



AdvaMed commends Chairman Upton and Congresswoman DeGette for their leadership on the 21st Century Cures initiative. We share the goal of accelerating the discovery, development and delivery of cures for patients, and we applaud their leadership on this important issue.

The medical technology industry is central to the development of technologies and diagnostics that will provide the life-saving and life-enhancing treatments of the future. But the innovation ecosystem that supports our industry is severely stressed. Policy improvements are essential if America is to retain its world leadership and the potential for medical progress in this century of the life sciences is to be fulfilled.

Below and attached, you will find both specific and general comments on provisions of interest that were included in the discussion document. We appreciate the opportunity to comment on this discussion document and look forward to continuing to work with the Committee towards this important goal.

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

Subtitle B—Surrogate Endpoint Qualification and Utilization

While we support the intent of this provision, the device center at FDA has long-used surrogate endpoints. We recommend removing the references to devices on p. 18, lines 3,4, and 7; and p. 26, line 7.

Subtitle E—Priority Review for Breakthrough Devices

AdvaMed strongly supports this provision. Investment in development of new medical technologies has declined dramatically in recent years. Moreover, clinical trials and first product introduction of new technologies that are developed have increasingly moved off-shore. This is especially troubling at a time when scientific progress has made development of true treatment breakthroughs more possible than ever before, and when the U.S. leadership in medical technology is increasingly challenged.

This provision would create an effective breakthrough pathway that provides a quick and seamless road to FDA approval and public insurance coverage and payment for medical devices and diagnostics that truly will make important improvements in human health. The proposal would create a stimulus for new investment in development of such products and would assure that once developed they become promptly available to American patients.

AdvaMed is pleased that the discussion document includes a placeholder for extending that pathway to improvements in Medicare's coverage and reimbursement process. AdvaMed has developed a comprehensive proposal to ensure that breakthrough products receive financial incentives to spur innovation and extend access to new products that have the potential to dramatically improve the care and treatment of Medicare beneficiaries.

Our proposal offers a series of improvements for products that receive approval as a breakthrough product under Section 1081. These enhancements include:

1. Transitional Medicare and Medicaid coverage of all products designated by FDA as breakthrough products for a five-year period, with a transitional evidence/data gathering plan, if needed, to help determine appropriate coverage at the end of the five-year period. Coverage also would be required under Medicaid and S-CHIP.
2. Accelerated assignment of appropriate coding and inclusion in Medicare payment systems for designated products on a quarterly basis.
3. Payment should cover the additional costs of the new innovation and reflect a reasonable market rate.
4. Elimination of disincentives for adoption of breakthrough products in Medicare risk-sharing programs

In our proposal, a product designated and approved by FDA as a "breakthrough" product would receive transitional coverage by the Medicare and Medicaid programs at cost for five years. During this period of transitional coverage, the product sponsor is able to collect more data and evidence to support permanent coverage of the technology after the transition period. By the second year, CMS would identify if it believes there are additional data or evidence needs that would be needed to ensure that these products meet their standard of "reasonable and necessary" for coverage. By the end of the fourth year of the transition period, CMS would issue a plan for determining how coverage would be addressed after the 5-year transition.

To facilitate this coverage, CMS would be required to provide and update the appropriate coding and placement of these products in the Medicare and Medicaid program on a quarterly basis. CMS should also modify its risk-sharing programs, such as the Medicare Shared Savings Program, to avoid disincentives for adopting these products. Finally, the proposal would require MA plans to cover these products at a payment rate not less than Medicare FFS rates.

We are committed to working with Committee staff to help ensure understanding of the proposal and development for inclusion in legislation.

Again, we strongly support this provision and offer the attached language, which are edits to the language in the discussion document and additional language regarding Medicare coverage and reimbursement.

Subtitle F—Accelerated Approval for Breakthrough Devices

While we appreciate the intent behind this provision, it is duplicative of the priority review provision in Subtitle E. We recommend removing this provision.

Subtitle G—Expanded Access

While we support the intent of this provision, FDA’s device center has several, effective programs currently in place with the same goal of ensuring expanded access. We recommend removing the reference to devices on p. 86, line 16. Additionally, this provision amends Section 561 of the FFD&CA, which is entitled “Expanded Access to Unapproved Therapies and *Diagnostics*.” (emphasis added). By citing this section of the law, devices are included by default in this provision, so additional changes may be necessary to ensure that devices are not captured in this section.

Subtitle H—Facilitating Responsible Communication of Scientific and Medical Developments

AdvaMed supports the concept of this placeholder to support the sharing of truthful and non-misleading information for the best interests of the public health. We look forward to continuing the dialogue on this important topic.

Subtitle I—Modernizing the Regulation of Social Media

AdvaMed supports appropriate flexibility in social media and internet platforms to convey medical product information. These provisions are consistent with AdvaMed recommendations for appropriate use of hyperlinks in abbreviated formats and more flexible policy to allow for meaningful use of social media and internet. As the intent appears to revise and/or replace current regulation and guidance, we would suggest that the language specifically call for any revised regulation or guidance to be issued separately for devices and drugs/biologics (which have different regulatory frameworks and risk considerations) or alternatively that device-specific statute and regulations, as well as device-specific examples, are appropriately referenced in any new or updated product-wide guidances.

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

Subtitle A—21st Century Cures Consortium Act

The need for another public-private partnership to address what appears to be the same issues as existing public-private partnerships is not clear.

Subtitle B—Medical Product Innovation Advisory Commission

The need for a commission within Congress to address innovation is not clear, nor is how such a commission would function. However, if this provision moves forward, representatives of medical technology companies should be included as Commission members, since they will have the most relevant expertise in assessing barriers to product invention and development and potential remedies.

Subtitle D—Genetically Targeted Platform Technologies for Rare Diseases

We understand these provisions are intended for drug products, but the term “genetically targeted platform technologies” is confusing. To avoid any misunderstanding, this language should be clarified to refer specially to drugs. The term “platform” is device-specific and should be replaced.

Subtitle E—Sensible Oversight for Technology Which Advances Regulatory Efficiency

We share the goal of achieving greater regulatory certainty in this area, and believe that FDA’s recent final guidance in this space was a good step forward. As we evaluate these issues, our input is driven by the core principle that regulation should be platform agnostic. Technology or software that runs on a medical device should be regulated the same way as software that does the same thing, or has the same functionality, that runs on an iPad, PC, phone, or other platform.

We have shared more detailed comments with the offices working on this issue and look forward to our continuing dialogue.

Subtitle F—Building a 21st Century Data Sharing Framework

AdvaMed has several concerns with this subtitle, which are detailed below.

Sec. 2081 – Standardization of Data in CT.gov – AdvaMed is concerned that this provision is vaguely worded and as a result, could be broadly and expansively interpreted by NIH which could impose new and burdensome clinical trial reporting requirements on device manufacturers. It is also not clear what problem is being addressed, as NIH already largely appears to have standardized CT.gov – at times to the detriment of the device industry. The original registry was constructed for drug studies, thus the current requirements are already not aligned with medical device studies; additional standardization could exacerbate this problem. In lieu of the language in Sec. 2081, AdvaMed proposes addressing issues with the underlying statute that have arisen.

Sec. 2082 – Clinical Trial Data System – AdvaMed appreciates that this section is limited to those trials sponsored by entities of the U.S. Dept. of Health and Human Services and those to which the sponsor consents to share their data with the system.

We believe that patient privacy regulations will require the sponsoring entity to provide de-identified data to the Clinical Trial Data System (CTDS) rather than having CTDS perform the

de-identification as appears to be contemplated by the legislation which could be costly for companies that voluntarily participate. In addition, given the growing challenge of maintaining the privacy of patient data in databases and predictions that more such attacks are likely because the data has high value on the black market, current anonymization or de-identification methodologies may be insufficient (i.e., IoM noted in their Jan. 2014 Discussion Framework for Clinical Trial Data Sharing that questions have been raised about the sufficiency of commonly used de-identification strategies). As a result you may want to first ensure that current de-identification methodologies are sufficient before significant efforts to share public clinical trial data, including de-identified patient information proceed.

