



American Hospital
Association®

800 10th Street, NW
Two CityCenter, Suite 400
Washington, DC 20001-4956
(202) 638-1100 Phone
www.aha.org

January 26, 2015

The Honorable Joseph R. Pitts
Chairman, Subcommittee on Health
Committee on Energy and Commerce
U.S. House of Representatives
2322 Rayburn House Office Building
Washington, DC 20515

Re: Telehealth Discussion Draft

Dear Chairman Pitts:

On behalf of our nearly 5,000 member hospitals, health systems and other health care organizations, and 43,000 individual members, the American Hospital Association (AHA) is pleased to provide feedback on your discussion draft of legislation on “Advancing Telehealth Opportunities in Medicare.”

Telehealth increasingly is vital to our health care delivery system. According to AHA survey data, in 2013, 52 percent of hospitals utilized telehealth and another 10 percent were beginning the process of implementing telehealth services. The AHA’s recent TrendWatch report, *The Promise of Telehealth for Hospitals, Health Systems and Their Communities* (attached), summarizes the benefits of telehealth and showcases successful hospital and health system efforts. It also speaks to barriers to greater use of telehealth.

The AHA applauds you and the other members of the Energy and Commerce Committee telehealth working group for recognizing the need to modernize Medicare’s approach to telehealth and seeking stakeholder comment on this issue. Our comments below set forth the limits of current Medicare policies, and respond to the general approach put forward in the discussion draft. The AHA looks forward to continued, and more detailed, discussions on this important topic in the coming months.

CURRENT MEDICARE POLICY

Despite recent expansions in covered services, Medicare is behind the private sector and many state Medicaid programs in promoting telehealth. For example, at least 20 states across the nation require private payers to pay the same amount for all medical services, whether delivered via telehealth or through an in-person encounter. In addition, many state Medicaid programs have more progressive policies than the Medicare program (TrendWatch, p. 8-9). Even within

Medicare, Medicare Advantage plans are beginning to provide telehealth benefits that are not covered under Medicare fee-for-service (FFS) rules, leaving the 70 percent of those utilizing FFS with limited access to these technological advances.

In order to modernize Medicare coverage and payment for telehealth, several statutory restrictions must be addressed:

- **Geographic restrictions.** By statute, Medicare will only pay for telehealth services that are provided to patients receiving care from a facility located in rural Health Professional Shortage Areas, a county that is not included in a Metropolitan Statistical Area (MSA), or in a rural Census tract. However, we know that urban areas (particularly inner cities) can also suffer physician shortages, and access to certain specialties (such as psychiatry) can be limited in all geographic areas. Further, the almost ubiquitous use of communications technology in American life today has created growing consumer expectations that, where safe and appropriate, health care services also can be accessed remotely, regardless of where the individual is located. Indeed, recent studies have shown that 74 percent of U.S. consumers would use telehealth services, and 70 percent are comfortable communicating with their health care providers via text, email or video in lieu of seeing them in person (TrendWatch, p. 1).
- **Covered services.** Medicare provides coverage only for a small, defined set of services, such as consultation, office visits, pharmacological management, and individual and group diabetes self-management training services. Many of these services were listed in the authorizing legislation, while others were added by the Department of Health and Human Services (HHS). In 2015, only 75 individual service codes out of more than 10,000 physician services covered through the Medicare physician fee schedule are approved for payment when delivered via telehealth. This constrained list stands in stark contrast to the private payers operating in telehealth parity states.
- **Patient location (originating site).** Telehealth services will be covered only if the beneficiary is seen at an originating site listed in law, such as a hospital, skilled nursing facility or physician office. As our nation's telecommunications infrastructure grows, however, it will become increasingly possible to safely provide care to patients in other settings, including, potentially, the office, school or home.
- **Approved technologies.** Medicare may only cover telehealth services that are furnished via a real-time video-and-voice telecommunications system. Outside of Hawaii and Alaska, Medicare may not pay for telehealth services provided via store-and-forward technologies. And, despite growing evidence of the benefits of remote monitoring technologies for quality of care and cost savings (TrendWatch, p. 3), they are not included in Medicare's telehealth policy.

COMMENTS ON THE DISCUSSION DRAFT

The discussion draft addressed some, but not all, of the constraints noted above. While the proposed mechanism for improving Medicare's telehealth policy is a step in the right direction, we believe more needs to be done. Specifically, it calls for HHS to implement a methodology that would expand the list of telehealth services covered, and remove geographic or other restrictions for those services, but only if the Centers for Medicare & Medicaid Services actuary certifies that adding those services would not add expenses to the program. Our concerns with the approach are outlined below.

Medicare already deploys a service-by-service consideration for telehealth that results in a "positive list" of covered services. It has proven to be a cumbersome approach that results in limited coverage. Given the rate of change in technology, and particularly in the use of technology in health care, a more nimble approach would be preferred. For example, other aspects of Medicare policy, such as payment for services delivered in ambulatory surgical centers, use a "negative list" that identifies those things that cannot safely be delivered in that setting and will not be covered.

In addition, removing geographic and other restrictions only for certain services creates real operational challenges. For example, it would be very challenging to establish a telehealth program to address access problems in the inner city if only certain services are covered. This approach also could be very confusing for Medicare beneficiaries. Why would they be able to benefit from the convenience of telehealth for one service, but not another?

Unfortunately, the proposal does not address the technology limitations of the current Medicare program. It does not address payment for remote monitoring, which is increasingly common and is demonstrated to provide significant benefits for patients. For example, Geisinger Health Plan (GHP) implemented a remote monitoring program for individuals at risk of heart failure, which led to significant reductions in admissions, readmissions and cost of care (TrendWatch, p. 10).

We also are concerned that the requirement for the Medicare actuary to certify telehealth cost neutrality for specific services would be hard for HHS to operationalize, and would add a time-consuming step when technology is advancing at a rapid pace. There is a growing body of evidence that telehealth improves care and saves money (TrendWatch, p. 4-5). Indeed, a recent actuarial study based on actual experience in the private sector projects significant savings for Medicare if it expanded reimbursement (Assessment of the Feasibility and Cost of Replacing In-Person Care with Acute Care Telehealth Services by Dale Yamamoto, December 2014; www.connectwithcare.org).

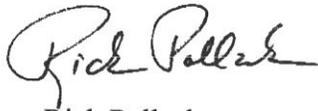
Finally, we support the section of the discussion draft that encourages the provision of telehealth services in demonstration projects and models under the Center for Medicare and Medicaid Innovation (CMMI) by waiving the current limitations on what qualifies to be an originating site and the geographic location of such sites, as well as the type of provider who may furnish telehealth services. However, it should be made clear that the providers of telehealth services under CMMI demonstration projects and models should be adequately reimbursed for provision of those services.

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In conclusion, the AHA strongly agrees with your goal of expanding coverage of telehealth services in Medicare, and appreciates the specification of a mechanism for doing so. However, given the growing body of evidence that telehealth increases quality, improves patient satisfaction and reduces costs, we believe a more global approach to expanding Medicare coverage of telehealth is warranted. The AHA greatly appreciates the opportunity to provide input and looks forward to continued discussion of this important policy issue.

If you have any questions or need further information, please contact Kristina Weger (202) 626-2369 or kweger@aha.org.

Sincerely,

A handwritten signature in black ink that reads "Rick Pollack". The signature is written in a cursive, flowing style.

Rick Pollack
Executive Vice President

February 23, 2015

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

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

Dear Chairman Upton and Representative DeGette:

On behalf of the physician and medical student members of the American Medical Association (AMA), I appreciate your leadership and comprehensive approach to identifying legislative proposals that would accelerate the discovery, development, and delivery of new cures. The AMA welcomes the opportunity to comment on this initial draft of the “21st Century Cures Act” (Cures). Physicians, along with patients, are at the forefront of a fundamental transformation in healthcare resulting from the intersection of genetic and genomic breakthroughs, the rapid growth of digital capabilities, and the resultant new tools for patients and physicians. Leveraging these new capabilities will require new pathways for research where patients and physicians have a greater role as part of a learning health care environment, strategic modernization of regulatory oversight, coverage and payment flexibilities, and, critical to all the foregoing, development of a workable, interoperable data sharing infrastructure. In our prior comments to the Committee, the AMA outlined needed reforms in five areas that directly impact physicians’ ability to deliver high quality care to patients in this new environment: 1) electronic health records (EHRs) and 21st Century technology; 2) telemedicine; 3) personalized medicine and laboratory developed testing services and procedures; 4) antibiotic development; and 5) protecting patient data. We appreciate that the Committee included provisions in the draft legislation that address a number of areas we outlined and include comments below on those and other provisions.

As a threshold matter, the AMA appreciates that the Committee continues to deliberate in a number of key areas of significant interest to physicians and their patients. Specifically, there remain placeholders for interoperability, precision medicine, and modernizing regulation of diagnostics. We would welcome the opportunity to meet with the Committee to discuss in greater detail our recommendations in these critical areas.

Section 2181. Interoperability

The AMA 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 inextricably linked with the ability of physicians and patients to use technologies that support effective communication and that allow them to move information seamlessly through the health care continuum. However, there are substantial barriers to making the foregoing a reality.

It is not possible to divorce the lack of an interoperable health care infrastructure from the prescriptive nature of the Meaningful Use (MU) program. The MU statute requires physicians to use certified EHRs in order to meet MU requirements. While the statute lists a discrete set of MU requirements—one of which is interoperability—the implementation of this program has resulted in a substantial expansion of the program, adding numerous and overly complex measures that have nothing to do with data exchange. Vendors must prioritize their development process to meet this unwieldy set of mandates in order to obtain certification. What this means is certified systems are created with the MU requirements as the first priority while physician client needs (and thus patient needs) are a distant second. The MU requirements are in effect a barrier to interoperability because they are taking away valuable time and resources that could be better spent addressing the key issue of interoperability.

Prior to MU, the early development of EHRs was centered on customer needs and was poised to flourish in a traditional consumer-driven marketplace. Although well intended, the heavy handed approach of the MU program is marked by regulatory overreach which is stifling innovation and is negatively impacting the adoption of new technologies. The program is excessively burdensome to vendors, physicians, and medical staff alike. In particular, the challenges physicians are experiencing with EHRs that cannot interoperate is evidenced by their low participation in the MU program and the high level of dissatisfaction with these products. Many MU requirements are tied to the assumption that EHRs are fully capable of interoperability. This is not the case, and as a result, the majority of physicians may face MU penalties. To date, many have elected to take these financial penalties rather than continue investing in systems that lack interoperability and force them to care for patients in a manner that does not improve quality or drive efficiency.

We strongly urge the Committee to consider that improving interoperability and usability of EHRs is tied to streamlining MU regulations for physicians. Specifically, the AMA urges the Committee to consider more effective approaches to the MU program and regulation of health information technology including:

- Removing the Pass-Fail Approach of the Meaningful Use Program. The most immediate action Congress can take to improve interoperability and usability of EHRs is to address the rigidity of the 100 percent pass/fail rate for the MU program. Under the current program, physicians must meet 100 percent of MU requirements to earn an incentive and avoid a penalty. In turn, vendors must certify to meet all of the MU requirements. As discussed above, this prioritizes MU measures over interoperability and usability.
- Promote interoperability. The MU incentives were predicated on significant cost savings associated with exchanging information across EHRs. Data exchanged today, however, essentially amounts to multi-page documents that cannot be easily transmitted or incorporated into the patient's chart, reducing the utility of this information. Additionally, physicians are often charged tens of thousands of dollars for costly interfaces and data exchange fees. Importantly, the information stored and exchanged in the EHR is not in a usable format for quality improvement and lacks standardized data elements, data formats, and definitions. This is a cornerstone of interoperability that must be adopted to improve outcomes and eliminate administrative cost to clinicians, hospitals, and others who have to map their data differently every time they send it to an external entity.

- Streamline EHR certification. The Centers for Medicare & Medicaid Services (CMS) MU requirements and the focus of the Office of the National Coordinator for Health Information Technology (ONC) certification process should prioritize interoperability and EHR usability. The current process simply ensures that EHRs meet the MU measures without addressing if information can be exchanged, incorporated, and presented to a physician in a contextual and meaningful manner.
- Align various Medicare quality reporting programs. MU includes a separate quality reporting program. Better alignment of the Physician Quality Reporting System (PQRS) program and MU quality reporting requirements is needed. Physicians who meet the more robust PQRS quality requirements should be deemed as meeting MU. This will ensure that physicians are still reporting on quality measures to improve care and will reduce administrative burden by not having to report on quality measures twice.
- Expand current hardship exemptions. Expansion of hardships will provide more ways for certain categories of physicians who face specific obstacles to meet the MU program (e.g., physicians close to retirement where this practice investment does not make sense) can avoid penalties.

The foregoing are concrete solutions that will increase the capability of physicians and the health care system to adopt technology solutions that are the necessary prerequisite to changes in the current approaches to research, regulation, clinical practice, and insurance coverage. All of the foregoing enterprises require access to reliable, high quality data that is available along the continuum. Creating silos of information will not accelerate cures nor will it create the requisite efficiencies needed to leverage the benefits of next generation technologies.

Section 2161. Modernizing Regulation of Diagnostics

Physicians have been at the forefront of one of the greatest revolutions in medicine—the application of genetic knowledge to clinical practice. Physicians have been and continue to be at the intersection of providing patients’ medical care and advancing clinical knowledge to improve upon the current standard of care. Millions of testing procedures are performed reliably, accurately, and safely every year running the gamut of simple clinical procedures to highly complex—including certain genetic and next generation testing services. It is estimated that approximately 70 percent of clinical decisions are guided in part by clinical testing. As a result, the AMA has serious concerns that the Food and Drug Administration’s (FDA) proposal to regulate laboratory developed testing services and procedures will choke off the primary development pipeline for new diagnostics, deny patients access to treatments and cures, and compromise the nation’s public health capabilities, including diminishing our ability to detect and combat bio-threats and infectious disease outbreaks.

The AMA is not alone in these concerns. During an FDA hosted two-day meeting in January on the Agency’s proposed regulation of laboratory developed testing services, a wide array of stakeholders raised the same or similar concerns—including the association representing public health clinical laboratories and member laboratories. The latter in comments to the FDA’s docket outlined a grim reality that the FDA’s proposal would not only curtail the capacity and needed flexibilities of community laboratories that provide surge capacity during an outbreak, and sentinel network laboratories that provide detection capabilities for the public health laboratories, but every state’s public health laboratory would be hamstrung should the guidance be finalized. Furthermore, the FDA’s proposal will impose another

layer of regulation—beyond the Clinical Laboratory Improvement Amendments (CLIA) and, for many laboratories, third-party accreditors and state regulatory oversight. In addition, the FDA’s proposal involves regulation of the practice of medicine—achieved by treating physician services and procedures as devices, a questionable legal fiction.

The AMA does agree that there is a need to modernize the existing regulatory framework for laboratory developed testing services that are offered by physicians to their patients and provided in laboratories subject to CLIA, as well as the regulations for commercial diagnostics kits mass produced by manufacturers that are currently regulated by the FDA. However, the steps for achieving the foregoing include modernizing CLIA by mandating third-party accreditation of all clinical laboratories and increased transparency of documentation of laboratory clinical and analytical validation. In addition, the AMA urges Congress to confer the FDA with explicit authority to regulate direct-to-consumer tests and testing services where incorrect results could cause harm to patients and the test methodology is not transparent nor well understood (as in the case of tests that use complex and proprietary algorithms to produce results). The AMA also supports streamlining the oversight for manufacturer commercial kits subject to FDA regulation, including greater flexibilities for manufacturers to incorporate modifications.

The push to regulate laboratory developed testing services appears to be related to concerns with highly complex genetic/genomic tests. The AMA agrees that a small subset of complex genetic/genomic tests, e.g., those that use proprietary and non-transparent algorithms that do not lend themselves to review and refinement by laboratory physicians and professionals, should be subject to oversight, potentially by the FDA. The AMA supports an oversight mechanism that would ensure the analytical and clinical validity of such tests. However, the FDA’s proposed framework goes far beyond addressing those “black-box” tests, and instead subjects a massive number of laboratory developed testing services to costly and burdensome requirements that would add little or no value to the testing services but would severely disrupt their availability to patients and treating physicians. It is notable that this massive interruption in clinical practice and commitment of the FDA’s time and resources into the development of a new infrastructure will divert limited time, resources, and effort from developing and implementing a viable and agile framework to address the complex regulatory challenges posed by next generation sequencing—a technology and method that will likely overtake existing methods the Agency is attempting to regulate. This will have implications for President Obama’s Precision Medicine Initiative which will rely upon next generation sequencing along with whole genome sequencing to generate relevant breakthroughs.

