomen
International Myeloma Foundation
Lymphoma Research Foundation
Men's Health Network

Myelodysplastic Syndromes Foundation

NAMI

National Association of Nurse Practitioners in Women's Health

National Consumers League

National Multiple Sclerosis Society

Rare Disease United Foundation

RetireSafe

Society of Gastroenterological Nurses and Associates

Schizophrenia and Related Disorders Alliance of America



UCB, Inc. - 1950 Lake Park Drive - Smyrna, Georgia 30080

March 18, 2015

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

The Honorable Diana DeGette
Ranking Member
Subcommittee on Oversight and Investigations
House Energy & Commerce Committee
2368 Rayburn House Office Building
Washington, DC 20515

Dear Chairman Upton and Representative DeGette,

On behalf of UCB, Inc. ("UCB"), I would like to thank you for your remarkable leadership in introducing the 21st Century Cures Act and making it a priority for the House Energy & Commerce Committee (the "Committee") during the 114th Congress.

Like you, UCB believes that the laws governing the review and approval of drugs and devices need to be updated to keep pace with innovation so that patients can benefit as soon as possible. At UCB, we are "Inspired by Patients, Driven by Science," which is consistent with your goals for 21st Century Cures, and we are certain that your bipartisan efforts to move this legislation forward will have a lasting impact on improving the lives of patients and their families for many years to come.

As you continue the legislative drafting process and move closer to a mark-up of the bill, I want to share UCB's strong support for your vision and express UCB's desire to assist the Committee in any way possible. As you may know, UCB is a global biopharmaceutical company focused on the discovery and development of innovative medicines and solutions for people living with severe diseases of the immune system and of the central nervous system. UCB currently has more than 1800 employees in the U.S. Please consider UCB a resource and don't hesitate to call on me or my colleagues if there is anything we can do to help.

UCB will continue to work with BIO and other partners to develop consensus positions on various parts of the bill to facilitate the Committee's work. The remainder of this letter identifies specific provisions of the bill that UCB strongly supports and explores areas where we would like to engage the Committee to address areas of concern.

- 21st Century Consortium (Title II, Subtitle A)

UCB strongly supports the Committee's recognition of the importance of strong public and private partnerships to accelerate healthcare innovation. In the context of a Consortium, it is of great importance to work across all the stakeholders to clarify first the need and more importantly how the Consortium will deliver on finding ways to accelerate the discovery, development, and approval of drugs and devices that patients need. Specifically, the Consortium's comprehensive mandate potentially overlaps with existing agencies and organizations, thereby rendering the Consortium's efforts redundant and perhaps unhelpful in achieving the mission set forth in the draft legislation.

- Real World Evidence (Title II – Subtitle G)

UCB strongly favors the inclusion of real-world evidence (“RWE”) to support the approval of a drug for a new indication and to support or satisfy post-approval study requirements. UCB would like this Subtitle included in the final bill.

- Streamlined Data Review (Title 1 – Subtitle J)

UCB also strongly supports efforts to allow the FDA to accept and review data summaries rather than full data packages for the purposes of adding indications to a drug label. UCB would like to see this Subtitle included in the final bill. This approach could expedite the submission and approval of data for new indications for drugs that are already on the market. This is especially true for indications that are closely related and for which the risk/benefit has been significantly addressed within the original application and approval.

- Lowering Medicare Patients Out-of-Pocket Costs (Title IV, Subtitle K)

UCB supports this Subtitle to help Medicare beneficiaries identify the out-of-pocket costs for a particular product or treatment through the establishment of a searchable database. UCB also supports BIO's proposal to allow Medicare Part D beneficiaries to appeal for a lower-tier cost-sharing amount to be applied to a therapy placed on a plan's specialty tier, as well as the enforcement of nondiscrimination in plans subject to Essential Health Benefits with respect to cost-sharing for drugs on different formulary tiers.

- Patient-Focused Drug Development (Title 1, Subtitle A)

UCB strongly supports the Committee's commitment to creating a mechanism to solicit and include patient experience data into the regulatory decision-making process. UCB's primary concern with this section is that it simply doesn't happen fast enough. To that end, UCB supports efforts by the Committee to accelerate the timeframe during which the FDA must establish and implement a process to incorporate patient experience data into the structured risk-benefit assessment framework that would be created by Section 1001 of the draft bill.