We are also concerned, this section allows third parties to obtain, analyze, and produce clinical trial information about currently marketed devices. There is no requirement to seek input from manufacturers; nor is there a right of review to evaluate the integrity of the data. There is the possibility of undermining marketed products with flawed data, without independent oversight or an adequate clinical context.

The legislation targets higher education institutes that do not plan to be involved in sponsoring, operating or participating in a clinical trial. We would note that many higher education institutions that would have the expertise to conduct the types of analyses that are envisioned are involved in clinical trials. Eligible entities will also need experimental design, biostatistical and epidemiological expertise which these institutions possess. It is not clear what may be gained by excluding such institutions especially given their analytical expertise.

Sec. 2085 – Expanding Availability of Medicare data – Under this section, use of Medicare data, especially in coordination with registry data, should be prohibited from disclosing patient identifiable information as well as confidential, proprietary data of manufacturers who participate in registries and manufacturers must be given sufficient time to review analyses (i.e., 120 days) in order to appeal or correct errors or to prevent their distribution if they are inaccurate.

Sec. 2091 – Commission on Data Sharing System for Research and Development– Given the objectives of the section which include real-time data migration between provider’s patient electronic health records and registries, protection of patient privacy will be paramount. As noted above there is a growing challenge in maintaining the privacy of patient data in databases. This Commission should be charged with ensuring that current de-identification methodologies are sufficient before significant efforts to share patient level data proceed.

It appears that the objective of the Commission is to provide a report to Congress which may include legislative recommendations. The Commission should also provide legislative recommendations on ways registry data can be used to achieve on-label or new indications for devices (e.g., allow registry data to be used that may not meet FDA’s strict requirements for clinical trial data monitoring, or Part 11 requirements). Similarly, the Commission should provide legislative recommendations on ways to reduce premarket regulatory approval burden in exchange for postmarket participation in a registry.

If the objective of this section is intended to develop general recommendations on processes and procedures related to registries (e.g., ensuring valid data are entered into registries, appropriate data integrity, security standards, access to data, sharing of data, etc.), the legislation should clarify that the Commission's processes and procedures are recommendations not requirements since most registries already have processes and procedures in place governing these activities and requiring compliance with any new methods, processes, etc. would be costly. The Commission should also develop recommendations that consider the effect of data access and release on market approval and patent exclusivity, including the effects upon product innovation which are critical considerations in the device sector. Given the high costs of developing, maintaining and sustaining registries the recommendations should also clarify that registries can charge for access to their data or decline requests for access to data (since many registries perform their own analyses and charge for such analyses in order to finance the registry).

We are also concerned that the process of obtaining stakeholder feedback is inadequate. If Congress were to adopt the Commission's recommendations, there would be little input from stakeholders, specifically manufacturers, whose interests are implicated by the use of data involving their products. We recommend that this section provide for an public meeting and an open comment period for the Commission's proposals.

The language should clarify that Commission members must have expertise in development of registries.

Sec. 2092 – Development and Use of Clinical Data Registries – AdvaMed has specific recommendations for this section. The section specifically tasks the Secretary with considering “how [clinical data registries] should be structured to facilitate the recording and reporting of postmarket data for the purposes of monitoring safety and efficacy of FDA-approved devices and drugs [used in the Medicare program].” To the extent such a registry is developed, the section is silent on critical aspects of the proposed program — such as who can access the registry, who can submit data, whether third parties can analyze and publish reports about the data, and what safeguards are in place to protect potentially confidential and proprietary information. Protections for stakeholders should be added. The section also limits input to “clinical experts” with whom the Secretary must consult before publishing her recommendations. There is no mechanism for stakeholders to provide input before or after the Secretary offers her recommendations, and this closed-off process could affect the quality of those recommendations. An open comment period for stakeholders to provide input should be added.

In addition, since this section contemplates interoperable exchange of information between electronic health records, clinicians and registries, initiatives to ensure current de-identification methods are sufficient should be considered. In addition to the elements listed, the Secretary of HHS should be directed to develop recommendations:

- To ensure that all relevant stakeholders (i.e., clinicians, facilities and manufacturers) have access to their own registry data, including for manufacturers, access to raw data on their medical products.
- On ways registry data can be used to achieve on-label or new indications for devices (e.g., allow registry data to be used that may not meet FDA’s strict requirements for clinical trial data monitoring, HIPAA, or Part 11 requirements).
- On ways to reduce premarket regulatory approval burden in exchange for postmarket participation in a registry.
- Appeals mechanisms for stakeholders to object to the development of a particular registry. There is a growing over-emphasis by governmental agencies to demand participation in registries which can be costly to develop and manage when other approaches (e.g., limited postmarket study, continued follow up of the initial patient cohort for a limited time period, use of OUS data, etc.) would be more appropriate.
- For establishing a clear purpose, objectives, analysis plan, and expected term for registry data collection.
- For ensuring that the purpose and objectives of registry data collection warrant the level of investment required to develop and maintain a registry.
- For ensuring stable and diverse sources of funding to sustain operations of the registry during its expected term of data collection.
- For assessing existing available evidence to determine whether additional data to be collected by the registry are needed.
- For the formation of a data governance committee for the registry, with representation of all stakeholders, that would establish:
 - procedures for ensuring that valid data are entered into the registry;
 - procedures for ensuring data integrity and security;
 - procedures for defining end points for data collection;
 - rules for access to data, use of data, and publication of registry data;
 - rules for protecting patient privacy and confidentiality of hospital, physician, and manufacturer data.

As mentioned in the AdvaMed comment on Sec. 2091 the legislation should clarify that the HHS recommendations are not requirements since most registries already have processes and procedures in place governing these activities and requiring compliance with any new methods, processes, etc. that may be potentially conflicting would be costly.

Subtitle H—Coverage With Evidence Development

Section 2121 attempts to give CMS explicit authority to use CED, including authority to carry out CED at the local level. CED is an important issue to our members and for ensuring access to new technologies and diagnostics. AdvaMed has concerns about the current language in the discussion draft. AdvaMed has been working with Committee staff and other stakeholders to revise the language. AdvaMed members are still working through our recommendations and, at this time, do not have specific recommendations. We will send additional recommendations on this section to the Committee at a later time.

Subtitle I—Combination Products

We greatly appreciate the inclusion of this topic. We believe that there are several issues related to the current regulation of combination products at FDA that should be addressed. On the pre-designation side, FDA has been equating any chemical action of the product as having a drug primary mode of action when such a determination often is not scientifically justified. On the post-designation side, there is a need for collaborative discussions and agreements on data requirements; questions and data requirements often go beyond what is required to address the incremental risk of the combination of the constituents; and the roles and responsibilities of the Office of Combination Products and the review centers (primary and consulting) in obtaining alignment, resolving disputes and meeting deadlines are not clear and must be better defined and implemented.

We look forward to a continuing dialogue on these important issues.

Subtitle J—Modernizing Regulation of Diagnostics

AdvaMed appreciates the Committee's interest in improving the regulatory oversight of all diagnostics. AdvaMed has long supported FDA risk based oversight of all diagnostics tests, regardless of where they are made (i.e., laboratory-developed tests). We greatly appreciated the opportunity to provide comments as part of the 21st Cures request for feedback on a modernized framework for diagnostic tests. We welcome continued constructive exchange on the issue of diagnostics regulation to promote the public health and innovation.

Should the committee choose to include additional provisions related to diagnostics, we continue to recommend the following principles that reflect AdvaMedDx's Risk-Based Approach to regulation for all diagnostics, regardless of where a test is developed. We believe such an approach serves to balance the need for patient safety and continue innovation.

The degree of regulation needed to ensure the safety or effectiveness should be determined by the risk associated with the test. For diagnostics, risk assessment considers harm that could occur if test results are incorrect. A risk based approach allows the Agency to focus resources on tests that pose the highest risk to patients while at the same time expediting patient access to lower risk tests—regardless of where made—by more efficient use of premarket review process.