Section 2301. Precision Medicine

The AMA is very interested in working with both Congress and the Obama Administration to advance a number of the broad objectives outlined to date concerning President Obama’s Precision Medicine Initiative (Initiative) including the 1 million genome project that would be led by the National Institutes of Health (NIH). The Initiative is not limited to personalized medicine (genetic and genomic testing and related tailored prevention or treatments), but contemplates novel research methods, uses of digital health, and is premised on a level of data interoperability and databases that do not currently exist. The AMA looks forward to specific language related to Section 2301. It is notable that the final Cures legislation could have a significant impact on the feasibility of the Initiative. For instance, lack of interoperability will be a serious barrier to these efforts as already outlined during a two day NIH meeting concerning the million genome project. In addition, FDA regulation of digital health and laboratory developed testing

services will have implications for the million genome project's use of such tools to advance medical knowledge and patient engagement.

Section 4181. Telemedicine

The AMA strongly supports the Committee's efforts to remove restrictions on Medicare coverage of telemedicine services that do not reflect the magnitude of technological changes since the Medicare telehealth statutory provisions were adopted. The AMA urges the Committee to reimburse for more telemedicine services as well as to promote telemedicine that supports care delivery that is patient-centered, promotes care coordination, and facilitates team-based communication. We appreciate that the framework outlined by the Committee as part of Section 4181 attempts to expand coverage, but it may add extra complexity by establishing a second coverage pathway. We urge the Committee to consider a streamlined approach that the AMA supports by including:

- provisions of H.R. 4015/S. 2000, the "SGR Repeal and Medicare Provider Payment Modernization Act of 2014," that would allow telehealth services not currently covered under Medicare to be covered services for alternative payment models (APM) and qualifying APM participants, including Pioneer Accountable Care Organizations, to promote care coordination;
- expanded access to telemedicine services under the Medicare program by removing current geographic requirements under section 1834(m) of the Social Security Act; and
- coverage of telemedicine services for dual eligible beneficiaries to the same extent as their Medicaid-only counterparts.

Furthermore, the AMA supports additional Medicare pilot programs to enable coverage of telemedicine services, including, but not limited to, store-and-forward telemedicine. Because the coverage of and payment for telemedicine services are related to the evidence in support of telemedicine, the AMA encourages additional research to develop a stronger evidence base for telemedicine. The AMA continues to regularly meet with national medical specialty societies to provide support for their efforts to expand the evidence base—this will lead to clinical practice guidelines as well as information that insurers need when making coverage determinations. The AMA opposes federal legislation that would preempt or waive licensure and medical practice laws for telemedicine encounters and strongly affirms that physicians must be licensed in the state where the patient receives services. Therefore, the AMA appreciates the Sense of Congress language included in this section and has suggested relevant modifications to the Committee to reflect the nature and scope of the Federation of State Medical Board's Interstate Compact. **We welcome the opportunity to continue working with the Committee to identify flexibilities to increase telemedicine coverage in the Medicare program.**

Sections 1061-1064. Antibiotic Development

For years, AMA has recognized that antibiotic resistance represents a serious public health threat and strongly supports the inclusion of provisions in the draft legislation that would establish important incentives and pathways to accelerate development of next generation antibiotics. The AMA has publicly supported H.R. 3742, the "Antibiotic Development to Advance Patient Treatment Act of 2013" (ADAPT), and appreciates the inclusion of similar provisions in the draft legislation. While certain

prescribed activities outlined in these provisions may need to account for FDA capacity and resources, overall there is a compelling need for these provisions and **the AMA strongly supports the inclusion of these provisions in the legislation that is ultimately introduced.**

Section 2087. Quality Activities Clarification; and Sections 3001-3002. Clinical Research Modernization Act

The AMA strongly supports efforts to clarify and modernize the quality reporting infrastructure protections and those protections related to research involving human participants. To that end, the AMA strongly supports Sections 3001-3002 that would modernizes the requirements vis-a-vis institutional review board (IRB) processes, particularly for clinical trials conducted at multiple sites. These provisions will reduce regulatory duplication and unnecessary delays that have plagued research that spans multiple sites. This is essential to increase the number of research activities that seek scale—including, for example, the President's Precision Medicine Initiative Million Genome project. Furthermore, Section 2087 provides much needed clarification that quality improvement activities are not subject to the Common Rule. This has been a source of confusion and a resource drain for national medical specialties that, as part of quality improvement activities, have established clinical data registries and are already complying with the Health Information Portability and Accountability Act (HIPAA) privacy and security requirements. When institutions insist on compliance with the Common Rule requirements when the activities are for quality improvement, it has imposed substantial and costly barriers to these essential activities that improve patient health outcomes. **Therefore, the AMA strongly supports the inclusion of these provisions in the legislation that is ultimately introduced.**

Section 2091. Commission on Data Sharing for Research and Development; and Section 2092. Recommendations for Development of Clinical Data Registries

The AMA applauds the Committee's efforts to develop an infrastructure that can support the continuum of activities (research, regulatory, quality improvement, clinical decision support, and coverage, for example) that can be facilitated by state-of-the-art clinical data registries. National medical specialty societies have led the way in the establishment of such registries to support quality improvement, development of the evidence base, and other essential activities. However, we do have a few concerns related to sections 2091(b)(2) and 2092, which create new categories of registries/registry requirements that fail to take into account existing and developing quality registries (including Qualified Clinical Data Registries (QCDR)) for quality reporting under PQRS, Medicare value-based modifier, and MU. Specialties are devoting substantial resources to create and maintain registries. Quality registries are also being used for research purposes, post-market surveillance, coverage decisions, and reimbursement, not just for quality improvement. **We would like to work with the Committee and with medical specialties to ensure that the new language is harmonious with existing registry features and requirements.**

Ensuring interoperability is another critical challenge in this space. Taking initial steps to improve the underlying data captured within the EHR and registries is a key component of moving medicine forward, but one that requires a collective effort from the medical community. These definitions should be developed through a consensus process that includes all specialties and practitioners (not just physicians) who understand the clinical context of the data elements based on the patients for whom care is provided. Semantic interoperability, syntactic interoperability, and functional standards are key to establishing the data exchange consistency needed across health information technology. Any future benefits from

alternative payment models and value-based pay are premised on registries, vendors, and payers working with medical associations to establish this level of standardization. For physicians and the research community to fully realize the full potential of data aggregation the following things must occur:

- Interoperability between registries and EHRs. There are specific formats to move data and program language to exchange data. However, not all registries are operating on the same standards. There is a need to encourage registries, such as QCDR to exchange data with EHRs through a uniform standard. CMS requires QCDRs to submit their data in one format and the CMS standards should be a sufficient starting point. It must be recognized that standards evolve over time and may be inappropriate to mandate a specific standard through legislation, especially as technology evolves.
- Clinical Data Definitions. There is a need to define clinical data definitions so any time a data element is captured/exchanged it means the same thing across registries and EHRs. There are some registries, large health systems, and third-party vendors who have begun this work. However, if every society, health system and vendor creates these standards, we still will not have a set of national standards. By requiring EHR vendors, registries and all other electronic data systems for performance measurement/evaluation and clinical decision support to use standard definitions it would facilitate “semantic” interoperability.
- Standard Formats. There is the need for the most common data elements to be standardized in a universal format. For example, date of birth can be entered as 012915 or January 1, 2015, into the EHR and/or registry. This level of variability makes it difficult to query and exchange data across systems. Here “syntactic” interoperability, like semantic interoperability, requires the establishment of standard data formats so that two exchanging systems know how the data should be formatted and incorporated.
- Functional Standards. EHR data is in an unstructured free text format. To enhance quality, a third party and/or an individual needs to scrub and clean this information to make it meaningful. For example, when a patient complains of shortness of breath, this is simply typed into the EHR, but for performance improvement you need to know exactly what the patient means by shortness of breath. Is it shortness of breath because the patient just walked a mile or due to a particular condition? The functional status types of definitions have not been widely defined because it is neither needed nor relevant for payment. To begin this work, stakeholders must start with the most universal data elements and most commonly used standards.

The AMA and national medical specialty societies are ready to assist with this task. We welcome the opportunity to work with the Committee on a grant program at the Department of Health and Human Services to launch and maintain this work within the private sector in the interest of the public good.

Section 4381. Exempting from manufacturer transparency reporting certain transfers used for educational purposes

The AMA has been a staunch advocate of transparency in the interactions between physicians and industry and inclusion of the Physician Payment Sunshine Act in the Affordable Care Act. We believe that inclusion of this provision in the final Cures legislation is needed to remedy onerous and burdensome reporting obligations imposed by CMS that have already chilled the dissemination of medical textbooks,

peer-reviewed medical reprints and journals, and to avert a similar negative impact on access to independent certified and/or accredited continuing medical education (CME). This provision would ensure that efforts to promote transparency do not undermine efforts to provide the most up-to-date peer-reviewed medical knowledge, which through timely dissemination improves the quality of care patients. **The AMA strongly supports this provision.**

Sections 4281. Medicare Part D Lock-In

The AMA has long advocated for public policy solutions that will combat prescription drug diversion, abuse, overdose and death. Supporting physician clinical decision-making at the point of care through modernized, up-to-date patient specific information on dispensed prescription medications has been a major public policy initiative that we continue to support because it is sensible, proven, and it works. The AMA is extremely concerned that a number of legislative proposals would limit clinical decision-making or prevent physicians from providing patients with necessary medical treatment and referral.

There have been a number of proposals for a Medicare lock-in program that would, for example, authorize Part D prescription drug plans (PDPs) to determine that certain patients are misusing controlled substances, and then impose coverage limits so patients could only obtain controlled substance prescriptions from one physician and have them filled at one pharmacy. In response to various iterations of the foregoing proposal, the AMA has noted that PDPs only have information about their subscribers' claims for Medicare-covered drugs; they do not know their health status, treatment plans, or diagnoses. Many problems would result from adoption of the policy. For example, hospitalized patients could be prevented from filling prescriptions provided at discharge because they were not from the designated prescriber. Patients may not be able to easily access a designated pharmacy or prescriber. Moreover, patients may be seeing more than one physician who legitimately prescribes needed controlled substances. The proposal to lock-in certain Medicare beneficiaries is not a proven strategy, could be expanded without adequate justification, is premised on the faulty assumption that insurance company decisions to lock-in patients to certain providers and/or pharmacies could actually be appealed in a timely way, and fails to account for a significant and carefully tailored set of policies that are already working in the Medicare Part D prescription drug program.

The AMA has been actively engaged with CMS, along with other stakeholder organizations representing providers and patients on Medicare Part D issues, and submits comments every year on draft guidance issued for Part D plans. For cost year 2013, CMS authorized Part D plans to implement utilization measures to address outliers in opioid analgesic prescribing/dispensing. The Medicare Part D Overutilization Monitoring System (OMS) was implemented on July 31, 2013, to help CMS ensure that sponsors have established reasonable and appropriate drug utilization management programs to assist in preventing overutilization of prescribed medications as required by regulation. This represented a second round of guidance issued to plans that began in 2011 for cost year 2012. The AMA provided comments to modify and target utilization review for outliers of opioid analgesics and emphasized the importance of communicating with prescribers where: (1) multiple prescribers were involved and may have been unaware of existing prescriptions issued by others; or (2) prescriber DEA number had been illegally used. Part D plans have been authorized since cost year 2013 to employ utilization review and directed to communicate with prescribers and, if necessary, beneficiaries prior to implementing point-of-sale edits or point of sale denials. While this places the burden on payers—Part D plan—to communicate with prescribers and pharmacies, it is an appropriate alternative to imposing substantial burdens on patients

who may be inappropriately locked-in and their health care providers who have to contend with a broken Part D appeals process that all major stakeholders agree is not functional.

Section 4281, like earlier Part D lock-in proposals, suffers from a number of infirmities that will harm patients and their access to medically necessary medication. First, this provision is overly broad and could eliminate pharmacy choice for a large number of beneficiaries. Unlike other lock-in proposals, Section 4281 would authorize PDPs to initiate lock-in without evidence that a patient is misusing, abusing, or diverting their medication, only that they have obtained coverage for medication that the plan believes has a potential for fraud or abuse. (Section 4281 does not limit PDPs to medications that are demonstrated to be diverted, abused, or misused by the Centers for Medicare & Medicaid Services, for example.) PDPs are not required to first notify prescribing physicians that the appropriateness of the prescription(s) are in question—instead PDPs are authorized to notify beneficiaries even though PDPs do not have access to the patient’s medical record. Second, this provision would permit PDPs to lock the patient into the pharmacy of the PDP’s choice. The foregoing is a glaring and obvious conflict of interest where plans are able to select pharmacies based on cost as opposed to patient accessibility. Furthermore, PDPs are not required to do anything more than what they currently do to monitor use of medications by their beneficiaries. PDPs are not required to provide any assistance to beneficiaries. These provisions are not designed to promote improved patient health outcomes nor to stop misuse, abuse, or diversion of covered Part D medication. In contrast, the OMS program includes an effective mechanism to facilitate communication between all relevant prescriber(s) and the PDP and ensures that clinical considerations are the basis of subsequent prescriptions and necessary therapeutic interventions. **The AMA strongly urges the Committee to remove this provision from the final legislation.**

Sections 2061-2063. Sensible Oversight for Technology which Advances Regulatory Efficiency

The transformation of medicine is already well underway and driven by the rapid uptake and use of digital health products and the software that supports these devices. The AMA supports efforts to increase regulatory flexibilities that are essential for innovation to occur. The AMA has generally welcomed the prodigious efforts of the FDA to update oversight and guidance in the digital health space to better reflect the appropriate balance between risk and benefits as well as the need to adopt a risk-based approach given the finite Agency resources and the looming wave of products and devices under development. We also appreciate that regulatory certainty is essential to ensure that developers understand the rules of the road and are able to forecast and plan an appropriate development pathway. It is for this reason the AMA is interested in sections 2061-2063 which would create a completely new regulatory framework. Directing the FDA to develop new regulations could delay finalization of the oversight structure for at least two to three years, potentially. **In addition, the AMA does have questions related to the risks that physicians would assume under the proposed framework under Sections 2061-2063.** These provisions also raise issues that are directly related to the Precision Medicine Initiative, and we would welcome the opportunity to discuss with the Committee.

Section 2088. Access to CMS Claims Data for Purposes of Fraud Analytics

AMA policy supports fraud prevention that is targeted and conducted by appropriate authorities. This section would allow authorized third parties to have real time access to claims data for fraud prevention. **The AMA would not support this provision since the U.S. Department of Health and Human Services Office of the Inspector General, the CMS contractors, the U.S. Department of Justice, and state Medicaid Fraud Units have access to this information and have appropriate safeguards and**

The Honorable Fred Upton
The Honorable Diana DeGette
February 23, 2015
Page 10

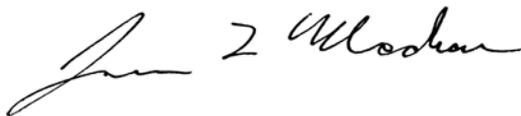
capabilities in place. Expanding access to entities without existing safeguards and less accountability to the public will only result in poorly targeted fraud efforts and other unintended consequences, such as identify theft.

Section 4241. Treatment of Global Surgery Services Rule

In the 2015 Final Medicare Physician Fee Schedule Rule, CMS finalized a policy to transition 10- and 90-day global period codes to 0-day global period codes in 2017, and 2018, respectively. Because the current CMS policy will have a wide-ranging impact on patients, physicians, hospitals, third-party payers, and Medicare, we appreciate that the Committee has included a provision that would prevent implementation of this policy. Global codes include necessary services normally furnished by a surgeon before, during, and after a surgical procedure. Global codes are classified as 0-day (typically endoscopies or some minor procedures), 10-day (typically other minor procedures with a 10-day post-operative period), or 90-day (typically major procedures with a 90-day post-operative period). Approximately 4,200 of the over 9,900 Current Procedural Terminology (CPT) codes are 10- or 90-day global codes. Despite the fact that the policy will affect 10-day global codes in 2017 and 90-day global codes in 2018, CMS has not yet developed a methodology for making this transition. The Agency has stated that it does not know how best to proceed. Nevertheless, CMS must begin to transition all these codes no later than February 2016. Implementation of this policy has consequences related to the objectives of the 21st Century Cures Initiative because, among other problems, it obstructs clinical registry data collection and quality improvement initiatives and will likely negatively impact patient care as it creates disincentives to follow-up care through imposition of additional co-pays. **The AMA strongly supports the inclusion of section 4241 in the bill that will be introduced.**

The AMA appreciates the opportunity to provide comments on the 21st Century Cures initiative and looks forward to working with you and the Committee to ensure the proposed policies support and promote physicians' ability to practice medicine in the innovative health care environment of the 21st Century through new technologies and cures.