In addition, because of our extensive experience and knowledge gained from working with patients, UCB would like to see the membership in workshops required by Section 1001(b)(2)(3) be expanded to include biopharma innovators, like UCB. Such input would help inform the guidance called for in this Section. However, to fully advance the Committee’s goals of including patient experience data into the regulatory decision-making process, language in the bill should require – not just suggest – that such data be included in that process. To make that happen in the most meaningful way for patients, biopharma innovators – those making the greatest investment and taking the greatest risk to find the newest and most novel treatments– should be afforded meaningful protections in the form of a “safe harbor.” That way, those companies can engage patients solely for the purpose of gathering data and feedback to inform research and development in a way that results in novel treatments that are as patient-focused as possible.

- Surrogate Endpoint Qualification (Title 1, Subtitle B)

UCB also strongly supports efforts to develop evidentiary standards to qualify surrogate endpoints for use in the drug development process. This will allow for a clear and consistent process for sponsors to follow, which in turn will expedite drug development. As with patient experience data, we would like to see this happen as soon as possible. UCB supports the provision related to the consultation with scientific experts only at the request of the requestor. UCB also supports the proposed public-private partnership to review requests for qualification of biomarkers for use other than surrogate endpoints.

- Breakthrough Therapies (Title 1, Subtitle C)

UCB is encouraged by the Committee’s commitment to facilitate the approval of breakthrough therapies. In particular, UCB strongly supports the use of a “sufficient evidence” standard, rather than the current “substantial evidence” standard, for approval. The use of the risk-benefit framework, including the voice of the patient, as part of the decision making will ensure that patients with unmet medical needs have access to new treatments as quickly as possible while mitigating the risks to patient safety.

In addition, limiting examples of “early stage clinical safety and effectiveness data” in Section 1041(g)(1)(C) to one or more phase 2 studies might be too limiting. To give the FDA more latitude in approving breakthrough therapies and getting those therapies to patients sooner, perhaps the Committee would consider referring more broadly to “one or more early phase clinical studies”, which could also include phase 1 studies that provide evidence of effectiveness.

- Expanded Access (Title 1, Subtitle G)

Expanded access programs are vital to making investigational drugs available to those in need and UCB supports additional transparency when it comes to expanded access programs. However, UCB would like to suggest that the proposed five (5) day timeframe for a sponsor to

post denials for a request for access to an investigational drug may be overly prescriptive, and perhaps the Committee would consider “a reasonable amount of time” instead.

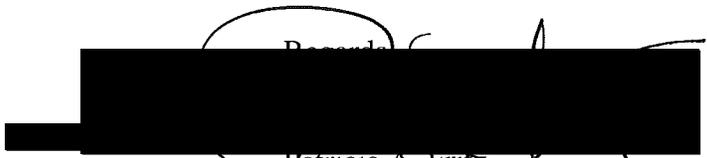
- Medical Product Innovation Advisory Commission (Title II, Subtitle B)

UCB is generally supportive of the Medical Product Innovation Advisory Commission (the “Commission”), but would like the Committee to provide more clarity as to why the Commission is needed, especially in light of the creation of the 21st Century Consortium under Subtitle B, the jurisdiction of Congressional Committees, and the authorities and responsibilities already delegated to federal agencies like the FDA and CMS. In addition, if the Commission is created, representatives from the biopharma industry should be included; industry has a remarkable depth and breadth of knowledge and expertise to share and UCB feels strongly that innovative companies like UCB should have a voice on the Commission.

- Modernizing Clinical Trials (Title III, Subtitles A, B, C, and E)

UCB is encouraged by the inclusion of Title III and generally supports all efforts to modernize and accelerate the clinical trial process, although UCB may want to meet with Committee staff to discuss some particulars of this Title.

Thank you again for your leadership in introducing and pressing forward with the 21st Century Cures Act. UCB stands ready to assist the Committee in any way possible and looks forward to discussing the ideas and concerns in this letter with Committee staff in the weeks and months ahead.



Patricia A. FITZ
Vice President
Corporate Affairs
UCB, Inc.