AdvaMedDx has long reiterated that efforts must be undertaken to assure that tests should be cleared or approved through an approach where the data submission requirements are commensurate with the level of risk of the test. With respect to specific application of risk based oversight for all diagnostics, the following elements should be considered:

1. Clinical use of a test (risk associated with how the test is used in the treatment of patients)—e.g., seriousness or prevalence of the condition, prevalence of condition, reversibility of intervention, or standalone use (not supplementary to other clinical information);
2. Novelty of analyte (the substance that is undergoing analysis or is being measured);

3. Novelty of technology (or test platform);
4. Experience or training of the person performing the test; and
5. Factors that reduce or mitigate risk—e.g., scientific information, literature, general and/or special controls.

The first four considerations are risk elements. Data that mitigates risk should be considered as available for all four categories and may be different (e.g., literature for 1 and 2, experience of FDA for 3, human factors studies/design elements for 4). The last consideration would embrace the use of valid scientific information to support clinical validity for the regulatory review process.

We view the issue of risk-based regulatory review and associated submission requirements for all diagnostics as part of a modernized framework as a vitally important issue for which industry continues to explore. We appreciate the Committee's interest and would be happy to discuss these and other concepts to best support a modernized approach.

Subtitle L—NIH—Federal Data Sharing

This section may impact small device companies who rely on NIH funding and it may chill innovation in the device sector. Small companies – which face the greatest funding challenges and who may rely on NIH funding – account for many device innovations and contribute greatly to maintaining strong health care price competitiveness. It is hard to see how the data would not reveal company commercial/financial information particularly next generation devices that would be developed utilizing the underlying trial data.

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

This provision is intended to allow the greater access to protected health information (PHI) by a “covered entity” for research purposes, including studies to attain generalized knowledge and comparative effectiveness research. While easier and greater access to PHI for research purposes ideally could benefit public health and industry, there are major issues with the language as written that would require a lot of work to resolve conflicts with existing regulation, including the following:

- The provision is in conflict with, and negates the protections afforded to subjects by the informed consent requirements (21 C.F.R. § 50.20) because it allows for an investigator to obtain one PHI authorization from a subject for all future research, and not just for each individual device study. This essentially prohibits the subject from knowing who will conduct future research, who will view the PHI, and how well the covered entities who access the data can maintain confidentiality.
- The provision allows a covered entity to use or disclose a limited data set for research purposes without the use of a data use agreement. This negates the protections afforded by a data use agreement, which sets forth permitted uses and disclosures; limits who can use or receive data; requires the recipient to agree to not disclose the data other than for

uses permitted by the agreement; and uses appropriate safeguards to prevent disclosure of the PHI.

Subtitle P—Fostering High-Risk, High-Reward Science

AdvaMed supports steady growth in funding for NIH, specifically for the Small Business Innovation Research and Small Business Technology Transfer programs, and it is unclear where appropriate funding to implement this provision will come from. Because the discussion document does not speak to an increase in overall NIH-wide funding, there is an open question as to whether the additional funds reserved to implement this program would need to come from somewhere else if NIH funding remained flat in years to come. Additional funding to support such programs should not be pulled from existing small business programs.

Subtitle Q—Precision Medicine

AdvaMedDx supports a greater focus on increased investments in precision medicine. Innovations in diagnostic tests, including genetic sequencing technologies, are a cornerstone of precision medicine. Diagnostic tests are the key to ensuring the right treatment for the right patient at the right time. We look forward to a continued dialogue on this important initiative.

Title III—Modernizing Clinical Trials

Subtitle A—Clinical Research Modernization

AdvaMed appreciates the inclusion of language to voluntarily allow the use of central IRBs by striking the requirement for local IRBs in the statute. Obtaining local IRB (Institutional Review Board) approval to conduct a multicenter clinical trial can be a major element in extending the time and cost of a clinical trial. The time involved in obtaining consent from different IRBs for a multicenter trial can raise the cost of device development. When the IRB at any one of the trial sites requires a change to the research protocol, investigators must resubmit the revised protocol to the IRBs at all the participating sites. Moreover, simply negotiating the protocol with numerous IRBs rather than one can be time-consuming.

However, we are concerned that the forgoing language of Sec. 3001 undermines this important objective with its emphasis upon incorporating community values through the use of local IRBs. It is unclear what objective Sec. 3001 seeks to address and we are unaware of any particular concerns governing human subject protection that are not already well-addressed by existing HHS regulations and guidance (the latter which is routinely updated). There is already a focus on vulnerable populations by HHS. We are concerned the vague and expansive language of Sec. 3001 will allow the Sec. of HHS to essentially re-interpret longstanding rules governing protection of human subjects which may have a negative impact on recruitment for clinical trials and their conduct. The objective and/or effect of stating that parts 50, 56, 312 and 812 are not subject to Subpart A of part 46 of Title 45 CFR is also unclear.

Subtitle B—Broader Application of Bayesian Statistics and Adaptive Trial Designs

The broader use of Bayesian methods and adaptive design for clinical trial designs should be encouraged. Both methods can reduce the number of subjects enrolled, shorten the length and reduce the cost of a clinical trial. It would be helpful to have the FDA expectations for the use of these methods clearly defined in guidances.

There is an existing guidance on the use of Bayesian methods for medical devices, so there is no need to include devices in a new guidance. However, the current guidance for devices could be reviewed to determine if updating is needed.

The use of adaptive design for medical devices should be addressed in a guidance. This guidance should be specific to medical devices; separate from the guidance for drugs and biologics.

Subtitle D—Pediatric Research Network Improvement

There is a strong network of pediatric consortia, some of whom may be developing devices for pediatric or rare diseases that may be impacted by the proposed changes in this provision. Although the changes may be helpful, the full effects of the proposed changes are unclear, as it is not clear to us what base law provision is being amended.

Subtitle E—Global Pediatric Clinical Trial

We are supportive of this section as it could be helpful in the development of pediatric medical devices. It can be challenging to conduct trials in pediatric populations which may not have sufficient numbers to accrue statistically meaningful clinical data over a reasonable timeframe and within a manageable number of investigational sites. To have meaning and impact, this provision may need to be revised to authorize NIH and FDA to implement the network.

Relying on its authority to utilize valid scientific evidence that meets the standard of reasonable assurance of safety and effectiveness (other than well-controlled trials), FDA should also be authorized to develop new regulatory pathways to accommodate device development for small populations (pediatric and rare diseases).

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

Subtitle A—National Institutes of Health

Section 4002 -- Biomedical Research Working Group to Reduce Administrative Burden on Researchers

AdvaMed supports the establishment of such a working group; however, we recommend the development of an unbiased selection process and inclusion of industry representation on the working group.

Section 4007 -- Additional Funding for NIH Common Fund

AdvaMed supports steady growth in funding for NIH, and we especially highlight the need for increases in the Small Business Innovation Research and Small Business Technology Transfer programs. We also support increases in Common Fund appropriations, as long as they do not come at the expense of existing small business programs, which are an important avenue for supporting development of innovative diagnostics, treatments and cures with immediate application to patient care.

Section 4009 -- NCATS Phase IIB Restriction

AdvaMed supports this provision. With that being said, funds to support the implementation of this provision should not be pulled from existing small business programs (please refer to our comment in Section 4007).

Subtitle G—Disposable Medical Technologies

Section 4151 represents language that AdvaMed and member companies have been working on to address shortfalls in the current durable medical equipment benefit. AdvaMed strongly supports this provision to provide Medicare coverage for disposable medical technologies that substitute for durable items and we believe this will go a long way to modernizing the benefit in a way that reflects current technologies available in the home. With this provision, Medicare beneficiaries will have access to technologies that were not available at the time the program was created and that will allow patients to be served in the least restrictive and most cost effective setting of their homes. Payments for the disposable technology would be set at 95% of amounts paid for the durable item. We look forward to continuing to work with staff on additional technical modifications as bill language continues to move forward.