Sincerely,

A handwritten signature in cursive script, appearing to read "James L. Madara".

James L. Madara, MD



American Urological Association

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Phone: 410-689-3700

Fax: 410-689-3800

Email: AUA@AUAnet.org

Websites: AUAnet.org

UrologyHealth.org

UrologicHistory.museum

February 24, 2015

The Honorable Fred Upton
House Energy & Commerce Committee
2125 Rayburn HOB
Washington, DC 20515

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

RE: 21st Century Cures Comments on January 26, 2015 Discussion Draft

Dear Chairman Upton and Representative DeGette:

The American Urological Association (AUA) appreciates the opportunity to provide comments in response to the 21st Century Cures January 26th discussion draft. The AUA, founded in 1902, is the premier professional association for the advancement of urologic patient care, and works to ensure that its more than 18,000 members are current on the latest research and practices in urology. The AUA also pursues its mission of fostering the highest standards of urologic care by providing a wide range of services—including publications, research, the Annual Meeting, continuing medical education (CME) and the formulation of health policy. As a result, we greatly appreciate your leadership to improve the discovery, development and delivery that support continued innovation in our health care system.

The AUA offers specific comments on the following provisions included in the discussion draft.

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

SUBTITLE B—SURROGATE ENDPOINT QUALIFICATION AND UTILIZATION

The AUA supports establishing a transparent process at FDA with specified timeframes for the development of evidentiary standards and the review and qualification of surrogate endpoints for broader utilization in regulatory decision-making. It is critical to support innovation in the drugs, biologicals and devices that diagnose, treat and monitor urologic patients. We support efforts to help expedite the development and approval of safe and effective drugs for unmet needs.

SUBTITLE C—APPROVAL OF BREAKTHROUGH THERAPIES SUBTITLE E—PRIORITY REVIEW FOR BREAKTHROUGH DEVICES

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SUBTITLE F—ACCELERATED APPROVAL FOR BREAKTHROUGH DEVICES

New drugs are being rapidly developed for treatment of advanced prostate cancer, but because it takes so long for these drugs to complete sufficient clinical trials to get through the FDA approval process, hundreds of patients suffer and die before they can legally be treated in this country. By adopting these breakthrough therapy and device measures in Sections C, E and F, scientific advances can more promptly be deployed to treat patients in need.

When considering new drugs as “breakthrough therapies”, the AUA would recommend that devices also be eligible for this designation. The MRI/Fusion process is a urology-specific example that could benefit from this policy change. Studies show very promising outcomes to more accurately detect and treat prostate cancer by using this new technology. Data so far suggests that using MRI-guided biopsies allows doctors to identify a larger percentage of high-grade, aggressive cancers than using conventional biopsies, potentially saving lives. A number of urologists have inquired of the AUA about their ability to utilize this technology and get reimbursed for it by the federal government. Specialized software is used to build a three-dimensional model of the MRI that can be fused with the ultrasound at the time of biopsy. Robotic spatial tracking allows doctors to align the biopsy needle guide with the MRI. Since it has not yet been approved by the FDA, the cost of this software cannot be defrayed and therefore the usage of this “breakthrough” technique is limited to research hospitals.

SUBTITLE H—FACILITATING RESPONSIBLE COMMUNICATION OF SCIENTIFIC AND MEDICAL DEVELOPMENTS

The FDA does not allow pharmaceutical, biological and medical device companies to actively distribute key clinical information, even if it is related to the on-label indication, unless it is explicitly referenced in the package insert. By limiting the sharing of information, physicians are hampered in their ability to gain all of the firm scientific rationale and sound medical evidence needed to treat patients. The AUA is **pleased to see that the committee included a placeholder** to address this issue and stands ready to work with you to clarify and rationalize these rules so that scientific and medical developments on pharmaceuticals, biologicals and medical devices can be shared with physicians, with appropriate safeguards, in order to optimize patient care. We recommend that the committee develop standards for qualifying real world data, through a public process; expand the current process of review of materials beyond what is included in the package insert to also cover other key data, such as subpopulation, pharmacoeconomic or comparative cost data; and ensure a timely review process for such information.

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



SUBTITLE A—21st CENTURY CURES CONSORTIUM ACT

The AUA supports the idea of establishing a public/private partnership to accelerate the discovery, development, and delivery of innovative cures, treatments, and preventive measures for patients in the United States. When considering the makeup of the consortium, **we would suggest the inclusion of leaders from the Department of Defense (DoD) and the Centers for Disease Control and Prevention (CDC)** to go along with officials from the National Institutes of Health, Food and Drug Administration, and Centers for Medicare & Medicaid Services. Programs like the the DoD’s Congressionally Directed Medical Research Program have long been focused on innovative research and also is a major funder of clinical trials. In addition, the DoD and CDC have tremendous experience in collaborating with existing industry partners. Finally, the association feels sunsetting the consortium in 2021 is unrealistic given the amount of time it would take to set up its framework.

SUBTITLE B—MEDICAL PRODUCT INNOVATION ADVISORY COMMISSION SEC. 2021. MEDICAL PRODUCT INNOVATION ADVISORY COMMISSION

The AUA urges you to slightly modify this provision which would create the Medical Product Innovation Advisory Commission. Similar to the Medicare Payment Advisory Commission (MedPAC), this commission will advise Congress, analyze medical product innovation in the United States and recommend policies to accelerate the discovery, development, and delivery of new medical products. We appreciate that the membership of the commission requires the participation of physicians to ensure the first-hand input of those on the front lines of patient care. However, we believe that this provision should also apply to products with indications that expand or change, and not merely apply to new products coming to market. Because it is important to continue to support innovation, the **AUA supports maintaining this provision with the suggested modification to strike “new” in the section.**

SUBTITLE D—GENETICALLY TARGETED PLATFORM TECHNOLOGIES FOR RARE DISEASES

The AUA supports acceleration of the approval pathway for applications of products for serious or life-threatening conditions that employ genetically-targeted therapeutic platform technology already in use for other applications or products. We feel that the breadth of evidence typically available from various sources, along with a determination of likelihood of clinical benefit in a patient population with a paucity of treatment options, substantiates the value of this provision, and further feel that the Secretary’s use of the “totality of the evidence” to determine approval is appropriate.

SUBTITLE E—SENSIBLE OVERSIGHT FOR TECHNOLOGY WHICH ADVANCE REGULATORY EFFICIENCY

The AUA is pleased to see the committee recognize the importance of providing regulatory



certainty for those developing applications and health information technologies by creating statutory definitions for “software,” “medical software,” “health software,” “accessory,” and “component.” **We do feel these sections must address the necessity of incorporating standardized data format to allow upload of captured information into any and all certified electronic health records (EHRs).** Otherwise, this simply becomes another barrier to interoperability.

SUBTITLE F—BUILDING A 21ST CENTURY DATA SHARING FRAMEWORK

While the AUA applauds the overall effort by the Energy & Commerce Committee to advance public discussion about the pace of cures in our country, **we are particularly pleased with the discussion draft’s focus on ways to encourage and facilitate the development and effectiveness of clinical data registries.** Much like numerous other national medical specialty societies and physician-led groups, the AUA has invested heavily and recently launched the AUA Quality (AQUA) Registry as part of its ongoing commitment to improving the quality of care for patients with urologic disease.

The discussion document includes several provisions that relate to clinical data registries; yet, as the document indicates, there is currently no statutory definition of a clinical data registry outside of the Medicare program’s definition of a Qualified Clinical Data Registry (QCDR). We suggest the committee consider adopting the following definition of clinical data registries, loosely based on the definition set forth in the registries user guide published by the Agency for Health and Research Quality (AHRQ):

A clinical data registry is an organized data collection system operated by or affiliated with a medical society, hospital association, or other health care association, that collects uniform data (clinical and other) to evaluate specified outcomes for a population defined by a particular disease, condition, or exposure, and that serves one or more pre-determined scientific, clinical, or policy purposes, including but not limited to describing the natural history of disease; determining clinical effectiveness or cost effectiveness of health care products and services; measuring or monitoring safety and harm; and/or measuring quality of care.

The AUA also echoes the sentiments of other registry stakeholders in urging the committee to add a section to the discussion document protecting clinical data registry data from legal discovery, particularly data that identifies or could identify specific patients, providers, or facilities. There is currently no adequate federal protection for such data from subpoenas or other legal discovery requests. The risk that such data may be subject to forced public disclosure creates a chilling effect on the ability of clinical data registries to recruit data sources.

PART 2—IMPROVING CLINICAL OUTCOMES FOR PATIENTS AND



PROGRAM INTEGRITY THROUGH CMS DATA

SEC. 2085(a). EXPANDING USES OF MEDICARE DATA BY QUALIFIED ENTITIES

The AUA supports allowing qualified entities to share Medicare data with individual health care providers and medical societies for quality of care improvement purposes and at no cost to such authorized users. This would allow clinical data registry stakeholders to use this information to evaluate their respective outcomes against national standards or benchmarks.

SEC. 2085(b). ACCESS TO MEDICARE DATA BY QCDRs

The AUA supports the requirement that HHS make Medicare, Medicaid, and CHIP claims data available to QCDRs, but we request that the committee broaden this provision so that it ensures access to such data for all clinical data registries. Furthermore, we are concerned that the discussion document requires the Secretary to charge a fee to cover the cost of such data. Running a registry already requires a significant investment of resources, a challenge that is heightened by the fact that many registries are run by non-profit entities. Registries should have unfettered access to federal claims data, which, when combined with more robust clinical data, can result in more accurate evaluations of quality and value performance.

SEC. 2087. HIPAA COMMON RULE EXCEPTION

The AUA appreciates the inclusion of language requiring an exception to the Common Rule for registries and other entities that collect identifiable data, but have no direct interaction with patients and comply with all applicable HIPAA regulations. Current regulations for informed consent are outdated and create unnecessary regulatory barriers that limit the ability of the AQUA registry to engage in prospective, systematic tracking of practice patterns and patient outcomes that lead to better care.

PART 3—BUILDING A 21ST CENTURY CLINICAL DATA SHARING SYSTEM

SEC. 2091. COMMISSION ON DATA SHARING FOR RESEARCH AND DEVELOPMENT

This provision would establish a Commission on Data Sharing for Research and Development. While the AUA supports efforts to ensure the integrity of clinical registry data and the need for guidelines related to the use of registries, we are concerned that overly prescriptive standards may result in a one-size-fits-all approach to registries and ignore the fluid and diverse nature of registries and the unique needs of different specialties and different patient populations. Government involvement in this issue should be restricted to setting standards that ensure an adequate infrastructure for the collection of registry data, such as ensuring that EHR vendors are interoperable with registries, protecting data privacy and security, and providing funding to promote innovative registry practices. The registry community, which is already well coalesced, should remain responsible for reaching



consensus on other standards related to how registries work.

If a commission is established for this purpose, we urge the committee to revise the language in this section to specify that the commission is advisory only; representative of relevant stakeholders, including physicians and others directly involved in registry design and implementation; and that appointments must be non-partisan and non-political (i.e., the Speaker of the House should not make these appointments; instead we recommend that the U.S. Government Accountability Office take on this task, similar to MedPAC appointments). The role of the advisory board should be to highlight best practices and potentially inform the Secretary's recommendations in Sec. 2092.

SEC. 2092. RECOMMENDATIONS FOR DEVELOPMENT AND USE OF CLINICAL REGISTRIES

The AUA appreciates many of the recommendations proposed under this section, particularly the promotion of bidirectional, interoperable exchange of information between EHRs and registries. As mentioned earlier, it is critical that the Secretary adopt and better enforce interoperability standards to ensure the seamless exchange of information between certified EHRs and qualified clinical data registries. The current language seems to put the onus on registries, while the most significant current barrier to integration of EHR data in registries is EHR vendor refusal to share data with registries or charging excessive fees for such access. We urge Congress to mandate that EHR vendors adopt interoperability standards as a condition of receiving federal certification.

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

This provision would amend the privacy-related provisions of the Health Information Technology for Economic and Clinical Health (HITECH) Act to help realize the research potential of health data currently isolated in health care facilities across the country. The AUA feels clinical researchers like our urologist-scientists would greatly benefit from this additional data sharing, especially if new safeguards are established to protect the privacy rights of individuals. **Therefore, we strongly urge the committee to maintain this provision.**

SUBTITLE N—21ST CENTURY CHRONIC DISEASE INITIATIVE ACT

There are a plethora of chronic urological diseases and conditions that our patients suffer from. As a result, the AUA supports this provision requiring the Secretary of HHS to develop a plan to carry out a longitudinal study designed to improve the outcomes of patients with chronic disease through a better understanding of risk, transition from wellness to disease, disease progression, diagnosis, and other factors related to chronic disease.



TITLE III—MODERNIZING CLINICAL TRIALS

SUBTITLE A—CLINICAL RESEARCH MODERNIZATION

SEC. 3001. PROTECTION OF HUMAN SUBJECTS IN RESEARCH; APPLICABILITY OF RULES

The AUA applauds efforts to streamline the institutional review board (IRB) process, particularly for clinical trials conducted at multiple sites. This provision is consistent with the recently released draft NIH policy on the use of a single IRB for multi-site research and **we urge the committee to maintain this provision.**

SEC. 3002. USE OF INSTITUTIONAL REVIEW BOARDS FOR REVIEW OF INVESTIGATIONAL DEVICE EXEMPTIONS

The AUA also **supports this provision** as it allows review by a centralized IRB.

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

The NIH, while of paramount importance to advancing high-quality biomedical research, has historically received criticism for lack of strategic planning in its investments in research. The AUA applauds the improvements that the NIH has made in this regard in more recent years, but additional improvements in strategic investments are greatly needed, especially in relation to research on urologic diseases, which has historically been underfunded considering the enormous impact of urologic diseases on the American people and economy. Therefore, the AUA supports this provision for more deliberative research investments by the NIH. In addition, we support the appointment of the directors of the national research institutes and centers by the National Institutes of Health director, as well as the provision that the director of each national research institute or center be required to approve each grant, provided that research on understudied diseases, which may not easily fit national research priorities, does not go unfunded.

SUBTITLE H—LOCAL AND NATIONAL COVERAGE DECISION REFORMS

The AUA supports improving the Medicare local coverage determination (LCD) process. We urge the adoption of the various review periods proposed for new or significantly revised LCDs. The association is also encouraged to see flexibility granted to Medicare Administrative Contractors (MACs) to make a revision without comment under specific conditions, including when an Administrative Law Judge ruling indicates the determination is wrong.

SUBTITLE I—TELEMEDICINE



SEC. 4181. ADVANCING TELEHEALTH OPPORTUNITIES IN MEDICARE

The AUA supports efforts to advance opportunities for telemedicine and new technologies to improve the delivery of quality health care services and improve Medicare beneficiaries' access to our physicians. We certainly agree with the sense of the Congress encouraging states to collaborate, through the use of state medical board compacts, to create common licensure requirements for providing telehealth services. This is necessary to facilitate multi-state practices and allow for urologists to provide services across state lines. The AUA, however, would urge that CMS be required to implement a methodology to cover and pay for certain telehealth services in a shorter time frame than four years after enactment. Otherwise, the R&D and capital investment necessary by urologists to perfect the processes will be impossible to recover.

SUBTITLE L—GLOBAL SURGERY SERVICES RULE

The AUA **strongly supports congressional action to permanently rescind the rule promulgated by CMS to transition all 10- and 90-day global procedures to 0-day global procedures** by 2017 and 2018 respectively. Despite this short implementation timeframe, CMS has not informed the surgical community of the methodology to be used to account for the removal of the post-operative services included in the global period. This means that all evaluation and management services will be billed separately for post-operative care, and that only the cost of the physicians work performed the day of the procedure will be reflected in the relative value units for each code.