Subtitle H—Local and National Coverage Decision Reforms

AdvaMed strongly supports the inclusion of Section 4161. AdvaMed has concerns about transparency and process used by Medicare contractors for developing local coverage policies and we believe the proposed language represents AdvaMed's recommendations to improve the LCD process. We would recommend deleting the reference to "National" coverage decisions in

the title of the section and note that the section language applies only to the Local Coverage Determination process.

Subtitle I—Telemedicine

This section would expand telehealth services to additional services and remove the barriers related to geographic location and originating site. AdvaMed supports the Committee's intent to expand telemedicine services. We are concerned that this expansion would be very limited and misses an opportunity to fully embrace the benefits of today's telehealth services and their ability to improve care and lower health spending. AdvaMed recommends an alternative approach to meet the dual goals of expanding beneficiary access to telehealth services while maintaining fiscal responsibility for the Medicare program.

AdvaMed recommends that coverage for a new benefit be established to allow for use of telehealth services without overly restrictive limitations on the type of technology used or the site of service. Reimbursement would be based on a new and separate telehealth fee schedule. This new benefit would be available through transitional coverage over a 5-year period, during which time the redesigned benefit and its covered services would be required to meet quality standards and demonstrate Medicare savings. CMS would continue coverage at the end of 5 years if such requirements are met. Finally, AdvaMed recommends that the Secretary would be authorized to cover and pay for telehealth services in ACO, Bundled Payment, and other demonstration projects or models without regard to limitations that apply to these services under current Medicare law, so long as they do not increase Medicare spending beyond levels that would occur in the absence of the expanded benefits. We are working with the committee offices involved in shaping this section of the Cures legislation, and look forward to continuing that dialogue.

Subtitle J—Revise IPPS New Technology Add-on Payment (NTAP) Reimbursement Amounts

Section 4201 allows for an appeal to be completed in 90 days for New Technology Add-on Payment applications. Separately, the provision includes language that would require use of NDC codes (instead of HCPCS Level II codes) for drugs and biologicals for coverage, coding and payment. This provision for drugs and biologicals does not appear to be related to NTAP.

AdvaMed supports adding an appeals process for NTAP determinations. We recommend an additional complementary provision to require that CMS implement, within a very short timeframe (e.g. 30 days), add-on payments for decisions that have been overturned on appeal. We would also encourage the Committee to expand these provisions to apply as well to the pass-through process for outpatient hospital care.

AdvaMed also recommends that the Committee make additional improvements to the NTAP program to address serious flaws in CMS's implementation of the program. A recent study published in the February 2015 issue of Health Affairs highlighted that CMS has allowed only 19 products to qualify for the NTAP innovation incentives and that spending on the program has been at a dismal 35% of CMS's estimates of spending. Important changes are needed to ensure

that patients have access to high-cost new technologies. AdvaMed recommends several changes, including:

1. CMS should use the most recently available cost data and information available (including data from surveys of providers of services and suppliers conducted by the Secretary, private payers, health plans, physician specialty societies or manufacturers as well as commercial price data and data from manufacturer invoices) for assigning new technology procedures to appropriate MS-DRGs.
2. Increase new technology add-on payments (NTAP) from 50 to 100%; a recent study found, that from 2002 through 2013, CMS projected NTAP spending of \$598 million, but analysis of MedPAR data showed that hospitals received only \$201.7 million over the entire 12-year period.
3. Lower the cost threshold for qualifying for NTAP. Currently the incremental cost of a new technology must exceed the lesser of 75 percent of the standard MS-DRG payment amount and 75 percent of one standard deviation above the charge for the MS-DRG or DRGs to which the technology is assigned. This cost threshold establishes a high bar for eligibility and should be reduced to the lesser of 50 percent of standard MS-DRG payment amount and 50 percent of one standard deviation above the charge for the MS-DRG or DRGs to which the technology is assigned.
4. Modify the criteria for “newness” so certain devices are not unfairly excluded from NTAP. For the newness test, CMS considers (1) whether a product uses the same or a similar mechanism of action to achieve a therapeutic outcome, (2) whether a product is assigned to the same or a different MS-DRG, and (3) whether a new use of the technology involves the treatment of the same or similar type of disease and the same or similar patient population. The criteria for determining whether a product meets the newness test should be expanded to include products that involve a significant technological change that do not raise different questions of safety and effectiveness (in comparison to the predicate device) and result in enhanced clinical advantages or reduced costs over an episode of care (outside the payment for the indexed MS-DRG), even though they use the same or similar mechanism of action or are assigned to the same MS-DRG. In addition, the Secretary should not disqualify a new service or technology as not meeting the newness criterion on the basis of a finding that fewer than 50 claims for payment for the new service or technology exist in Medicare claims data.
5. Criteria applied in making substantial improvement determinations. In order to be eligible for add-on payments, a new technology must represent an advance that substantially improves, relative to technologies previously available, the diagnosis or treatment of Medicare beneficiaries. In making this determination, the Secretary should consider whether the new technology or medical service meets one or more of the following criteria: (a) provides more effective treatment or diagnosis of life-threatening or irreversibly debilitating human diseases or conditions; (b) no approved alternatives exist; (c) offers significant advantages over existing approved alternatives (d) results in reduced costs over an episode of care; (e) improves patient quality of life; (f) creates long-term clinical efficiencies in

treatment; (g) addresses patient-centered objectives as defined by the Secretary (h) meets such other criteria as the Secretary may specify.

6. Simplification of process for defining period of “newness” for purposes of add-on payments. Under current policy, the period of “newness” for a technology or medical service to receive add-on payments is based generally on the date of FDA approval, and not when the assignment of a new ICD code allows specific identification of the new technology in claims data. Although CMS has taken steps to consider delaying the start of the newness period in cases where an applicant can demonstrate a documented delay in market availability, CMS should simplify the process by requiring the use of the later of either the assignment of a new code or FDA approval as the controlling date for starting add-on payments.

Subtitle K—Lowering Medicare Patients OOP Costs

AdvaMed supports building a transparent value-driven health care system to improve quality of care, increase efficiency, and promote informed patient choice. AdvaMed believes that transparency initiatives should strive to provide consumers and other purchasers of health services with tools to make informed decisions. It should be understood that transparency is not an end in itself but a mechanism to achieve specific goals that will require different tools for different health sectors, processes, and transactions. Transparency initiatives should be tailored to the goal they are trying to achieve and only implemented if they have been designed, developed, and assessed for the likely impact on patients, the health care system, and future innovation.

Greater transparency should recognize that value is a function of both quality and cost. Patients cannot determine the value of care based solely on its cost. Public reporting of cost information should be accompanied by reliable and relevant quality information that provides an accurate representation of the true quality of the care provided. Additionally, to the extent possible, cost measures should be based on the resources needed to deliver a group of services, or entire episode of care. Transparency initiatives, such as the language in this section, should focus on patients’ and other purchasers’ need for information and the focus of this section on patient out-of-pocket costs is a good measure to help patients make informed decisions.

Subtitle L—Global Surgery Services Rule

This provision prevents the Secretary from enforcing provisions contained in the CY2015 Final Physician Fee Schedule (PFS) rule related to global payment policy changes. AdvaMed did not take a position on this issue in the CY 2015 proposed PFS rule. However, we are concerned that any change in payment policy related to global payments not affect patient access to appropriate care.

Subtitle M—Providers Consolidation and Medicare Payments Examined Through Evaluation

Section 4261 would require the Secretary to seek public comment on and to evaluate how changes to Medicare payment systems will affect provider consolidation. Provider consolidation is an important issue and can have a significant impact on patient access and affordability. AdvaMed recommends that the Federal Trade Commission continue to be the lead agency in evaluating issues of provider consolidation.

Subtitle P—Medicare Pharmaceutical and Technology Ombudsman

Section 4321 provides for a pharmaceutical and technology ombudsman that would receive and respond to complaints, grievances and requests from drug, biologic, device, and diagnostic manufacturers regarding Medicare payment and coverage decisions. The ombudsman would report to Congress on activity annually.

AdvaMed generally supports the creation of this new ombudsman role and we recommend that the Committee include certain parameters to enhance effectiveness of this new office. We recommend including language describing how the new ombudsman must respond to such complaints or grievances, particularly when those complaints or grievances are related to Medicare decisions that could harm patients or limit patient access to new technologies. Example language could include:

“The ombudsman shall evaluate and respond to complaints or grievances received from manufacturers, and assist in the resolution of such complaints or grievances. The ombudsman shall identify specific and systemic issues and make recommendations to the Secretary [or to Congress] to improve processes that will ensure appropriate coverage, coding and payment determinations, and ensure that Medicare beneficiaries have access to appropriate clinical applications for new technologies. The ombudsman shall create a process for tracking the receipt and disposition of grievances and shall provide an opportunity for public input. The annual report shall include information regarding the volume of cases handled by the new office, as well as information regarding the average time spent on cases, how such cases are resolved and other activities carried out by the office of the ombudsman.”

Subtitle Q—Ensuring Local Medicare Administrative Contractors Evaluate Data Related to Category III Codes

The language in Section 4341 partially addresses concerns of our members and providers regarding the current problems with coverage and payment for Category III codes under Medicare. The draft addresses our concern related to Medicare contractors adequately evaluating the data related to Category III codes prior to making a coverage determination. However, the draft language fails to address our concerns that the contractors routinely deny these services simply because they are designated by Category III codes .

AdvaMed supports the Committee’s efforts to improve the coding process. In addition to the current proposal, AdvaMed also recommends that the Committee add additional language

requiring contactors to cover these codes unless a formal explanation and rationale for non-coverage is provided. Medicare’s contractors frequently deny Category III codes without any review of the merit for coverage and payment. This provision should be amended to require that contractors not be permitted to deny coverage for these codes without first conducting an evaluation of all relevant data.

Specific legislative changes and additions are below:

Page 323, beginning on line 22, strike subparagraph (D) and insert in lieu of thereof the following new subparagraph (D):

“(D) DATA EVALUATION REQUIREMENTS FOR LOCAL COVERAGE DETERMINATIONS.—The Secretary shall include the following requirements as part of the requirements developed under subparagraph (A) for a medicare administrative contractor performing the function of developing local coverage determinations (as described in subsection (a)(4)) with respect to an item or service:

“(i) DATA EVALUATION FOR CATEGORY III CODES.—In the case of an item or service included as a Current Procedural Terminology Code that is a Category III Code, the contractor shall not deny coverage of such item or service through a local coverage determination before evaluating all data related to such code.

“(ii) DATA EVALUATION FOR CATEGORY I CODES CONVERTED FROM CATEGORY III CODES.—In the case of an item or service included as a Current Procedural Terminology Code with respect to which the Category III Code is converted to a Category I Code, the contractor shall not deny coverage of such item or service through a local coverage determination before evaluating all data related to such Category I Code.”.

Subtitle S—Continuing Medical Education Sunshine Exemption

We support this provision. These clarifications would ensure that physicians can continue to receive information and educational training related to medical devices. For your consideration, we have attached suggested edits to align w the statutory scope and reporting cadence.

Subtitle T—Medical Testing Availability

While the FDA final Research Use Only (RUO) guidance was significantly revised and consistent with industry expectations, we understand this provision is intended to reinforce appropriate application of RUO policy. We appreciate the efforts to support lawful manufacture of research use only products and appropriate communications regarding functioning of the product (e.g., technical support, instructions for use).

Additional Recommendation for Issues NOT Included in the Discussion Document:

Medicare Coverage of FDA-Approved Investigational Device Exemption (IDE) Trials

While not included in the discussion draft, AdvaMed recommends that the Committee include a proposal (draft legislative language attached) that would require CMS automatically to provide Medicare coverage for an FDA-approved Investigational Device Exemption (IDE) clinical trial, after the FDA has approved the IDE. Since 1995, CMS contractors have been responsible for reviewing and making decisions regarding coverage of FDA-approved IDE trials; however, as of January 1, 2015, CMS has implemented a centralized review process for reviewing and approving coverage for these studies. Historically, CMS has covered routine costs of Medicare beneficiaries in the vast majority of trials.

CMS also automatically covers the routine costs of Medicare beneficiaries participating in certain drug trials, including investigational new drug application (IND) trials that are reviewed by the FDA, and drug trials that are exempt from having an IND. These trials are deemed to meet Medicare's qualifying criteria for coverage, while device trials are subject to review by CMS.

AdvaMed proposes that CMS automatically cover FDA-approved IDE trials, including both routine care costs (for Category A and B) and the Category B device cost, after the IDE is approved by the FDA.

Title V—Modernizing Medical Product Regulation

Subtitle D—Medical Device Reforms

We strongly support these provisions, which would add consistency and predictability in the FDA device review process. We are currently discussing these provisions with FDA, and we look forward to further discussions on them with the Committee.

Subtitle E—Supply Chain Security for Devices

While we understand the intent of this provision is to ensure secure distribution of medical devices, it is unclear why additional regulations are necessary when existing FDA rules and other bodies oversee the safe distribution of prescription devices. We are concerned that some of our companies might be negatively impacted, specifically through unnecessary costs and burdens without an added benefit to safety. We are continuing to study this provision and its impact, and look forward to discussing this issue.

AdvaMed Comments
21st Century Cures Discussion Document
Suggested Language

Title I—Subtitle E—Priority Review for Breakthrough Devices

SEC. 1081. PRIORITY REVIEW FOR BREAKTHROUGH DEVICES.

Chapter V of the Federal Food, Drug, and Cosmetic Act is amended—

(1) in section 515(d)—

(A) by striking paragraph (5); and

(B) by redesignating paragraph (6) as paragraph (5); and

(2) by inserting after section 515A (21 U.S.C. 360e–1) the following:

“SEC. 515B. PRIORITY REVIEW FOR BREAKTHROUGH DEVICES.

“(a) IN GENERAL.—In order to provide for more effective treatment or diagnosis of life-threatening or irreversibly debilitating human diseases or conditions, the Secretary shall establish a program to provide priority review for devices—

“(1) representing breakthrough technologies;

“(2) for which no approved alternatives exist;

“(3) offering significant advantages over existing approved or cleared alternatives, ~~;~~ or including the

~~“(4) the availability of which—~~

~~“(A) has~~ the potential to, compared to existing approved alternatives, reduce or eliminate the need for hospitalization, improve patient quality of life, facilitate patients’ ability to manage their own care (such as through self-directed personal assistance), or establish long-term clinical efficiencies; or

“(4B) the availability is ~~otherwise~~ in the best interest of patients.

“(b) REQUEST FOR DESIGNATION.—A sponsor of a device may request that the Secretary designate the device for priority review under this section. Any such request for designation may be made at any time prior to ~~or, concurrently with, or subsequent to,~~ the submission of an application under section 515(c), a petition for classification under section 513(f)(2), or a notification under section 510(k).

“(c) DESIGNATION PROCESS.—

“(1) IN GENERAL.—Not later than 60 calendar days after the receipt of a request under subsection (b), the Secretary shall determine whether the device that is the subject of the request meets the criteria described in subsection (a). If the Secretary determines that the device meets the criteria, the Secretary shall designate the device for priority review.

“(2) REVIEW.—Review of a request under subsection (b) shall be undertaken by a team that is composed of experienced staff and managers of the Food and Drug Administration and is chaired by a senior manager.

“(3) DESIGNATION DETERMINATION.—In issuing a determination approving or denying a request under subsection (b), the Secretary shall provide a written, substantive summary of the basis for the determination.

“(4) RECONSIDERATION ~~BY DIRECTOR OF CENTER FOR DEVICES AND RADIOLOGICAL HEALTH.—~~

“(A) REQUEST FOR RECONSIDERATION.—Any person whose request under subsection (b) is denied may, within 30 days of the denial, request reconsideration of the denial ~~by the Director of the Center for Devices and Radiological Health—~~

“(i) based upon the submission of documents by such person; or

“(ii) based upon such documents and a meeting or teleconference.