We also are concerned with several unintended consequences of eliminating the 10- and 90-day globals. With 0-day global codes, patients will now pay copays on other services as well, including each of the follow-up visits. This could considerably increase the administrative burden on patients, or worse, discourage them from coming back for follow-up care. In addition, CMS initiatives for payment are all moving towards larger bundled payments. Deconstruction of the current payment structure for urologists and other physicians is counterintuitive to the end goal of providing more comprehensive and coordinated care for the patient. Finally, the administrative burden on surgical practices and CMS (and its contractors) will be significant. The American Medical Association estimates that eliminating the global package will result in 63 million additional claims per year to account for post-surgical evaluation and management services. Clearly, this will add unnecessary costs to the claims processing system.

SUBTITLE S—CONTINUING MEDICAL EDUCATION SUNSHINE EXEMPTION SEC. 4381. EXEMPTING FROM MANUFACTURER TRANSPARENCY REPORTING CERTAIN TRANSFERS USED FOR EDUCATIONAL PURPOSES

The AUA **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. Urologists must have



American
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access to the most up-to-date independent medical knowledge to support their delivery of high quality patient care.

The AUA appreciates this ongoing process toward the introduction of bipartisan legislation and looks forward to continuing to work with you on this initiative. Please let us know if our expertise may be of assistance, especially as you seek additional feedback or would like assistance in developing content for the placeholders.

Sincerely,

A handwritten signature in black ink, which appears to read "David F. Penson". The signature is fluid and cursive, with a long horizontal stroke at the end.

David F. Penson, MD, MPH
Chair
Public Policy Council

CC: Members of the House Energy & Commerce Committee

Karen Ignagni
President &
Chief Executive Officer



February 27, 2015

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

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

Dear Chairman Upton and Representative DeGette:

We are writing on behalf of America's Health Insurance Plans (AHIP) to offer comments regarding the 21st Century Cures initiative discussion draft released in January. This draft marks a key point in the Committee's ongoing dialogue to increase patient access to innovative treatments and cures.

We applaud your efforts to continue bringing stakeholders together to identify and address roadblocks that may hinder the discovery, development, and delivery of new treatments and cures to patients. We also strongly support efforts to encourage innovation and bring new, safe, and effective treatments to patients, particularly those with serious medical conditions or areas of unmet need.

As you and the members of the Energy & Commerce Committee review the discussion draft and explore this key subject, we will continue to advocate for policies that we believe are essential to ensuring access to affordable, quality health care. Our priority areas include:

- *Encouraging Competition and Innovation.* Congress and the FDA should seize opportunities to improve value to patients and reduce costs such as targeted incentives to true breakthrough therapies or to diseases where no treatments are available, constructing a clear pathway for approval of biosimilars, and encouraging additional market entrants – and greater competition – for expensive classes of treatments.

- *Promoting Quality for Patients.* While innovation in clinical trials is important, evidentiary standards should not be lowered solely to approve drugs more quickly. Patients benefit from pre-market clinical trials with robust evidentiary standards and post-market studies that monitor the drug or device in the broader population – both of which are critical components of ensuring quality in a value-driven health care system.
- *Ensuring Safety for Patients.* Balancing expedited approval pathways with quality and patient safety is critical. Any drug approved on a “fast track” or with alternative evidentiary standards should be: (1) coupled with requirements for post-market studies to ensure safety, and (2) enforced through penalties for failure by manufacturers to meet those requirements.
- *Promoting Transparency.* Greater transparency of clinical research and drug approval data would help physicians and patients select the optimal course of treatment. The timely availability and accessibility of clinical data from drug trials about efficacy, complications, and safety are critical to that decision-making process.

Increasing access to safe and effective treatments and cures will help millions of patients live better, healthier lives. Health plans want patients to have access to the best treatments, but new drug prices are not sustainable. An improved drug and device approval process should include provisions that drive increased transparency and greater competition that will result in lower prices for consumers.

We look forward to the ongoing dialogue as you continue your important work to ensure that patients have access to innovative, effective, and safe treatments.

Sincerely,

A handwritten signature in black ink, appearing to read "Karen Ignagni". The signature is fluid and cursive, with a prominent initial "K".

Karen Ignagni
President and CEO

**Comments on
“The 21st Century Cures Discussion Document”
from the
Arkansas Research Alliance**

We are grateful to Chairman Upton, Rep. DeGette, Chairman Pitt, and all of the others who have recognized this important national concern, assumed the leadership to address it and have put so much effort into gathering information and developing practical solutions. The portions of the document that we wish to address are primarily within the areas led by Rep. Rodgers. We are very appreciative of her hard work and leadership. We give our thanks to all of you.

Components of the problem we wish to address:

The document makes several important points about the current state of medical product assessment, including:

- 95% of drugs fail during development
- The current system discourages investment in the development of some needed therapies
- Needed treatments are not available to patients quickly enough
- There is a need to review and qualify surrogate endpoints
- Communications restrictions about new indications for approved products need review
- Although many entities in the U.S. support biomedical research, no one entity coordinates the development of a strategic research agenda nor comprehensively defines resource needs
- Health data are siloed. (We would add one clarification that, beyond patient data, this also involves research data, particularly from failed development efforts)

Does a part of the solution already exist?

We believe we offer a piece of good news that could (and we believe should) be a part of the solution to some of the problems identified in the document. Clearly, what we describe is only a portion of a very great need, but it is a piece that already exists, was originally designed to address such problems, and could be used more effectively.

Although not widely publicized, one of FDA’s centers is a world-class laboratory facility that has no regulatory responsibilities. We believe this center offers a huge opportunity to bridge a gap in regulatory science and provide solutions to problems raised in the document. It is the National Center for Toxicological Research (NCTR), **the only FDA center without a regulatory mission** and the only FDA center outside of the D.C. metropolitan area. NCTR is a multi-disciplinary leader in toxicology with 600 employees (over 150 PhD staff and post-doctoral fellows) with a strong focus on developing surrogate markers of toxicity and a very strong international reputation. We want to stress the fact that NCTR’s mission separates it from direct regulatory responsibility. We believe that point is enormously important and becomes the basis of what could be a creative approach to solving some of the issues raised in the document.

Important History:

NCTR was created to address over-arching scientific issues. Its history is both interesting and relevant to the issues before us today. The rationale for its creation came from a report from a (then Health, Education and Welfare) Commission on Pesticides and Their Relationship to Environmental Health, chaired by Dr. Emil Mrak, Chancellor of the University of California at Davis. The Commission recognized that at the time (late 1960's) the public was being exposed to an array of chemicals for which there was far too little information available about the harm caused by low-dose exposures. The Commission recommended the establishment of a **national laboratory** that would work cooperatively with industry, academia, and government to resolve these scientific issues.

In 1971, President Nixon announced the establishment of NCTR¹. The newly created Environmental Protection Agency (EPA) took administrative control and became the first funding agency for NCTR. At the time, FDA Commissioner Charles Edwards, being concerned about other chemicals in food such as cyclamates, arranged for NCTR to be transferred to FDA as a venture supported by both FDA and EPA. Joint policy direction of NCTR continued between FDA and EPA for another 10 years, after which time EPA discontinued its core support.

NCTR designed to solve major national problem:

We mention this history because it describes a vision designed to solve earlier versions of problems with many similarities to the problems of today. The same question was posed 45 years ago:

- **“How can we most effectively utilize our national resources to find answers to difficult scientific questions that affect the broad U.S. population?”** Clearly the technology has changed during that time, but a primary question remains:
- **“How can our regulators protect the public health, an important component of which is to not stifle the commercial capability nor the entrepreneurial climate that produces the products that then protect the public health?”**

It seems clear that we must use every reasonable approach to provide those regulators with the latest scientific tools, assure that they are validated by the best expertise known, and to assure their regulatory application. If that involves collaboration across sectors--industry, academia, and government, can we not find ways of doing so in a transparent, safe and equitable fashion? The absence of these needed tools is too profound and the social and economic impact too great not to find ways of harnessing the total of our scientific abilities. We believe NCTR offers an opportunity to move in this direction.

Why then, was the problem not solved with the creation of NCTR in 1971? Perhaps due to conflict of interest concerns with placement of NCTR within the regulatory structure, industry collaborations, although important to NCTR, did not develop to the extent envisioned by some. In addition, funding issues described in the following paragraph are of major importance. It was never supported at the level of a “national laboratory.” Creating a stronger ability and mandate for NCTR to collaborate with industry is an important recommendation from the Arkansas Research Alliance.

¹ “NCTR’s Noteworthy Achievements: the Early Years”: Independently produced article by Morris Cranmer, Ph.D., NCTR Director, 1971-1977

NCTR: A strong national resource:

The people who have staffed NCTR are some of the most educated, dedicated individuals available anywhere. We would ask the Committee to review the funding history of NCTR compared to other initiatives with similarly important missions. When we review the resources provided to national laboratories, we find that some of their smaller divisions have larger budgets than NCTR. If we look at some of the important and successful laboratories mentioned in this report, we note that their budgets are much larger than that of NCTR. This year, the President's budget request proposes reducing NCTR's budget from \$63 million to \$59 million. That level of funding is difficult for us to understand in light of the importance of the NCTR contribution. We contend that if NCTR and its important national role were to be reviewed as a national initiative rather than as an "incremental" budget item within FDA, and perhaps below the level of national strategic focus, it would be given more attention.

This discussion is certainly not a complaint against FDA. FDA has recognized the importance of NCTR and has provided as much support as possible over the years. As described in the report, the regulatory (and primary) mission of FDA put the Agency in the difficult position of trying to provide for those regulatory needs and then provide what it could to invest in scientific research.

A new adjustment to the NCTR mission:

NCTR's capabilities could be the basis for an important new initiative. The Center has used its resources wisely to invest in the latest equipment and technological capabilities. That, and its approach, have allowed it to recruit top level scientists. It is interesting to note that the employee turnover at NCTR is very low and morale quite high. It has also invested in creating a diverse scientific capability, covering many sub-disciplines essential to what is now needed.

From its beginning, NCTR has established itself as the place where the gold standard of animal research is conducted. The multiple levels of experimental controls, the characterization of test materials, and the attention to the care and observation of the animals is now an asset important to creating a needed surrogate validation program. NCTR does not just conduct animal testing. NCTR animal experiments utilize the full breadth of scientific capabilities to isolate the intricate mechanisms of action at the most basic biological and biochemical levels. This capability, using the latest technologies, provides insights into biomarkers that may develop relatively soon after exposure to a potentially toxic compound, but are not recognized clinically for long periods of time. We are aware that there are currently 34 biomarker projects ongoing at NCTR. The knowledge gained in this work is also critical to developing accurate *in vitro* and *in silico* assays that avoid, minimize, or complement whole animal testing. We understand how the refinement of this preclinical work is critical to the drug approval process, among other reasons, to provide participants in clinical trials the best possible basis for agreeing to exposure.

This capability is also important with respect to validation of new assays and potential surrogate markers of toxicity. With NCTR's background in this preclinical work, it has the tools necessary for validation studies of new technologies and surrogate markers. When appropriate, validation can occur using the full range of assessment tools including animal studies. Considering NCTR's long and successful relationship with the National Toxicology Program (NTP) at the National Institute for Environmental Health Sciences, the Committee may want to consider a joint NCTR/NTP program for identifying surrogate markers. Similarly, NCTR's

capabilities could be helpful to the national need for the validation of other emerging processes, *e.g.*, organ-on-a-chip.

NCTR: A platform to accelerate time to market:

Accordingly, our proposal to the Committee is that you review carefully what NCTR is capable of providing and explore options not previously implemented. For example, might NCTR be given a mandate separate from the rest of FDA to engage more often and at a more significant level with regulated industry? The Center has the authority to enter into Cooperative Research and Development Agreements (CRADAs) with industry. However, we understand that such agreements are reviewed by each of the FDA regulatory centers to avoid conflicts of interest. Might some statutory separation of NCTR from those conflict of interest concerns be possible and advisable? Might NCTR be encouraged to seek more such industry and academic agreements and might the Center be given the resources required in order to do that? With more such interactions, promising new technologies, when validated, would have an official imprimatur that would provide needed assurance to venture capitalists who need a vision of product success through the high levels of uncertainty now clouding the process. Success in this area would serve both the public health and global competition.

Consistent with the idea of working more with industry, we also mention a laboratory we understand could be one model for how to achieve more venture support. The National Characterization Laboratory (NCL), a National Cancer Institute facility at Ft. Detrick, apparently has enjoyed success in demonstrating efficacy and safety of some cancer therapies developed through nanotechnology. In so doing, the NCL has provided sufficient assurance to potential investors that product development would be a good risk. The Committee may want to review that model because if it is as we understand, we believe NCTR, with some modest changes in approach, could provide that kind of scientific support to a broader category of products.

21st Century Cures Consortium:

We applaud the recognition of need for a coordination point for assessing needs, developing strategy, and requiring accountability. NCTR has developed its programs with heavy reliance on external peer review for direction and accountability through an eminent Science Advisory Board as well as other reviews. We believe it would be a relatively easy step to incorporate NCTR into the Consortium's coordinating process. In fact, we suggest that the Committee might want to consider utilizing NCTR as a component of such a process.

Although we do not have a full recommendation as to how NCTR might relate to the Consortium, we believe it may offer a way for the Consortium to make progress quickly. The established programs at NCTR could form a base from which the Consortium could move to advance its programs. In addition to the strong laboratory-based biomedical research programs, the very strong bioinformatics program at NCTR would be useful to the Consortium in data mining and validation.

We at the Arkansas Research Alliance also want to stress the strong culture of education and training at NCTR. Not only does NCTR have a rich history of training scientists, as a part of a Memorandum of Understanding between FDA and the State of Arkansas, the University of Arkansas for Medical Sciences has developed a certificate program in Regulatory Science. The University is now working to expand the program to a Master's program and to provide it on-line. This and other programs could be used to help analyze policies and train scientists.

The culture at NCTR is rich and diverse. There is a level of enthusiasm and high energy that is difficult to describe but tangible during a visit. With that said, we believe it would be important for the Committee to visit the Center to experience directly what we are describing. We at the Arkansas Research Alliance are able to provide this information because of strong interest, periodic tours, and reading published literature. More precise information about these programs should be obtained directly from the excellent staff at NCTR. During such a visit, you would find:

- the gold standard for animal assays, establishing the world-wide standard for widely used chemicals such as Bisphenol-A. A host of chemicals have been assessed with this capability.
- a strong program to assess the safety of pediatric drugs, using a primate model and current imaging technology in conjunction with clinical programs at Arkansas Children's Hospital.
- an unparalleled capability to assess the safety and presence of nanomaterials in animal tissue and other material.
- a strong biomarkers program using the latest technologies to assess the genome, the proteome, the metabolome, the microbiome and others.
- a very strong bioinformatics and statistics program developing *in silico* assessment models, knowledge bases, and tools to handle massive volumes of experimental data. Many of these systems have been developed with industry use and validation.
- a personalized medicine program.
- many other programs to identify and assess the mechanisms behind toxic events, in genetics, cancer, skin exposures and other disciplines, much more than can be quickly summarized.
- an energetic workforce with a very positive attitude.

In conclusion, we again thank the Committee for addressing this important issue. **We urge you to consider re-establishing NCTR as a true national laboratory, giving it a mandate and the ability to work with all sources of technologies associated with medical product development and assessment, including private industry.** We believe such an arrangement would be invaluable in identifying surrogate markers, validating new technologies, and providing potential pathways that are viable for private investment.

The Arkansas Research Alliance (ARA):

The ARA is a non-profit, public-private partnership that invests in research that stimulates innovation, encourages collaboration and strengthens economic opportunity. Current research projects include drug development, stem cell research, and cutting edge membrane technology and purification processes, data visualization, multiple myeloma research, health and safety effects of graphene and bioinformatics. The ARA is headed by Jerry Adams, president and CEO, who has worked for decades building collaboration between industry, academia and government. The ARA Board of Directors includes the chancellors of five Arkansas universities and 15 top level managers of Arkansas corporations including Walmart, Tyson Foods and many others. The Board and CEO have entrée into the corporate, academic and government offices in a way that achieves the highest level of interest and cooperation.

In 2011, ARA was instrumental in developing a Memorandum of Understanding between FDA and the State of Arkansas. Later in 2013, ARA signed FDA's first Partnership Intermediary

Agreement, seeking to help FDA move technology from the laboratory into products that protect the public health.

ARA is vitally interested in helping the commercialization process, having been very active in that process within Arkansas. “Innovation for Healthier Americans” is a welcomed report, and we offer any support available to us to help the Committee achieve the important changes needed to address issues raised by that report.