~~“(B) **DIRECTOR’S** RESPONSE.—A designation determination is a significant decision subject to the appeal provisions of Section 517A including the timeframes for issuing a response to the appeal. The Director of the Center for Devices and Radiological Health shall respond to a request under subparagraph (A)—~~

~~“(i) in the case of a request for reconsideration described in subparagraph (A)(i), not later than 30 days after the date on which the Director receives the request; or~~

~~“(ii) in the case of a request for reconsideration described in subparagraph (A)(ii), not later than 30 days after the date of the meeting or teleconference.~~

“(5) WITHDRAWAL.—If the Secretary approves a priority review designation for a device under this section, the Secretary may not withdraw the designation based on the fact that the criteria specified in subsection (a) are no longer met because of the subsequent clearance or approval of another device that was ~~previously approved for such designation~~ under this section or section 515(d)(5) ~~prior to (as in effect on the day before the date of~~ the enactment date of the 21st Century Cures Act).

“(d) PRIORITY REVIEW.—

“(1) ACTIONS.—For purposes of expediting the development and review of devices designated under subsection (c), the Secretary shall—

“(A) assign a team of staff, including a team leader with appropriate subject matter expertise and experience, for each device for which a request is submitted under subsection (b);

“(B) provide for oversight of the team by senior agency personnel to facilitate the efficient development of the device and the efficient review of any submission described in subsection (b) for the device;

“(C) adopt an efficient process for timely dispute resolution;

“(D) provide for interactive communication with the sponsor of the device during the review process;

“(E) expedite the Secretary’s review of manufacturing and quality systems compliance, as applicable;

“(F) if the Secretary intends to consult with external experts or an advisory committee concerning the sponsor’s device—

“(i) disclose to the sponsor of the device in advance the topics of any such consultation; and

“(ii) provide an opportunity for the sponsor to recommend such external experts;

“(G) for applications submitted under section 515(c), provide for advisory committee input, as determined by the Secretary or at the request of the sponsor, as appropriate; and

“(H) assign staff to be available within a reasonable time to address questions by ~~communicate with~~ institutional review committees concerning the conditions and clinical testing requirements applicable to the investigational use of the device pursuant to an exemption under section 520(g).

“(2) ADDITIONAL ACTIONS.—In addition to the actions described in paragraph (1), for purposes of expediting the development and review of devices designated under subsection (c), the Secretary, in collaboration with the device sponsor, may, as appropriate—

“(A) coordinate with the sponsor regarding early agreement on a data development plan;

“(B) take steps to ensure that the design of clinical trials is as efficient as practicable, such as through adoption of shorter or smaller clinical trials, application of surrogate endpoints, and use of adaptive trial designs and Bayesian statistics, to the extent scientifically appropriate;

“(C) facilitate, to the extent scientifically appropriate, expedited and efficient development and review of the device through utilization of timely postmarket data collection, ~~with regard to applications for approval under section 515(c) and petitions for classification under section 513(f)(2)~~; and

“(D) agree to clinical protocols that the Secretary will consider binding on the Secretary and the sponsor, subject to changes agreed to by the sponsor and the Secretary or other changes that the Secretary determines are required to prevent an unreasonable risk to the public health or if a substantial scientific issue essential to the safety or effectiveness of the device involved has been identified.

“(e) PRIORITY REVIEW GUIDANCE.—

“(1) CONTENT.—The Secretary shall issue guidance on the implementation of this section. Such guidance shall include the following:

“(A) The process for a person to seek a priority review designation.

“(B) A template for requests under subsection (b).

“(C) The criteria the Secretary will use in evaluating a request for priority review.

“(D) The standards the Secretary will use in assigning a team of staff, including team leaders, to review devices designated for priority review, including any training required for such personnel on effective and efficient review.

“(2) PROCESS.—Prior to finalizing the guidance under paragraph (1), the Secretary shall propose such guidance for public comment.

~~“(f) PREDICATE DEVICES.—If a device has been classified in response to a petition for classification under section 513(f)(2) pursuant to priority review under this section, and such classification and review includes the use of postmarket data collection pursuant to subsection (d)(2)(C), the device may not be cited as a predicate device for purposes of determining substantial equivalence under section 513(f) unless such postmarket data collection has been completed.~~

“(g) CONSTRUCTION.—

“(1) PURPOSE.—This section is intended to encourage the Secretary and provide the Secretary sufficient authorities to apply efficient and flexible approaches to expedite the development of, and

prioritize the agency’s review of, devices that represent breakthrough technologies.

“(2) CONSTRUCTION.—Nothing in this section shall be construed to alter the criteria and standards for evaluating an application pursuant to section 515(c), a report and request for classification under section 513(f)(2), or a report under section 510(k), including the recognition of valid scientific evidence as described in section 513(a)(3)(B), and consideration of the least burdensome means of evaluating device effectiveness or demonstrating substantial equivalence between devices with differing technological characteristics, as applicable. Nothing in this section alters the authority of the Secretary to act on an application pursuant to section 515(d) before completion of an establishment inspection, as the Secretary deems appropriate.”.

SEC. 1082. CMS COVERAGE OF BREAKTHROUGH DEVICES

1 SEC. 2. COVERAGE AND PAYMENT FOR BREAKTHROUGH

2 PRODUCTS UNDER THE MEDICARE PRO- 3 GRAM.

4 (a) IN GENERAL.—Part E of title XVIII of the Social
5 Security Act (42 U.S.C. 1395x et seq.) is amended by add-
6 ing at the end the following new section:

7 “SEC. 1899C. MEDICARE COVERAGE OF BREAKTHROUGH 8 PRODUCTS.

9 “(a) BREAKTHROUGH PRODUCTS.—The term ‘break-
10 through product’ has the meaning given such term in sec-
11 tion [515(d)(5)] of the Federal Food, Drug, and Cos-
12 metic Act.

13 “(b) COVERAGE.—

14 “(1) TRANSITIONAL COVERAGE.—The Secretary
15 shall provide for coverage under this title of a break-

16 through product for the 5-year period that begins on
17 the date of the approval or clearance of such break-
18 through product by the Food and Drug Administra-
19 tion for any medically accepted indication approved
20 by the Food and Drug Administration for such
21 breakthrough product during such 5-year period.
22 During such 5-year period, the coverage with evi-
23 dence development process shall not be required for
24 a breakthrough product for any medically accepted
25 indication approved by the Food and Drug Adminis-
26 tration for such product.

1 “(2) DETERMINATION OF COVERAGE AFTER 5-YEAR
2 PERIOD.—For

3 purposes of the application of section 1862(a)(1) to
4 breakthrough products furnished after the 5-year pe-
5 riod provided for under paragraph (1), the Secretary
6 acting through the Council for Technology and Inno-
7 vation (established under section 1868(b)) in con-
8 junction with the Coverage and Analysis Group of
9 the Centers for Medicare & Medicaid Services shall
10 determine during such
11 5-year period the need for additional
12 evidence to demonstrate compliance
13 with such section for purposes of coverage of such
14 breakthrough products under this title after such 5-
15 year period as follows:

16 “(A) IDENTIFICATION OF ADDITIONAL EVI-
17 DENCE.—

18 “(i) IN GENERAL.—Not later than 2
19 years after the date of the approval or
20 clearance of a breakthrough product by the
21 Food and Drug Administration, the Sec-
22 retary shall identify whether any additional
23 data or evidence is required with respect to
24 medically accepted indications for a break-
25 through product for purposes of the appli-
26 cation of such section 1862(a)(1) to the
 breakthrough product for such indications.

Title IV—Subtitle S—Continuing Medical Education Sunshine Exemption

Section 4381(a)(1):

- **Explanation:** Given CMS’s previously narrow interpretation of the “educational materials” exclusion, we note that the agency could likewise adopt a narrow construction of the revised exclusion as drafted—for example, interpreting the exclusion as applying only to reprints/textbooks that are for “patient use,” or adopting an interpretation in which some (but not all) reprints/textbooks are considered to “directly” benefit patients. To avoid any potential misunderstanding about the scope of the exclusion, E&C could amend section 4381(a)(1) in one of two ways.

- **Proposed Technical Edits:**

OPTION #1: Rather than amend the existing exclusion (Social Security Act section 1128G(e)(10)(B)(iii)), create a clean, stand-alone exclusion for reprints/medical textbooks, as follows:

Modifying Section 4381, Subsection (a), Paragraph (1) of the Discussion Document legislative text as follows:

- (1) ~~in clause (iii), by inserting~~ by adding at the end the following new clause “~~including~~ (xiii) peer-reviewed journals, journal reprints, journal supplements, and medical textbooks.” ~~after “patient use”;~~ and

The effect of this on the current statute would be:

42 U.S. Code § 1320a–7h - Transparency reports and reporting of physician ownership or investment interests

(e) Definitions

(10) Payment or other transfer of value

(B) Exclusions - An applicable manufacturer shall not be required to submit information under subsection (a) with respect to the following:

...

(xiii) peer-reviewed journals, journal reprints, journal supplements, and medical textbooks.

OPTION #2: (a fallback option, should the standalone exclusion proposal in option 1 not be feasible) Modifying Section 4381, Subsection (a), Paragraph (1) of the Discussion Document legislative text as follows:

- (1) in clause (iii), by inserting “, including peer-reviewed journals, journal reprints, journal supplements, and medical textbooks” after ~~“patient use”~~ “Educational materials” and by striking the word ~~“directly”~~; and

The effect of this on the current statute would be:

42 U.S. Code § 1320a–7h - Transparency reports and reporting of physician ownership or investment interests

(e) Definitions

(10) Payment or other transfer of value

(B) Exclusions - An applicable manufacturer shall not be required to submit information under subsection (a) with respect to the following:

...

(iii) Educational materials, including peer-reviewed journals, journal reprints, journal supplements, and medical textbooks, that directly benefit patients or are intended for patient use.

Section 4381(a)(2) [p. 345, lines 1-3]:

- Explanation: These edits are intended to: (i) clarify that the exception does not apply only to grants that subsidize the cost of a physician’s attendance at a CME program but also applies, for instance, to grants that fund honoraria to CME faculty; (ii) clarify that the exception applies to “continuing education” for any type of “physician” covered by the Sunshine Act; and, (iii) limit this exception to “accredited and/or certified” continuing education, consistent with the concerns that CMS addressed in the Preamble to the final Sunshine Act rule (78 Fed. Reg. 9458, 9479 (Feb. 8, 2013)).
- Proposed technical edits: Modifying Section 4381, Subsection (a), Paragraph (2) of the Discussion Document legislative text as follows:

(2) by adding at the end the following new clause:

~~“(xiii)”~~(xiv) A transfer of anything of value to a covered recipient who is a physician if the thing of value is intended solely for purposes of providing accredited and/or certified continuing ~~medical~~ education ~~to the physician~~.”.

The effect of this on the current statute would be:

42 U.S. Code § 1320a–7h - Transparency reports and reporting of physician ownership or investment interests

(e) Definitions

(10) Payment or other transfer of value

(B) Exclusions - An applicable manufacturer shall not be required to submit information under subsection (a) with respect to the following:

...

(xiii) peer-reviewed journals, journal reprints, journal supplements, and medical textbooks.

(xiv) A transfer of anything of value to a covered recipient who is a physician if the thing of value is intended solely for purposes of providing accredited and/or certified continuing education.

Section 4381(b) [p. 345, lines 5-6]:

- Explanation: By applying to reports submitted after the date of enactment (rather than transfers of value made after the date of enactment), this edit avoids a situation in which manufacturers must submit a report that includes these items as transfers for only part of a year.
- Proposed technical edits: Modifying Section 4381, Subsection (b) of the Discussion Document legislative text as follows:

(b) EFFECTIVE DATE.—The amendments made by this section shall apply with respect to ~~transfers of value made~~ information submitted under Section 1128G(a) on or after the date of the enactment of this Act.

Title IV—Additional Recommendation for Issues NOT Included in the Discussion Document

Medicare Coverage of FDA-Approved Investigational Device Exemption (IDE) Trials

SECTION __. COVERAGE FOR FDA-APPROVED CLINICAL TRIALS.

(a) Section 1862(m) of the Social Security Act [42 U.S.C. § 13957(m)] is amended --

(1) in the caption, by striking “CATEGORY A” and inserting “MEDICAL”;

(2) in paragraph (1) by striking “category A” and inserting “category A and category B” and by inserting at the end “and in the case of category B clinical trials, the costs of the devices under investigation”; and

(3) by inserting at the end the following paragraphs:

“(3) CATEGORY B CLINICAL TRIAL.-- For purposes of paragraph (1), a “category B clinical trial” means a trial of a medical device if--

“(A) the trial is of nonexperimental/investigational (category B) medical device (as defined in regulations under section 405.201(b) of title 42, Code of Federal Regulations (as in effect as of January 1, 2015); and

“(B) the trial meets criteria established by the Secretary to ensure that the trial meets criteria established by the Secretary to ensure that the trial conforms to appropriate scientific and ethical standards.

“(4) AUTOMATIC QUALIFICATION OF TRIALS.-- A category A or category B trial shall be deemed to be qualified under this subsection if the trial is conducted under an investigational device exception granted by the Food and Drug Administration for the purposes of conducting a clinical study in accordance with 21 U.S.C. 360j(g).”

(b) EFFECTIVE DATE.-- The amendment made by subsection (a) shall be effective **[REVIEW:]** 90 days after enactment.



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February 25, 2015

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

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

Re: Discussion Draft of 21st Century Cures Legislation

Dear Chairman Upton and Representative DeGette:

The American Cancer Society Cancer Action Network (ACS CAN) is pleased to offer comments on the Energy and Commerce Committee's draft 21st Century Cures Legislation. We share the Committee's goal to accelerate the discovery, development, and delivery of promising new treatments, and believe that this requires a strong research enterprise, incentives for improving patient care and an efficient regulatory environment while maintaining safety and efficacy standards.

Our comments focus on provisions of particular interest to cancer patients. We welcome the opportunity to continue to work with the Committee on this important legislation.

RESEARCH ENTERPRISE

Targeted Funding Without Increased Resources (Sections 1202, 4007, 4008)

ACS CAN is concerned that the 21st Century Cures discussion draft does not include a general authorization for increased NIH funding or any mechanism for increasing NIH funding. While we are mindful of the Committee's desire to emphasize certain new areas of research, the absence of additional resources puts current funding devoted specifically to cancer research at risk at a time when new genomic based discoveries in precision medicine are making the fight against cancer more productive and lifesaving than ever before. Targeted authorizations of appropriations that codify new research priorities should be funded from a new, higher baseline so that ongoing successful research on cancer and other diseases continues.

Flexible Research Authority and Multi-Year Funding (Section 1201)

The Flexible Research Authority that would be authorized for the Cures Acceleration Network under section 1201, as well as the authorization that funds appropriated to the Cures Acceleration Network be available until expended, are positive enhancements to the program. However, we suggest that these

authorities be granted more broadly. There are effective drug development and clinical research programs at multiple NIH research institutes, including the National Cancer Institute (NCI). These other programs would also benefit from these enhanced authorities.

Additionally, NIH as a whole would benefit from the authority to carry over appropriations for more than one year, in a manner that is consistent with other grant-making scientific agencies. Having the authority to carry over funds for a second year would assist NIH in making the best decisions possible and would minimize the need to obligate resources “last minute” before they expire at the end of a fiscal year.

Innovative Cures Consortium (section 2001)

ACS CAN strongly supports innovation in biomedical research and the intent of the consortium. It is unclear, however, the extent to which it duplicates existing NIH and NCI programs that support public-private research partnerships. We would have concerns if the intent were to divert resources for similarly structured research projects that are now being undertaken in the institutes.