February 27, 2015

The Honorable Fred Upton
The House of Representatives
Washington, DC 20515

The Honorable Frank Pallone
The House of Representatives
Washington, DC 20515

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

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

I want to thank you for including a provision in your draft of the 21st Century Cures Act exempting medical textbooks and peer-reviewed journals from burdensome reporting requirements. Section 4381 of your bill draft is critical to ensuring high-quality information gets in the hands of physicians to inform the best possible medical care for Americans.

Together with a wide range of organizations committed to ensuring the availability of peer-reviewed scholarly research, AAP-PSP recently sent a letter to Congressmen Burgess and DeFazio in support of the Protect Continuing Physician Education and Patient Care Act, upon which Section 4381 is based. I have attached a copy of this letter for your reference to indicate the breadth of support for these provisions.

On behalf of countless medical researchers, physicians, and the more than 38,000 Americans employed in scholarly publishing, we thank you for working to remove barriers that prevent physician access to the most current medical information. Your legislation will ensure that physicians are able to provide the best possible care to their patients through access to the highest quality publications in the world.

Sincerely yours,

A handwritten signature in black ink, appearing to read "John Tagler". The signature is fluid and cursive, with a long horizontal stroke extending to the right.

John Tagler
Vice President and Executive Director
Professional and Scholarly Publishing
Association of American Publishers, Inc.
jtagler@publishers.org
212 255-1407

February 27, 2015

The Honorable Michael Burgess, MD
US House of Representatives
Washington, DC 20515

The Honorable Peter DeFazio
US House of Representatives
Washington, DC 20515

Dear Representatives Burgess and DeFazio:

The undersigned organizations write to thank you for introducing The Protect Continuing Physician Education and Patient Care Act, H.R. 293, and to express our support for the provision exempting medical textbooks and peer-reviewed journals from reporting requirements under the Sunshine Act.

The facts are straightforward: better informed physicians provide better care to patients. Making sure that medical professionals have access to the highest quality information should be a goal of public policy, and H.R. 293 does just that. Your legislation ensures that physicians' access to the highest quality publications in the world will remain unobstructed. Medical textbooks, consults, conference reports, journal articles and their reprints are written and published for the purpose of building upon and improving clinical understanding. These universally respected publications not only meet the rigorous standards of the highest quality peer-review and editing, but also provide impartial and independent information that provides direct benefit to patients through improved care.

Physicians routinely rank among the most respected professionals in the United States because of their understanding of the ever-changing science of medicine. That respect is built upon patients' trust that their healthcare professionals remain well-informed on the latest breakthroughs in health science. Maintaining an open channel for the flow of educational information is paramount to ensuring that patients continue to be cared for by informed physicians. Provision of these materials accrues directly to the benefit of patients and to improved healthcare for all Americans.

On behalf of countless medical researchers, physicians, and the more than 38,000 Americans employed in scholarly publishing, we thank you for introducing this important legislation which will remove barriers to ensuring that physicians are able to provide the best possible care to their patients through access to the most current medical information.

Sincerely,

The following undersigned organizations

ACSESS (Alliance of Crop, Soil, and Environmental Science Societies)

Alpha Med Press

American Anthropological Association

American Academy of Forensic Sciences

American Academy of Otolaryngic Allergy

American Academy of Pain Medicine

American Association for Dental Research

American Association for the Study of Liver Diseases

American Association of Anatomists

American Association of Blood Banks

American Association of Neuromuscular & Electrodiagnostic Medicine

American Chemical Society

American College of Chest Physicians

American College of Clinical Pharmacology

American College of Clinical Pharmacy

American Geriatrics Society

American Mathematical Society

American Psychiatric Association Publishing

American Physiological Society

American Society for Bone and Mineral Research

American Society for Indexing

American Society for Investigative Pathology

American Society for Laser Medicine & Surgery
American Society for Microbiologists
American Society for Preventative Cardiology
American Society of Agronomy
American Society of Clinical Oncology
American Society of Hematology
American Society of Hypertension
American Society of Transplant Surgeons
American Society of Transplantation
American Thoracic Society
Association for Molecular Pathology
Association of American Publishers/Professional & Scholarly Publishing
Brill
Coalition for Healthcare Communication
College of Rheumatology
Content Ed Net
Crop Science Society of America
Davies Publishing
Elsevier
The Endocrine Society
F.A. Davis Company
Gerodontology Association
Heart Failure Society of America
Heart Rhythm Society
Human Factors & Ergonomics Society
International Continence Society
International Forum of Allergy & Rhinology
International League Against Epilepsy
International Publications Media Group
International Parkinson & Movement Disorder Society (MDS)
International Society for Sexual Medicine
International Society of Pediatric Oncology
International Society on Thrombosis & Haemostasis
John Wiley & Sons

McGraw-Hill Education
National Kidney Foundation
National Lipid Association
New England Journal of Medicine
New York Academy of Sciences
Northwest Graphics Inc.
Oxford University Press
Peripheral Nerve Society
SAGE Publications
Silverchair Information Systems
Slack Inc.
Society for Pediatric Dermatology
Society for the Study of Reproduction
Society of Cardiovascular Computed Tomography
Society of Hospital Medicine
Soil Science Society of America
Springer Publishing Company
Taylor & Francis
Thieme Publishers
The Triological Society
Wolters Kluwer

February 25, 2015



The Honorable Fred Upton
US House of Representatives
2183 Rayburn House Office Building
Washington, DC 20515

311 Arsenal Street
Watertown, MA 02472

The Honorable Frank Pallone Jr.
US House of Representatives
237 Cannon House Office Building
Washington, DC 20515

Submitted via cures@mail.house.gov

Dear Congressmen Upton and Pallone,

We appreciate the opportunity to comment on behalf of athenahealth, Inc. on the recent draft of the 21st Century Cures Act (the “draft Act”). As a company driven to bring healthcare into the digital age, we applaud the Energy and Commerce Committee for its bipartisan efforts to make the reforms necessary to modernize the ways in which we discover, develop, and deploy cutting-edge cures.

Of course to provide cures, our nation’s medical professionals must first be equipped to provide cutting edge *care*. Today, in 2015, the US care delivery system is chronically hampered by the prevalence of information technologies have not been cutting edge since before the turn of the century. Too often federal healthcare policy goals aspire to a level of functionality that was routine across the non-healthcare information economy a decade or more ago. Such goals can at best bring continued mediocrity and frustration for care providers and patients alike. We therefore commend the authors and supporters of the 21st Century Cures Act for daring to reach higher, and encourage you to resist the efforts of those who would have you believe that the level of information technology that virtually every one of us carries around in his or her pocket is somehow permanently out of reach of the professionals who provide our medical care.

athenahealth provides electronic health record (“EHR”), practice management, care coordination, patient communication, data analytics, and related services to physician practices, working with a network of more than 60,000 healthcare professionals who serve over 60 million patients in all 50 states. We envision and work to establish a nationwide health information backbone that makes healthcare work as it should by connecting patients and care providers with the information they need to seek and provide high-quality, cost-effective, efficient care. All of our providers access our services on the same instance of continuously-updated, cloud-based software. Our clients’ successes, exemplified by a Meaningful Use attestation rate more than double the national average, underscore the very real potential of

health IT to improve care delivery and patient outcomes while increasing efficiency and reducing systemic costs.

We have commented only on those sections of the draft Act that are relevant to modernizing the health IT infrastructure necessary to accelerate 21st century cures.

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

A. Subtitle E – Sensible Oversight for Technology Which Advances Regulatory Efficiency

We believe that as a matter of good government and in the interest of providing the greatest possible degree of statutory clarity, the ideal approach to differentiation of low-risk health IT from potentially high-risk medical devices currently and properly subject to the FDA’s devices framework would be to explicitly exclude all health IT from the statutory definition of a “medical device,” and then explicitly subject only the highest risk software to regulation under the devices framework. We understand, however, that this approach may not be politically feasible. We remain concerned that the alternative approach taken in the draft Act—which removes only health software from the definition of a medical device—increases the risk that the definition of health software will inadvertently omit existing or future low-risk functionality. This could result in a confusing, counter-intuitive framework in which some low-risk functionality remains subject to medical device regulations even more stringent than the new medical software framework. However, subject to our few comments below, we think that this draft does a good job in minimizing the likelihood of such an outcome, and we applaud your ongoing efforts to harmonize the need for a new framework with the applicable political realities informing this effort.

Specific Comments

Definition of Medical Software

“Opportunity” vs. “Need”

There appears to be a potentially impactful drafting error in section (ss)(2)(C)(i), which describes medical software as software that recommends a single treatment or course of action “without the **need** for such professional to perform additional interpretation of, or to independently confirm the means for, such recommendation.” The correct determining factor of the risk profile of health IT is whether there is an **opportunity** for a health professional to perform additional interpretation or independently confirm the recommendation, not the need. In this context, need could be subjective; a piece of software could be considered higher risk when used by a more proficient or less conscientious physician who does not feel the need to independently confirm the software’s recommendation. Instead, the bill should define as higher risk that software which does not provide the

opportunity for health professionals to confirm recommended treatment or course of action. This will preserve the principle that when a learned intermediary sits between the software and the patient with the ability to exercise his or her independent judgment there is lower risk to patient safety.

We also suggest revising the phrase “the means for” (line 22 of page 154) to more clearly convey the idea of whether a health professional has the opportunity to independently confirm the way in which the software arrived at its recommendation. Our proposed language is below and should apply to both the definitions of medical and health software.

Diagnosis

It is unclear how diagnostic functionality fits in the definition of medical software. Section (ss)(2)(B) excludes software that is intended to provide a diagnosis from the definition of medical software. We understand this to be an attempt to clarify that diagnostic devices, such as laboratory tests, are not to be considered medical software. However, the lack of any qualifying language means that software that is intended to provide a recommendation for a diagnosis could be unintentionally excluded.

Additionally, section (ss)(2)(C) is unclear as to how diagnostic software should be handled. The structure suggests that only software that recommends treatment or course of action can be in the higher-risk category of medical software, so software that provides a diagnosis without any opportunity for independent confirmation is not covered.

Therefore, we suggest revising the definition of medical software as follows:

- (2) The term ‘medical software’ means software that—
 - (A) is not a component;
 - (B) is not intended to provide a **definitive** diagnosis; and
 - (C) is intended to analyze patient-specific information and other information ~~to recommend to health care professionals a single treatment or course of action to produce a single recommendation—~~
 - (i) without the ~~need-opportunity~~ for such professionals to perform additional interpretation of, or to independently confirm **the way in which the software arrived at the means for**, such recommendation; and
 - (ii) for the purpose of informing or influencing health care decisions in the prevention, diagnosis, prognosis, treatment, cure, or disease management related to any disease or condition in humans.

Definition of Health Software

Information vs. Data

We perceive a need to clarify the intended distinction, if any, between the use of the term "data" (e.g., (3)(C), (3)(D)(ii), (3)(G)), and "information" (e.g., (3)(E), (3)(H), (3)(I)). Our interpretation is that "information" is intended to include "data".

Display of Patient-Specific Information

None of the subcategories of health software cover the presentation or display of patient-specific data. We recommend that this concept be added to both subsections (B) in addressing clinical workflow and recordkeeping, (C) so that that section more closely aligns with the FDA's definition of Medical Device Data Systems and their recent final guidance stating that the agency will not regulate such technologies, and (H) in addressing clinical decision support, as outlined in the language below.

Diagnosis

As is the case in the definition of medical software, it is unclear how diagnostic software fits in the definition of health software. Software that provides a diagnosis suggestion with any opportunity for independent confirmation is not covered in the present draft.

Therefore, we suggest revising the definition of health software as follows:

(3) The term 'health software' means software that is not medical software, is not a component, is intended to be used for or in support of a health care purpose, and—

...

(B) is intended for use for clinical, laboratory, or administrative workflow and related record keeping **or information display**, including electronic health records;

(C) is intended for use for **use** aggregation, conversion, storage, management, retrieval, **display**, or transmission of data from a device or thing;

...

(H) is intended for use to **present or** analyze patient-specific information or other information for purposes of presenting patient-specific recommended treatments, **diagnoses**, or courses of action to inform health care professionals' decisions with respect to the prevention, diagnosis, prognosis, treatment, cure, or management of a particular disease or condition, with the opportunity for additional interpretation or an independent confirmation of **the way in which the software arrived at the means for** such treatments, **diagnoses**, or courses of action; or

Functionality That Spans Multiple Definitions

The draft requests input on how to ensure that software that has functionality of a medical device, medical software, or component does not evade proper regulation when incorporated into health software. Though we agree with the definition of accessory, we reiterate our comments to the previous discussion draft that the regulatory treatment of accessories should not be included in the term's definition. An additional section is needed here. We believe that this will help address the concern of combination software being incorrectly classified.

Section 524B should have an additional section (c) that reads:

(c) Accessories.—Accessories shall be classified based on their own intended purpose, functionality, and risk, and not that of the device or software product in conjunction with which it is used.

Additionally, the FDA already has in place a policy requiring that combination devices be classified according to their highest risk element. That policy should extend to medical and health software. For example, if an electronic health record has elements that qualify as medical software, those elements should be independently reviewed as such if they are accessories, but if they are not accessories, then the entire electronic health record should be regulated as medical software.

Regulation of Medical Software

Section 524B(a)(1) directs HHS to promulgate regulations governing various aspects of the design and development of medical software, including the manufacturing of and labeling requirements for medical software. This represents a very device-centric approach, and these two requirements are prime examples of components of the FDA's quality system regulations (Code of Federal Regulations, Title 21, Part 820) that are not applicable to software. Though medical software should be regulated similarly to medical devices, this bill should not require HHS to perpetuate regulations that are inapplicable to software. The new regulations for medical software should reflect the differences between devices and software.

B. Subtitle F, Building a 21st Century Data Sharing Framework – Part 2, Improving Clinical Outcomes for Patients and Program Integrity Through CMS Data

athenahealth strongly supports expanding the permitted uses of Medicare data by Qualified Entities under Section 10332 of the Affordable Care Act. To date, the value of the Qualified Entity program has been severely limited by the fact that CMS regulations prohibit Qualified Entities from conducting additional non-public

analyses and providing or selling such analysis to healthcare providers to assist in patient care. The original goal of the Qualified Entity program is laudable: to provide CMS claims data to certain entities with research expertise so that valuable insights from that data can be gleaned for the benefit of the general public. However, Qualified Entities should also be enabled to provide these valuable data-driven insights to care providers who desperately need the patient-specific information that exists outside of their practices to coordinate and identify gaps in care.

By allowing Qualified Entities to conduct additional non-public analysis and provide or sell those analyses to healthcare providers, this bill will trigger the creation of new technological tools to help physicians and patients make well-educated care decisions, such analytics built into health IT workflows that show gaps in care or complete downstream care costs. These technologies, already common in nearly every other sector of our data-driven economy, are desperately needed in health care.

Specific Comments

Prohibition on Charging for Provision of Medicare Claims Data

We seek clarification that the provision in section 2085(a)(2)(C), stating that a Qualified Entity may not charge a fee for providing Medicare claims data, means that a Qualified Entity may not charge providers or other authorized users for the provision of the raw claims data, and that it does not prohibit a Qualified Entity from charging fees for an analysis that is based on such claims data.

Provision of Patient-Identifiable Claims Data

We are extremely supportive of section 2085(a)(3)(B), allowing a Qualified Entity to provide individually-identifiable information about the patients of providers and suppliers, including information regarding items or services furnished by other providers or suppliers. The ability to provide this patient-specific information to providers is essential to care coordination, utilization management, and closing gaps in care, which is underscored by the fact that Accountable Care Organizations are already provided with this information under the Medicare Shared Savings Program. We commend the Energy and Commerce Committee for acknowledging that the successful transition to value-based care depends on greater access to this information.

Prohibition on Use of Medicare Claims Data for Marketing Purposes

We recommend that section 2085(a)(3)(C) be revised to clarify that an authorized user shall not use any analysis or data provided or sold for marketing purposes, *as defined under HIPAA*. This will clarify that the information may still be used for treatment-related purposes such as population health campaigns and medication adherence reminders.

C. Subtitle K, Interoperability

athenahealth is gravely concerned that our nation's aspirations for interoperation in health IT have been continually downgraded, to the point where policymakers and stakeholders now set *future* goal for interoperation and health information exchange that if achieved *today* would still leave health IT lagging many years behind routine information sharing capabilities in common use across the information economy. This is as unacceptable as it is unnecessary. While a stepwise approach is necessary to achieve full interoperation of health data, policymakers also must ensure that the ultimate goal of health IT policy is be a level of functionality that keeps continual pace with modern information technology.

Our first comment is semantic, but important nonetheless. Federal policy should strive for *interoperation* (an activity) in healthcare, not mere *interoperability* (a capability). Much of the confusion that exists in this area of health IT policy arises from the fact that vendors of closed information systems are able with straight faces to claim that their systems are “interoperable,” while continuing to erect financial, operational, and technological barriers to actual, systemic interoperation.

We suggest that interoperation can rationally be conceptualized in three tiers, allowing for policy recognition of the unfortunate status quo and steady, incremental progress toward the goal of catching health IT up to the rest of the information economy and enabling it to keep pace with innovation thereafter. Crucially, policy should be structured in a way that enables continual innovation and evolution even beyond what we can envision today. At the rapid pace of innovation in information technology, policy that defines a “final” standard will eventually result in yet more stagnation in healthcare, as innovation inevitably progresses beyond the limits of our current collective imaginations.

In our conceptualization, **tier 1 interoperation** is intra-organization, enabling the sharing of demographic information, clinical orders, and lab results. Developed and achieved years ago, this is the rudimentary level of interoperation that was required to glue together the disparate departmental systems within the four virtual walls of a single hospital or health system. The standards for intra-organization interoperation, developed by the organization Health Level Seven International (“HL7”) are well-established and sufficient for their limited purpose, but they are incapable of servicing the interoperation needs of the wider healthcare economy. Intra-organization interoperation is limited to a world of wired connections and local networks—a world long gone in most industries, but not in healthcare. In healthcare, to the tremendous daily detriment of countless care providers and patients, this 20th century paradigm is still the norm.

Tier 2 interoperation allows for inter-organization information sharing. It enables transitions of care across organizations and is most frequently articulated as the desired final outcome of most interoperability policy in healthcare. Documents and notes across the continuum of care—lab results, clinical orders, images, problem

lists, medications, allergies, discharge summaries, and others—can be exchanged among different organizations and health IT systems. Inter-organization interoperation requires the use of complicated standards or interfaces that require significant technical resources and expertise, making them quite costly and difficult to scale. Such technical and financial barriers are too often exacerbated, sometimes deliberately, by organizations that either lack the business incentive or have an affirmative disincentive to share the data that corresponds with sharing patients.

These barriers have largely prevented the healthcare industry from achieving inter-organization interoperation. Healthcare providers should be able to see in one place a summary of a patient's longitudinal health history across the entire continuum of care, but this is rarely the case today.

To achieve this limited goal, two things must happen: 1) Health systems and health IT vendors must accept widespread exchange of information as a requirement of doing business in the 21st century. Lawmakers should require that patient information be exchanged freely regardless of the health or IT system from where it originated. And 2) technically, the standards underlying this type of information exchange need to be further refined, particularly for single sign-on and patient matching or consent standards. Such refinement and, ultimately, adoption cannot and should not be mandated by the government. Through organizations like the CommonWell Health Alliance, many major health IT vendors and their care provider clients are already much further along the road to functioning tier 2 interoperation than was the case a mere five years ago. The key from a policymaking standpoint is to avoid unintentionally impeding marketplace evolution and innovation by mandating uniform standards that end up proscribing efforts that are improving interoperation already.

Even if it is widely achieved in the near term, tier two interoperation will still leave healthcare lagging significantly behind the rest of the information economy. **Tier 3 interoperation**, which should be the ultimate, open-ended goal of federal interoperation policy, is the open platform. Virtually non-existent in healthcare today, this type of information exchange is prevalent in other sectors where open application program interfaces (“APIs”) are used to seamlessly weave together data from multiple disparate systems. Amazon, Kayak, Google Maps, and Mint, for example, all use APIs to pull data from multiple other systems and sources, but users only see a simple interface and user-friendly experience that presents all of that information in one place. In healthcare, open platform interoperation would enable an EHR to use APIs to integrate with countless other systems: scheduling services like ZocDoc, or patient genome sequencing services like 23andMe. Healthcare providers and patients would have the “one-stop shopping” experience that is standard in other industries but currently all but nonexistent in healthcare. An increasing number of health IT industry stakeholders use the terminology of tier 3 interoperation to create the illusion of openness, but continue to lock health information into closed networks build largely on tier 1 frameworks.

Again, federal policy cannot mandate open platform interoperability. Progress toward this paradigm in other industries has been made through the natural technical evolution of parties that have vested business interests in sharing information, not hoarding it. That motivation cannot be sufficiently created, much less sustained, through regulation, mandates, or government incentives. To provide the necessary business case for information exchange in healthcare, policymakers should require open access to data, focusing on proving that widespread inter-organization interoperability is technically possible. While remaining silent (and thereby non-prescriptive) on the evolution of interoperability beyond this point, Congress should mandate the data access necessary for inter-organization interoperability by requiring as a condition of participation in government reimbursement programs (whether the Meaningful Use program specifically or Medicare generally) that access to clinical or other information is provided to any healthcare provider, supplier, payer, or patient who requests it for HIPAA-permitted purposes through a standard incorporated in the Meaningful Use 2014 Certification Rule or a substantially equivalent standard, and without the imposition of any technical, financial, or operational barriers.

D. Subtitle M, Accessing, Sharing and Using Health Data for Research Purposes

As health data becomes increasingly digital, so does the potential to harness such data to foster a learning health system that improves quality and accelerates the discovery and development of new treatments and cures. athenahealth supports the provisions of the draft Act that seek to modernize health data privacy rules. As a general remark, we encourage the Committee to bear in mind that these 21st century discoveries are likely to come not just from traditional research facilities but also from other parties, such as information technology companies, that support the digital infrastructure of the healthcare system.

Specific Comments

Remote access for reviews preparatory to research

We appreciate the addition of section 13443, permitting remote access to protected health information by researchers, which recognizes that it is not sensible to require researchers to review health data on site in the digital age. However, as a business associate that provides remote access to protected health information to thousands of care providers and other entities, we take seriously our obligation to safeguard the privacy and security of that information. In an age where cyber attacks and hacking are all too common, it is essential that remote access be through a secure portal or access point. Therefore, we suggest adding the following language to ensure that information is protected while remotely accessed by researchers:

SEC. 13443. PERMITTING REMOTE ACCESS TO PROTECTED HEALTH INFORMATION BY RESEARCHERS.

“Subparagraph (B) of section 164.512(i)(1)(ii) of title 45, Code of Federal Regulations (prohibiting the removal of protected health information by a researcher) (or any successor regulation) shall not prohibit remote access to health information by a researcher from a **secure** portal or other **secure** access point outside of the covered entity **for appropriate research purposes** so long as—

- (1) appropriate security and privacy safeguards are maintained by the covered entity; and
- (2) the protected health information is not copied or otherwise retained **or transmitted** by the researcher.

One-time patient authorizations

athenahealth also supports the addition of section 13444, allowing one-time authorization of use and disclosure of protected health information for research purposes. The proposed language will help to strike a better balance between protecting patients’ right to privacy and providing them with the flexibility to authorize broader uses of their data to contribute to future healthcare discoveries. We are concerned, however, that patients need to be appropriately notified that this one-time authorization will also authorize the use of protected health information collected in the future. Therefore, we suggest the following revision to ensure that patients are knowingly making such an authorization:

SEC. 13444. ALLOWING ONE-TIME AUTHORIZATION OF USE AND DISCLOSURE OF PROTECTED HEALTH INFORMATION FOR RESEARCH PURPOSES.

- (a) IN GENERAL.—In applying section 164.508(c) of title 45, Code of Federal Regulations, with respect to the use or disclosure of protected health information of an individual for research purposes, the individual may submit a one-time valid authorization for the use or disclosure of protected health information of the individual with respect to all future research purposes, including the use and disclosure of protected health information of the individual that is collected after the date of such authorization, and such one-time authorization shall satisfy the requirement under paragraph (1)(iv) of such section with respect to such future research if such authorization—
- (1) sufficiently explains that the information will be used and disclosed for future research;
 - (2) **sufficiently explains that the authorization covers current protected health information as well as protected health information collected in the future;**
 - (3) states that the authorization will remain valid unless and until it is withdrawn by the individual; and
 - (4) permits the individual, and provides instruction to the individual on how to opt-out of, or otherwise withdraw, such authorization at any time.

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

A. Subtitle I, Telemedicine

athenahealth is encouraged by the ongoing review and refinement of Medicare's coverage of telemedicine services. We support and agree with the comments submitted by Health IT Now on this subtitle. Additionally, we feel that the loosening of certain requirements, such as geographic limitations and originating sites, will help increase telemedicine's adoption. Telemedicine can be tremendously useful for chronic care and transition care management and could help reduce readmissions as well as overall costs.

We commend this Committee for recognizing the importance of modernizing the way in which the United States discovers, develops, and deploys cures. Please do not hesitate to contact us if we can answer any questions or provide additional input. Thank you again for the opportunity to comment on the draft Act.

Sincerely,

A handwritten signature in blue ink, appearing to read 'Dan Haley', is positioned above the typed name.

Dan Haley
Vice President
Government and Regulatory Affairs

Case Western Reserve University
Response to Select Provisions of 21st Century Draft Discussion Paper

February 17, 2015

Title I

- Subtitle A – Patient-Focused Drug Development:
 - We are in favor of this provision - incorporating patient input on suggested benefits and risks of clinical trials is very important.
- Subtitle C – Approval of Breakthrough Therapies:
 - We are strongly in favor of this provision – increasing the use of a “breakthrough therapy” designation is a good policy.
- Subtitle J – Streamlined Data Review:
 - Attention should be given to the incorporation of a drug fact box on labels as has been previously suggested to the FDA.
- Subtitle K – Cures Acceleration Network:
 - We believe that the National Center for Advancing Translational Sciences (NCATS) should have the flexibility through use of Other Transaction Authority (OTA) funds. However, we are concerned that, barring other provisions, expanded efforts within NCATS outside the Clinical & Translational Science Awards (CTSA) programs may damage CTSA. We need the national CTSA infrastructure to be robust for the Cures agenda to be sustainable. This is not currently addressed in the legislation.
 - Also, inter-institute cooperation is a key area that could accelerate therapies. Funds from the institutes should be brought together within NCATS if these initiatives are to be successful.
- Subtitle M – New Therapeutic Entities:
 - Incentivizing new therapeutic entities related to process/product improvements will extend patent life.

Title II

- Subtitle A – 21st Century Cures Consortium Act:
 - We are strongly in favor of the proposed Consortium.
- Subtitle L: NIH – Federal Data Sharing:
 - Data sharing is already mandated in NIH grants.
 - It is our concern that this additional provision may place more burdens on investigators who are already under significant administrative burdens.
- Subtitle M – Accessing, Sharing, and Using Health Data for Research Purposes:
 - Due to existing regulations, fulfilling this proposal would be challenging for researchers.
 - When data is used for research as opposed to commercial purposes, reducing fines and penalties for inadvertent record release would be a big help.
 - By making research a “safe harbor” for data, researchers will have an easier time accessing, sharing, and using health data – to the benefit of patients.

Title IV

- Subtitle A, Section 4001 – NIH Strategic Investment Plan:
 - We support a strategic plan within NIH, particularly one focused on inter-institute initiatives and burden reduction for researchers.
 - Streamlining the reporting and regulatory burdens is critical for national science productivity – these steps will enable more science to be done while operating safely and effectively.
 - Given this, we would encourage additional language to emphasize these initiatives.

- Subtitle I – Telemedicine:
 - There is no physical patient contact or examination with the process, so the issue should be well defined if a standard reimbursement is considered. As outlined, a physician would not be reimbursed if they called a patient on the phone to discuss their problem, but would if they happened to do so via Skype. As it is, it could be ripe for misuse and not achieve the good intentions of the concept.
 - Criteria for reimbursement for Telehealth Services should include: 1) documented medical necessity, 2) a provider licensed in the State, 3) have an established patient-physician relationship (will exclude new patients and consults), 4) proper documentation in medical records, 5) specific guidelines for reimbursement (complexity vs. time-based vs. flat fee) and 6) security measures that are in place.
 - It is essential that there is a related language regarding Telehealth Monitoring, as both issues will be germane for value-based reimbursement.

For more information, contact:

Jennifer Ruggles, Case Western Reserve University, 216-368-6519, jor15@case.edu
Elizabeth Littman, Case Western Reserve University, 216-368-1841, eal2@case.edu



February 23, 2015

Committee on Energy and Commerce
U.S. House of Representatives
2125 Rayburn House Office Building
Washington, DC 20515

Dear Chairman Upton, Ranking Member Pallone, and Members of the House Energy & Commerce Committee:

On behalf of the Federation of State Medical Boards (FSMB), the national non-profit representing the 70 state medical and osteopathic boards of the United States and its territories, I am pleased to submit comments in response to the Energy and Commerce Committee's draft legislation, *21st Century Cures Act*.

Recommendation 1: Sense of Congress Regarding State Medical Board Compact (Subtitle I – Telemedicine, pg. 299)

The FSMB and its Member Medical Boards offer our sincere appreciation to the Energy and Commerce Committee for voicing support for the development and implementation of the Interstate Medical Licensure Compact, a new expedited pathway for qualified physicians seeking licensure in multiple jurisdictions.

In September 2014, following an 18 month drafting process, final model legislative language of an Interstate Medical Licensure Compact was released to states and their respective medical boards for their formal consideration. As of February 23rd, at least 27 state medical and osteopathic boards have formally voiced support for the Compact, as well as the American Medical Association, American Academy of Dermatology, Council of Medical Specialty Societies, Society of Hospital Medicine, and many other health management associations and hospital systems across the nation. We expect the list of supporters and legislative activity to continue to grow as state legislatures begin to formally consider the Compact during the 2015 legislative session. The Compact legislation has already been introduced in 14 states, including Idaho, Illinois, Iowa, Maryland, Minnesota, Montana, Nebraska, Oklahoma, South Dakota, Texas, Utah, Vermont, West Virginia, and Wyoming.

For the purposes of clarity as to the Compact's functionality, the FSMB respectfully recommends that the (c) SENSE OF CONGRESS REGARDING STATE MEDICAL BOARD COMPACTS be rephrased to read:

(c) SENSE OF CONGRESS REGARDING INTERSTATE MEDICAL LICENSURE COMPACT

It is the Sense of Congress that States' enactment of the Interstate Medical Licensure Compact will expand access to care, facilitate multistate practice and enable the use of telehealth services across state lines, by streamlining licensing processes and ensuring the necessary state medical regulatory authority to protect the public.

Recommendation 2: Standard of Care / Definition of Telehealth (pg. 294)

In selecting and defining telehealth services eligible for payment, the FSMB recommends that the Energy and Commerce Committee review and consider the *Model Policy on the Appropriate Use of Telemedicine Technologies in the Practice of Medicine*, adopted unanimously in 2014 by the FSMB House of Delegates. The *Model Policy* defines telemedicine as "the practice of medicine using electronic communications, information technology or other means between a licensee in one location, and a patient in another location with or without an intervening healthcare provider."

Among its key provisions, the model policy states that the same standards of care that have historically protected patients during in-person medical encounters must apply to medical care delivered electronically. Care providers using telemedicine must establish a credible “patient-physician relationship,” ensuring that patients are properly evaluated and treated and that providers adhere to well-established principles guiding privacy and security of personal health information, informed consent, safe prescribing and other key areas of medical practice. The guidelines are designed to provide flexibility in the use of technology by physicians – ranging from telephone and email interactions to videoconferencing – as long as they adhere to widely recognized standards of patient care.

The FSMB recommends that legislative language be included in the bill to reflect that providers of payable telehealth services must adhere to the same rules, regulations and laws, as they relate to the standard of care and licensure, of the state where the patient is located, as the provider would in a traditional face-to-face medical encounter.

The FSMB was proud to endorse *H.R. 3750, The Telehealth Modernization Act of 2013*, introduced by Reps. Matsui and Johnson, which establishes a much-needed federal definition of telehealth, and hopes the Energy and Commerce Committee will consider its inclusion in the legislation.

Recommendation 3: Geographic Limitations

The FSMB recommends that language associated with “any geographic limitation” (Pg. 292 and Pg. 297) be clarified as in relation and solely for the purposes of payment, and not in terms of licensure requirements. The FSMB has regularly affirmed that the practice of medicine occurs where the patient is located, rather than where the provider is located. This patient-centered model is both time-tested and practice-proven, and is the nationwide standard that ensures that state medical boards have the legal capacity and practical capability to regulate physicians treating patients within the borders of their state, and to attest that those physicians meet the qualifications necessary to safely practice medicine.

Conclusion

The FSMB commends the Energy and Commerce Committee for its efforts to expand access to telehealth services to patients in a safe and accountable manner. The FSMB would be pleased to meet with you to discuss our recommendations. We thank you for your bi-partisan leadership on this important issue, and look forward to working with you in the 114th Congress.

Sincerely,



Humayun J. Chaudhry, DO, MACP
President and Chief Executive Officer
Federation of State Medical Boards



Mauro Ferrari, Ph.D.
President and CEO
Houston Methodist Research Institute
Executive Vice President
Houston Methodist

6670 Bertner St.
Houston, TX 77030
Office: 713-441-8439
Email: mferrari@ houstonmethodist.org
houstonmethodist.org

The Honorable Fred Upton
21st Century Cures Initiative
House Energy and Commerce Committee
2125 Rayburn HOB

The Honorable Diana DeGette
21st Century Cures Initiative
House Energy and Commerce Committee
2322 Rayburn HOB

Dear Chairman Upton and Representative DeGette:

Thank you again for your leadership in creating and spearheading the 21st Century Cures initiative. As conversations have progressed and the legislative process has begun, we are delighted to see a promising framework take shape in the discussion draft. In my experience as a researcher, academic department director, successful entrepreneur, advisor to the National Cancer Institute, research institute CEO, and hospital Vice President, I have participated in every aspect of the discovery, development and delivery continuum. From all sides, the 21st Century Cures initiative is paving the way to revolutionize medical research and spur a level of innovation that has never been seen before.

Facilitating medical research outcomes through improvements such as the consideration of surrogate endpoints and clinical evidence, fostering new techniques and technologies like repurposing and nanomedicine, supporting personalized medicine, clarifying and streamlining the regulatory processes, and encouraging health care coverage are among the many significant measures in your discussion draft that we were glad to see.

We would like to offer three additional suggestions to contribute to your impressive work. These include shifting the current allocation of NIH funding to support the clinical objectives of translational research, defining translational research in statute to optimize the use of taxpayer dollars, and adding patients and experts to peer review panels to reinforce a focus on patient-centered solutions.

NIH funding shift from basic to translational

Basic research is an integral step in the discovery to delivery continuum but it often only advances academic or scientific knowledge. Taxpayer dollars could be used more effectively by shifting NIH funding to increase support of translational research, which goes beyond the scope of basic and applied research to directly address an observed clinical need. It is critical we prioritize medical research that will make real and lasting differences in the lives of patients.

It is frustrating to see successful medical discoveries in our Research Institute obstructed from further progression because of current funding allotments. In the case of my targeted therapy for breast cancer metastasis, a frequently fatal diagnosis, almost 50% of the test animals in my lab have been completely cured. However, my project has been unable to progress to patient trials due to a lack of financial resources for the next stage of FDA-required preclinical trials. Worse yet is crossing the hallway from the Research Institute to Houston Methodist's cancer clinic and seeing the very patients who are hopeful for promising new treatments.

Many researchers are discouraged from continuing basic research beyond successful publication in academic journals because of a lack of resources and financial support beyond that stage. Academic researchers have no incentive but to carry on with finessing more basic discoveries to advance their careers as scientists. Not surprisingly, the repetitive nature of preclinical trials needed to prove reliability is, in itself, not an attractive enough venture. By increasing NIH funding designated for translational research, this incentive will create a gravitational pull that promotes continuing on with promising research for clinical solutions.

Pharmaceutical, biotech and medical device companies no longer begin product development from basic research discoveries due to the strain on resources in today's economy. They instead look for marketable solutions that have already been proven to be safe, effective, and reliable in animal studies and still have sufficient patent life to develop and distribute to patients. Researchers need financial resources to conduct their studies and prove the merit of further development. By adjusting the NIH funding structure to support translational research, you can encourage, facilitate and expedite the process of turning knowledge into action and realizing the future of medical innovation.

Statutory definition of translational research

Translational research is referenced by federal agencies in various forms but no specific definition exists in law. As such, any entity can claim their research is translational even if they do not have the experience or ability to advance basic research through the bench to bedside continuum. Without a statutory definition, taxpayer dollars for translational research are not guaranteed to be used in the most efficient or effective manner.

We have drafted language for your consideration (attached). The proposed definition prioritizes the resources and capabilities required to conduct research that can truly be translated into real clinical solutions. Equally important is that research development be primed for FDA approval and industry handoff by using FDA-certified Good Laboratory Practice (GLP) and Good Manufacturing Practice (GMP) facilities, and completing early phase trials. The definition not only clarifies qualifications, but also broadens the opportunity for participation in translational research through collaboration.

As an example, the Clinical and Translational Science Center (CTSA) program within the NIH's National Center for Advancing Translational Sciences (NCATS) requires that federal grant applicants be degree-granting, even though awarding academic degrees has absolutely no bearing on the real world clinical translation of drugs, therapies or medical technologies. To truly do translational research, it is vastly more important to have access to patient care environments and facilities to conduct FDA-compliant trials.

Certain types of studies can only be done if everything is located on one site, such as therapeutics or radiopharmaceuticals produced in a GMP that must be delivered to patients quickly to still be effective. If a researcher does not have access to GLP or GMP facilities, it is next to impossible to achieve the consistent, measurable conditions required to show reliability of the treatment. Many innovative therapies may be abandoned because of this logistical complication.

A statutory definition of translational research would not exclude all of the current institutions receiving translational research funding, but would allow and encourage greater collaboration among even more entities. Working in coordination with facilities capable of and experienced in translational research would make it possible for more parties to participate in discovering, innovating, creating, and testing a greater number of promising drugs, therapies and technologies to address patient needs.

Peer review participants

In the selection process for grant funding, peer review panels are mostly comprised of academics or basic researchers. Although scientific review of medical science will create even better science, the focus of medical research funding should be on the advancement of patient-centered clinical solutions. This is often not the result when academic researchers inexperienced in translating basic research into clinical results are responsible for choosing the research projects that receive funding. Peer review panels need to include stakeholders involved in the entire discovery, development and delivery continuum to foster research that is feasible for real-world production and clinical use.

In addition to the current participants, panelists should also include successful translational research experts, regulatory agency officials, doctors and caregivers, industry professionals, entrepreneurs and venture capitalists, health insurance providers, and most importantly, patient advocates.

By including patients in the evaluation process, their feedback would provide the insight that only firsthand experience can bring. Their involvement would also change the dynamic of a review to no longer prioritize science for the sake of advancing science, but remind everyone involved that the goal is a patient-centered medical solution. At Houston Methodist, we require that proposed research projects be translational, transformational, interdisciplinary, and always patient-oriented.

Conclusion

Translational research is critical for priming promising discoveries for FDA-compliant clinical trials and ultimately transforming the research into innovative drugs, therapies or technologies that can be produced and delivered to patients. By increasing funding for translational research, creating a definition to ensure that entities are equipped to conduct translational research and expanding the participants in peer reviews to include patients, clinical knowledge can be turned into innovative cures and treatments that will make real and lasting differences in the lives of those that have funded it through tax dollars.

We thank you again for this opportunity to provide our suggestions and contribute to your outstanding efforts. I had the privilege of testifying at a 21st Century Cures field hearing held in the Texas Medical Center last year and I look forward to continuing this dialogue.

We are available to you at any time to provide technical expertise or assistance in your endeavors. It is an honor to be a part of the 21st Century Cures solutions to streamline, modernize and optimize our medical research paradigm. The United States has always held the legacy of being a pioneer of medical innovation and together we can continue our helm at the forefront.

Sincerely,

Mauro Ferrari, Ph.D.
President and CEO
Houston Methodist Research Institute
Executive Vice President
Houston Methodist

Draft language to be inserted in Title IV of the 21st Century Cures Act

Section XXX: For the purposes of this title, the term “translational” means medical research and development that directly addresses clinical needs, and is undertaken by an entity with direct access to a patient care environment which, seamlessly and without duplicative regulatory requirements, performs in-tandem steps through discovery, preclinical testing, and early phase clinical trials in a facility that is--

- (a) certified for current good laboratory practices by the Food and Drug Administration;
- (b) certified for current good manufacturing practices by the Food and Drug Administration;
- (c) capable of Phase I trials, as described by the United States National Library of Medicine;
- and
- (d) capable of Phase II trials, as described by the United States National Library of Medicine.

February 22, 2015

The Honorable Fred Upton
U.S. House of Representatives
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:

As a lupus patient, longtime advocate and leader of the Lupus and Allied Diseases Association, I would like to applaud you in your efforts to accelerate the discovery, development, and delivery of promising new treatments and cures with the 21st Century Cures Initiative. I especially appreciate your inclusion of policies on patient-focused drug development, biomarker qualification, streamlining the regulatory process, and modernizing clinical trials in addressing unmet medical needs. Based on my experiences as both a lupus patient and advocate, I offer the following comments in response to your request for additional stakeholder input and greatly appreciate the opportunity to assist in ensuring therapies reach those who desperately need them.

I. Systemic Lupus Erythematosus (Lupus) Overview & Impact

Lupus is an extremely complex chronic inflammatory autoimmune disease in which a triggering agent causes the immune system to dysregulate and attack the patient's own tissue affecting virtually any organ system of the body; including the skin, joints, kidney, brain, heart, lungs, blood and blood vessels and there is no known cause or cure. Lupus is a leading cause of kidney disease, stroke and premature cardiovascular disease in young women and is highly individualized, extremely volatile, debilitating, life-diminishing, and potentially fatal. It is estimated that 1.5 to 2 million Americans suffer from lupus,¹ affecting women 9 times more often than men,² with 80% of new cases developing between the ages of 15 and 44 during the prime of life.³ Among rheumatic conditions lupus has a relatively high mortality; 14.5% of all rheumatic disease mortality in 1997.⁴

Presently, no single test exists to identify lupus, resulting in many patients suffering more serious complications before a diagnosis is reached. Lupus is an unpredictable condition in which symptoms come and go (flares) and complications can arise suddenly, frustrating patients and the physicians who treat them. It is also a costly multi-system disease as patients must see several specialists regularly and because it can affect virtually any part of the body it is the prototypical autoimmune disease.^{5,6} It ranges from mild to life-threatening and patients with lupus have an increased incidence of being diagnosed with other autoimmune conditions, including thyroid disease, Sjögren's syndrome, Raynaud's phenomenon, and

¹ Bruskin-Goldring Research Study conducted through telephone survey for the Lupus Foundation of America, 1994

² Hahn BH, Wallace, DJ The epidemiology of systemic lupus erythematosus. In *Dubois' Lupus Erythematosus* (5th Edition). Philadelphia: Williams & Wilkins, 1997

³ Wallace DJ, *The Lupus Book: A Guide for Patients and Families*. New York: Oxford University Press, 1995

⁴ Sacks JJ, Helmick CG, Langmaid G, Sniezek JE. Trends in deaths from systemic lupus erythematosus—United States, 1979–1998. *MMWR* 2002;51(17):371–374. Centers for Disease Control and Prevention.

⁵ Pisetsky DS, Buyon JP, Manzi S. Chapter 17. Systemic lupus erythematosus. In: Klippel JH, Crofford LJ, Stone JH, Weyand CM. *Primer on the Rheumatic Diseases*. Edition 12. Arthritis Foundation, Atlanta, GA., 2001.

⁶ Rus V, Hajeer A, Hochberg MC. Chapter 7. Systemic lupus erythematosus. In: Silman AJ, Hochberg MC (eds.) *Epidemiology of the Rheumatic Disease*. 2nd edition. Oxford University Press, New York, 2001.

clotting disorders, as well as a high incidence of co-morbid conditions, such as depression, cardiovascular disease, migraine headaches, fibromyalgia, seizures, atherosclerosis, central nervous system disease, neuropsychiatric manifestations, and gastrointestinal problems.

Many lupus patients experience ongoing inflammation and anemia which contributes to general fatigue, chronic pain and mood disturbances, poor sleep quality, and cognitive impairment. Lupus patients have a significantly lower quality of life than that of patients with other chronic diseases.⁷ Fatigue is the most prevalent and incapacitating symptom experienced by about 85 to 92% of lupus patients, resulting in decreased physical and mental function, and 50% of patients rated it as the most disabling symptom.⁸ Lupus patients also take a multitude of medications, requiring careful attention to drug-drug interactions and many have unique allergies and sensitivities.

I can tell you from firsthand experience that “Lupus is: extremely complex, difficult to diagnose, potentially fatal, presently incurable, totally capricious, painfully limiting, life altering, dream stealing, career ending, and financially, emotionally, and physically devastating. Living with lupus is like swimming in shark-infested waters. The danger and uncertainty is always present and one is armed with nothing but a will to survive. We try to stay afloat while anticipating the next attack and remain ever hopeful that a rescue ship will soon appear on the horizon.”

I once had a life filled with dreams and promise; my future was bright. As a language major I dreamt of going off to Europe, falling in love with a Prince, and living the life of a romance novel character. Instead I was diagnosed with Systemic Lupus Erythematosus, secondary Sjögren’s Syndrome and Raynaud’s after years of testing, emergency abdominal surgery, and my first brush with death. The numerous aches & pains, digestive impairments, overwhelming fatigue, constant craving for water, itchy, inflamed eyes, intolerance for the sun, cold hands and feet, and recurrent infections I had experienced since childhood finally had a name.

My schedule soon revolved around prescription drugs, doctor appointments and medical procedures, getting enough rest, avoiding the sun, and staying away from people with germs; all while trying to attend college. My athletic days ended abruptly, social occasions were rare, and what remained was a bleak, uncertain future. After experiencing aspirin-induced hepatitis and missing a semester of college, I was finally able to graduate and start a career. For every 2 years I worked, I would end up on disability for a year. I felt like I was constantly chasing my tail and never getting ahead. At my physicians’ urging, I finally threw in the towel and filed for disability, winning my case 24 years ago. Lupus stole my dreams of ever being a mother, having a career or being financially secure.

Like many others with lupus, I suffer from several other autoimmune disorders so here are my current numbers: 8 autoimmune conditions, 35 medications per day, monthly treatments costing over \$6,000, annual health care costs of about \$150,000. I used to weigh 220 lbs. due to drug side effects which made dating as a young woman extremely difficult due to my negative body image and low self-esteem. My entire digestive tract is impaired and it takes 5 different drugs to allow me to digest food each day. I haven’t eaten fruits or vegetables in 18 years now and suffer from constant colicky abdominal pain. To date I have had 25 upper GI endoscopies and colonoscopies and so far have refused a colostomy. I am forced to wear compression stockings to maintain a normal blood pressure and avoid blood clots due to hypotension and anti-phospholipid antibody syndrome.

⁷ Pettersson S, Lovgren M, Eriksson L, Moberg C, Svenungsson E, Gunnarsson I, Welin Henriksson E. An exploration of patient-reported symptoms in systemic lupus erythematosus and the relationship to health-related quality of life. *Scand J Rheumatol.* 2012;14:383–390. doi: 10.3109/03009742.2012.677857.

⁸ Zonana-Nacach A, Roseman JM, McGwin G Jr, Friedman AW, Baethge BA, Reveille JD, Alarcon GS. Systemic lupus erythematosus in three ethnic groups. VI: factors associated with fatigue within 5 years of criteria diagnosis. LUMINA Study Group. *Lupus in MI*nority populations: NAture vs Nurture. *Lupus.* 2000;14:101–109. doi: 10.1191/096120300678828046.

The veins in my arms are pretty much useless due to scarring from decades of lab draws so I am on my second infusaport for monthly labs and all infusions. I have long hair because it is too difficult to hold my arms up to style shorter hair. For the past 3 years, I have been treated with weekly 7-hour infusions for hypokalemia due to my lupus kidney disease. I am also infused bi-monthly for ongoing iron and zinc deficiencies due to disease inflammation and dietary limitations.

Between the stiffness, dizziness and letting the GI therapies run their course, it takes me 3-4 hours each day before I can function well enough to leave my house or a hotel room if traveling. Besides my infusions I have an average of 6-8 medical appointments a week which does not take into consideration the travel time to and from the medical centers since I live in rural upstate NY where the number of specialists are limited. The ubiquitous fatigue I experience is so extreme at times I feel lifeless, like a vampire drained every bit of blood and energy from me. My life is filled with missed opportunities, limitations and loss and I am unable to go to most events I would like to attend. It takes a tremendous amount of self-motivation to manage my medical care, maintain my dignity and attempt to have any quality of life.

Somehow, I have survived thus far due in part to becoming empowered and proactive in my care, the tremendous support of my family and expertise of my health care providers. Being down in the trenches with other lupus warriors from all over the country for 29 years now has also fueled my passion and inspired me as a national advocate. “Like so many others living with lupus every two steps I take forward, I take one backward. It feels like I am constantly climbing a mountain, struggling to reach the peak and place my feet on solid ground, but as soon as I am close to the summit I lose my foothold plummeting backward into the unknown abyss; desperately grasping for anything substantial to grab onto. Not only does this drain one physically but the emotional toll is devastating. Dreams and goals are always being reassessed and some days it is a victory just to get out of bed, shower and put on clean pajamas.”

“Imagine helplessly watching someone you love suffer relentlessly from an incurable, ravishing, life-threatening disease. Imagine learning that most lupus patients suffer for years prior to being diagnosed. Many, like your loved one become very ill before a diagnosis can be made. Imagine hoping for a donor as your loved one is repeatedly connected to a kidney dialysis machine. Imagine experiencing the loss of a stillborn child or multiple miscarriages or even a stroke. Imagine having to explain to your loved one that their limbs have been amputated while they were in a coma because of an infection. Imagine watching someone you love lose their job and health benefits only to be forced to then deplete their assets to meet public assistance eligibility standards. This is lupus. Every minute of every day another person loses a little piece of themselves and who knows what potential any of them could have achieved in their lives were it not for this debilitating disease.”

II. Lupus and Clinical Trials

Lupus is considered a disease of unmet medical needs due to lack of efficient diagnostic tools, effective therapies, and well-designed clinical trials. Lupus clinical research has been unsuccessful due to the diversity of the patient population, absence of reliable biomarkers, limitations of clinical outcome measures and non-existent uniform control groups. Often, classification criteria are used to define study populations because there are no specific biomarkers. This results in combining patients with various disease manifestations and different pathophysiology and pathogenesis together into one group. The absence of reliable biomarkers is a challenge for clinicians in providing the most optimal patient care and is impeding the development of new lupus therapies.⁹ There is a critical need for more sensitive and reliable biomarkers that can predict susceptibility, activity, severity and disease subtype in lupus.

⁹ Liu C-C, Ahearn JM. The Search for Lupus Biomarkers. *Best practice & research. Clinical rheumatology* 2009;23(4):507-523. doi:10.1016/j.berh.2009.01.008.

Due to the heterogeneous nature of lupus, unpredictable relapsing and remitting course of the symptoms, lack of validated biomarkers, clinical endpoints and outcome measures, uniform control group, and existence of concomitant medications—usually immunosuppressives, it is very difficult to develop new treatments in lupus. Clinical research trials are not designed to measure what is most important to those who are participating such as improved daily quality of life, reduction in current drug regimen, side effect tolerability, and co-morbid conditions. Patients are concerned with potential cosmetic side effects such as hair loss, rashes, weight gain, gastrointestinal problems; things that may be socially challenging to young women in the prime of their life.

Lupus research should focus on the goals of controlling symptoms, preventing complications, limiting organ damage, increasing survival, improving overall health and day to day functioning for patients. We need innovative, collaborative efforts—private, public partnerships among various stakeholders and multi-center projects.

Understanding the importance of clinical trials in getting new treatments to patients, I participated in clinical research for over 5 years until suffering a serious adverse event and withdrawing from the study. Patients do not all have access to trials in their geographic area and many do not have the resources to travel to participate in trials. Participation is time consuming; anyone who is working, attending school or taking care of their children has limited time. The paper work is complicated and lengthy, and it is difficult to concentrate with fatigue and cognitive problems. Patients do not want to be on steroids or chemotherapy drugs, we just want something that works with few if any side effects.

III. Lupus Therapies

Currently, there are only four FDA-approved treatments for systemic lupus erythematosus: aspirin, antimalarials, corticosteroids, and belimumab. In 2011 the FDA approved the fourth treatment for lupus after many years of limited accepted therapeutic options that included: corticosteroids, antimalarials, and aspirin. These older medications continue to be standard treatment for lupus today.

In addition, the FDA has acknowledged that immunosuppressives are a standard treatment for lupus, even though none are indicated.¹⁰ Belimumab, recently approved in 2011, is a biologic that is only approved in combination with standard treatments. Hydroxychloroquine, the most commonly used antimalarial drug, and corticosteroids, including prednisone were approved in the 1950s. Many of the companies that developed the brand name drugs used for lupus treatment discontinued production, resulting in mostly generic versions remaining on the market.

Due to the heterogeneous nature of autoimmune diseases like lupus, no two cases are alike and treatment is highly individualized; effectively treating patients like me is like balancing on a pinhead. Numerous times therapies have failed me over the years, forcing me and my physicians to take a step backwards and think outside of the box to treat my complicated medical picture. Existing treatments for lupus are absolutely inadequate; many are toxic and cause detrimental side effects with long-term use and since there are only 4 drugs currently approved for lupus many therapies are off-label such as cancer treatments and transplant drugs.

My treatments are tailor made and 14 years ago my physician made the compassionate decision to try an expensive off-label immunosuppressive drug to reduce my steroids. This drug has allowed me the ability

¹⁰ This off-label use is so well accepted that the FDA's news release announcing Benlysta's approval stated, "standard therapy [for Lupus], including corticosteroids, antimalarials, immunosuppressives, and nonsteroidal anti-inflammatory drugs." (FDA News Release. FDA approves Benlysta to treat lupus. Mar. 9, 2011, *available at* <http://www.fda.gov/newsevents/newsroom/pressAnnouncements/ucm246489.htm>)

to function better when I could barely think, walk or raise my arms above my head and had spent months in bone gnawing, soul wrenching pain going from physician to physician begging for help. It is a desperate place to be.

Many treatments have significant side effects, especially with continuing use. For decades the “go to” drug for lupus patients has been prednisone, a corticosteroid. I call it, “the drug you love to hate.” It certainly saves your life and fights inflammation quickly, especially in an acute situation, but this comes at a high price with horrific side effects such as glaucoma, cataracts, hypertension, diabetes, acne, atherosclerosis, avascular necrosis, osteoporosis, an increased susceptibility to infections, elevated cholesterol, obesity, edema, fat deposition, manic feelings, and an appetite equal to that of 4 growing teenage boys. Other treatments include immunosuppressives that ablate the entire immune system and antimalarials which can cause retinal toxicity. Off-label therapies such as cancer treatments can cause infertility and miscarriages. Patients get numerous infections and many of us take drugs to treat the side effects of other medications which is ludicrous. Current treatments are just band-aids treating the symptoms and never getting to the root of the problem.

Partially due to allergies to aspirin and nonsteroidal anti-inflammatory drugs, I took prednisone for 30 years straight, and every time my physicians tried to taper me down I would flare. I now have avascular necrosis, osteopenia, glaucoma, and cataracts as a result of steroid use. I am extremely sensitive to inactive ingredients in drugs. For the past year I have been dealing with complications from severe shingles in my eye and on my face. It has permanently damaged my vision and the eye drops have caused complications resulting in more drugs being prescribed. This is a vicious cycle. When the immunosuppressive drugs are reduced, my lupus flares. I have 6-10 infections a year and am allergic to all antibiotics except penicillin so treating any infection that arises is extremely difficult. My physicians are as frustrated as I am with the current status of lupus therapies. I have endured decades of destruction and disfigurements from the treatments I have endured.

Individuals with complex care needs like me require unique strategies and personalized medicine to manage their care. I know that newer, innovative treatments can offer therapeutic advantages over conventional medicines. Older immunosuppressive therapies attacked a patient’s entire immune system; causing harmful side effects, while newer targeted therapies target a particular cell or biomarker making the treatment more efficient and safer. Right now we are in dire need of new more efficacious treatments and are unequivocally frustrated with the drug development process regarding lupus and so tired of waiting.

IV. Lupus Subpopulations

Lupus disproportionately affects women of color in the United States; it is 2 to 3 times more common among African-Americans, Hispanics & Latinos, Asians, and Native Americans.¹¹ Minority women tend to develop lupus at a younger age, experience more serious complications, and have higher mortality rates—up to three times the incidence and mortality of Caucasians.¹² It is estimated that as many as one in every 250 African American women in America has lupus.¹³ 90% of those affected are women, but men and children are also diagnosed with lupus.¹⁴

¹¹ National Institute of Arthritis and Musculoskeletal and Skin Diseases, National Institutes of Health. *Strategic Plan for Reducing Health Disparities*. 2006.

¹² Sacks JJ, Helmick CG, Langmaid G, Sniezek JE, Centers for Disease Control and Prevention, *ibid*.

¹³ Harley JB, Kelly JA. Genetic basis of systemic lupus erythematosus: a review of the unique genetic contributions in African Americans. *Journal of the National Medical Association* 2002;94(8):670-677.

¹⁴ Hahn, BJ & Wallace DJ, *ibid*,

Mortality rates show the existence of distinct age, sex, and race-specific disparities in lupus; individuals aged 15 to 44 years of age had an occurrence of 36.4%, crude death rates increased with age, were 5 times higher among women than men, were 3 times higher among blacks than whites, and were highest among black women, increasing 69.7% among those aged 45--64 years despite the overall decrease in the mortality rates associated with lupus.¹⁵ Five-year survival in lupus patients has improved from 50% in the 1950s to over 90% currently, but the mortality still remains high compared with the general population.¹⁶

Eighty percent of newly diagnosed patients are women in their child-bearing years or prime of life.¹⁷ From the time I was diagnosed as a young woman in college, my life took on a new direction. As my classmates looked forward to careers and romances I wondered whether I would ever graduate or even be alive in 5 years. I had been an athlete, physically fit and popular. That all drastically changed with the diagnosis and treatments.

There is no specific test to diagnose lupus, resulting in delayed diagnosis, proper medical intervention, more severe disease manifestations, and worse patient outcomes. Diagnosis is based on numerous medical appointments and lab results, a process of elimination, extreme patience and open to clinical interpretation. One can only hope they see a physician who takes them seriously and was paying attention in medical school when autoimmunity was covered.

I also feel that many of us in our mid 40's to late 60's are an overlooked subpopulation when it comes to research, awareness and interest in lupus. We have beaten the odds and survived and are still dealing with lupus, co-morbid conditions and the devastating effects of the decades of treatments and disease damage. Most of us were diagnosed during the prime of life and have a wealth of information and experience to share about the disease, treatments, lifestyle, coping mechanisms, and survival tips.

V. Conclusion

Since lupus is the prototypical autoimmune disease in that it affects virtually any part of the body including organs, any lupus research initiative has the potential to impact millions of Americans suffering from over a hundred different autoimmune and related conditions. Lupus disease research is under-funded in comparison with other diseases of comparable magnitude and severity. The benefits of lupus research are unquestionably far reaching.

In conclusion, I feel that my perspective as both a longstanding lupus patient and advocate is worthy of your consideration and appreciate the opportunity to provide comments. I applaud you again for the 21st Century Cures Initiative and look forward to collaborating with you to ensure any related legislation moves forward and commend you for including the patient perspective during this process. After all, lupus and other diseases of unmet need do not just affect patients, but also impacts our loved ones and the health care professionals who treat us. As millions in the lupus community eagerly await the development of new therapies, we passionately urge you to accelerate the development and delivery of new, more efficacious treatments and ultimately a cure for diseases of unmet need like lupus and establish public policies to ensure patients have access to these treatments.

Individuals living with conditions of unmet need require individually tailored treatments and unencumbered access to the full array of treatments as prescribed by their treating physicians who are most familiar with the patient's history. Policies such as specialty tier pricing, step therapy, therapeutic

¹⁵ Sacks JJ, Helmick CG, Langmaid G, Sniezek JE, Centers for Disease Control and Prevention, *ibid*.

¹⁶ Lateef A, Petri M. Unmet medical needs in systemic lupus erythematosus. *Arthritis Research & Therapy* 2012;14(Suppl 4):S4. doi:10.1186/ar3919.

¹⁷ Wallace DJ, *ibid*.

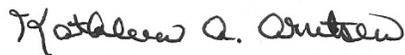
switching and prior authorizations result in treatment delays. Reducing benefits or limiting treatment choices in the Medicare and Medicaid entitlement programs also result in delay in care. Limiting access to vital life-saving medications disrupts continuity of care, especially in vulnerable populations and increases costs because of unnecessary hospitalizations and emergency room visits. Patients endure physical, emotional, and financial distress due to delays in proper treatment, intolerable side effects from inadequate medicine, and initial cost-sharing for ineffective therapy and medical visits.

The lupus community waited 56 years for a new treatment to be approved and within months of it being approved over 30 states had step therapy protocol applied to the drug. Therefore the new treatment did not make its way to the lupus patient. Just think about this. We fight for disease research funding, we struggle to get patients into clinical trials, we battle for treatments to get FDA-approval, and once approved we fight to get them covered. It took us over 50 years to get a new drug approved for lupus but what good is it if physicians cannot get insurers to cover it and patients can't get it. Think of all of the innovative therapies waiting to be discovered and eventually make their way to patients. Poof they will disappear! There are 50 million Americans with autoimmune disease and the incidence is on the rise. We are at a critical juncture right now in getting new treatments for diseases of unmet need. We all need to step up here and become proactive on this issue.

As Americans, we must recognize that public policy decisions have direct implications on the development of new and safer therapies. Drug research and development needs to be encouraged and supported and not stifled by bureaucracies so that all individuals, regardless of race or socioeconomic status can live longer and healthier lives.

After all, lupus and other diseases of unmet need do not just affect patients, but also impacts our loved ones and the health care professionals who treat us. As millions in the lupus community eagerly await the development of new therapies, we passionately urge you to accelerate the development and delivery of new, more efficacious treatments and ultimately a cure for diseases of unmet need like lupus. Most of us living with lupus cling to the belief that there will be better treatments and a cure during our lifetime. We need innovative research initiatives that include cross-sector collaborations, public-private partnerships and robust basic, clinical and translational projects that will enable scientists to investigate disease pathogenesis and physiology, identify biomarkers, design better clinical trial methodologies, prevent complications, develop precise diagnostic measures and safer, more effective treatments, and ultimately eradicate this devastating disease. It is imperative that the next generation of lupus patients is given the chance at a better quality of life and the opportunity to pursue their dreams.

Sincerely,



Kathleen A. Arntsen
Patient, Advocate and President/CEO
Lupus and Allied Diseases Association, Inc.
P.O. Box 170
Verona, N.Y. 13478
315-264-9101

Proposed Amendment to H.R. _____, the “21st Century Cures Act” Discussion Draft Dated January 26, 2015

In Title II (Building the Foundation for 21st Century Medicine, Including Helping Young Scientists), in the appropriate subtitle, add the following new paragraph:

“The National Institutes of Health is authorized to award grants to develop and build a coordinated system of classification and measurement, including the associated biomedical research data, to encompass and identify correlations between the common, combined roles of significant comorbid medical, behavioral, or mental health conditions across the lifespan of individuals with Trisomy 21 (Down syndrome). The comorbid conditions to be considered shall include, but are not limited to, Alzheimer’s disease, intellectual disability, childhood leukemia, congenital heart disease, autism spectrum disorders, sleep apnea and seizure disorders, including epilepsy.”

Explanation

A lack of research on individuals with Down syndrome who have numerous co-occurring and/or simultaneous psychiatric and medical conditions has been an impediment to the development of clinical and behavioral treatments and interventions related to the cognitive function of individuals with Down syndrome. According to the NIH, at least one-half of all children with Down syndrome also have one or more comorbid conditions. During the early years of life and across their lifespan, these comorbid conditions could have the potential to significantly affect cognitive function and overall health.

Authorizing NIH to develop a system of classification and measurement of comorbid conditions would significantly expedite ongoing efforts in the scientific research and medical community to develop effective clinical and behavioral treatments and interventions to ameliorate cognitive dysfunction associated with the intellectual disability specific to the approximately 400,000 people in the U.S. who have Down syndrome. It would also address the health conditions of people in the broader population as common comorbid conditions associated with Down syndrome, such as Alzheimer’s disease, childhood leukemia, congenital heart disease, autism spectrum disorders, sleep apnea and epilepsy, could benefit from a concerted and coordinated interdisciplinary effort to examine their correlation and impact on individuals with Down syndrome.