NIH Research Strategic Investment Plan and Evaluation (Section 4001, 4004)

ACS CAN is also concerned about certain aspects of the proposed “NIH Research Strategic Investment Plan.” The draft calls for a biomedical research strategic plan to be written in consultation with patient groups and industry that would identify strategic focus areas, and include measurable objectives for each focus area. While this is a reasonable goal, we want to make sure vital areas of research that have been instrumental in the fight against cancer continue to be supported. Behavioral, social science, and health services research in particular are critical to the prevention and early detection of disease. We know that most serious diseases have been controlled through prevention and public health strategies (smallpox, polio, tuberculosis), and we know that 50 percent of cancer deaths can be prevented if people engage in the right healthy behaviors. It is critical that these behavioral research programs, which already make up a very small portion of the NIH budget, yet help us understand how to motivate people to wellness, are not undermined in any way.

We also believe that the GAO study on duplication in federal biomedical research, authorized under section 4004(c), should have appropriate benchmarks for comparison. Studies on duplication of research should compare the NIH research portfolio with that of private industry. However, careful attention should be used in this examination. Multiple research projects on similar questions and topics are not always duplicative.

Precision Medicine (Sec. 2301)

While no text was provided in the draft legislation, we support the Committee's and the President's emphasis on precision medicine. Cancer research has been leading the field in development of precision (or personalized) medicine, and increased investment in this type of research has the potential to greatly benefit cancer patients.

INCENTIVES FOR INNOVATION

DORMANT Therapies (Sections 1221-1223)

ACS CAN strongly supports efforts to create economic incentives to encourage and promote innovation and clinical development of life-saving drugs that have medical promise. We believe the extended period of exclusivity envisioned in the DORMANT Therapies sections could encourage drug companies to pursue drugs that they might not have otherwise reviewed for further development, and this would be a positive step. We also know, however, that extended exclusivity can lead to higher drug prices for patients and payers. Accordingly, we recommend a balanced approach that motivates companies to look at dormant drugs, but doesn't go so far as to extend exclusivity to drugs that would have been developed anyway.

Sufficient Medicare Coverage for Colorectal Cancer Screening

As we asked in our earlier letter to the Committee, we urge you to incorporate the text of H. R. 1070, Removing Barriers to Colorectal Cancer Screening Act from the 113th Congress in the final 21st Century Cures legislation. This legislative language would simply eliminate an anomaly in the law that causes coinsurance to be charged in Medicare when a polyp is found during a colonoscopy. We believe this text to be germane given the other Medicare provisions (Sections 4221, 4261, 4281, 4284 and 4341) included in the larger draft released by the Committee. Currently, federal law waives the beneficiary coinsurance (copays and deductibles) for covered preventive services that have a grade "A" or "B" from the U.S. Preventive Services Task Force (USPSTF).

Colonoscopy, sigmoidoscopy, and fecal occult blood testing (FOBT) have all been assigned an "A" rating from the USPSTF for adults beginning at age 50 and continuing until age 75. However, patients who receive a screening colonoscopy that also involves the removal of precancerous polyps during the same clinical encounter as the screening test are responsible for the coinsurance, which can exceed \$500. This is because under Medicare coding rules, removal of any polyp reclassifies the "screening" as a therapeutic procedure. This glitch in the law creates a "bait and switch" scenario for beneficiaries who get a colonoscopy with the understanding that there is no coinsurance associated with the procedure, and then later are hit with an unexpected charge. Colorectal cancer screening by colonoscopy can remove precancerous polyps during the procedure, thereby making it a unique preventive service. Not only can cancer actually be prevented, small *in situ* cancers also can be removed during the procedure.

For those living on a fixed income, the chance of this unexpected cost can prevent them from receiving a potentially life-saving screening. Including the *Removing Barriers to Colorectal Cancer Screening Act* (H.R. 107; 113th Congress) would correct this oversight and allow men and women on Medicare to receive these important screenings without risking coinsurance. By removing this financial barrier, Congress would help increase screening rates and reduce the incidence of colorectal cancer. Accordingly, we ask that you consider including this legislation as part of the 21st Century Cures legislation.

REGULATORY ENVIRONMENT

The regulatory infrastructure for medical products is meant to evaluate evidence of safety and efficacy in order to protect the interest of public health. Over time, Congress has provided FDA with a variety of tools and alternate product approval pathways that allow provisional, faster, and more efficient approvals. The Office of Hematology and Oncology Products (OHOP) has been very aggressive in the use of all of the tools at its disposal to approve cancer drugs. In 2014 eight novel therapeutic cancer drugs were approved and half enjoyed fast-track designation, five were designated breakthrough status, all of the drugs were granted priority review, and seven received accelerated approval. All but one of these drugs was approved in the U.S. before being approved anywhere else in the world. While many of the proposals contained in the draft legislative proposal are meant to promote the use of advanced trial designs and flexible review processes, (e.g. surrogate endpoints, small single-arm trials and accelerated approvals), it is important to acknowledge that these tools have been successfully employed by OHOP, and should be looked to as a potential model for other product offices.

Patient Involvement at FDA (Sect. 1001)

Patient involvement throughout the drug and device approval process is imperative. Patients are uniquely positioned to provide valuable information about patient needs and preferences; they provide input on benefit and risk assessments; and, they have a unique perspective that can lead to improved quality of care for patients with a particular disease.

Section 1001 of the legislation would establish a new mechanism for FDA to receive patient experience data. The provision would require FDA to develop guidance, and provide a framework for patient advocacy organizations to gather patient data, quantify benefits and risk, and use patient reported outcomes that could be incorporated into the drug development and review process. ACS CAN supports the provision. However, we encourage the Committee to also include language in this section to build on provisions enacted in the Food and Drug Administration Safety and Innovation Act (FDASIA) specific to patient engagement in the drug approval process.

ACS CAN supported language in Sections 1137 and 1142 of FDASIA to maximize patient input on drug and device advisory committee and sponsor meetings, and allow a unique opportunity for the patient experience around benefit- risk assessment to be included during multiple points of the drug and device development process. Since the law was enacted, we have not been able to assess the overall impact of the FDASIA provision, and no statistics have been published with respect to patient participation in meetings either before or after FDASIA enactment. Given that it has been difficult to ascertain specific data on how FDA is responding to the law, we urge the Committee to include directive language, which is attached.

The proposed language would direct FDA to provide statistics indicating the number of meetings in which patient representatives have participated sorted by review division, disease, and type of meeting so that the Committee is able to monitor implementation of the program, its impact and workability. We also urge the Committee to include language directing GAO to undertake an analysis of existing FDA programs intended to facilitate patient representation and/or participation to provide a clearer understanding of the organizational structure and mandates of the various offices within FDA that have duties relating to patient representation and participation.

We commend the Committee for identifying patient engagement in the drug and device development process as an area of focus for the 21st Century Cures legislation by including the topic as one of the five priority areas of focus the Committee identified last year. We welcome the opportunity to work closely with the Committee to build on section 1001 of the draft bill.

Inclusion of Patients on Advisory Bodies (e.g. Section 2001, 2021, 4002)

The draft legislation includes proposals for a number of new commissions and bodies intended to provide advice and recommendations on various aspects of research, innovation, and drug development. Patients are the ultimate stakeholders in the quest for medical innovation, and therefore should constitute meaningful proportions of any new advisory groups. The Board of Directors of the Innovative Cures Consortium (Section 2001) does not reserve specific positions for patients, but rather reserves a set of board seats for a group of five stakeholder groups which includes patients. The Medical Product Innovation Advisory Commission (Section 2021) lists the types of individuals who must be included, but patients are not among those listed. The biomedical research working group (Section 4002) is an example of another group without specific requirements for patient representation.

As the Committee finalizes legislation we request that wherever new bodies are created greater representation is reserved for patients. Patients can provide beneficial insights and perspectives into the research, drug and device development process, and their views should be valued and acknowledged through appropriate representation on advisory groups and commissions.

**Sensible Oversight for Technology Which Advances Regulatory Efficiency (SOFTWARE Act)
(Sections 2061-2063)**

Software and mobile applications (apps) that are increasingly being integrated into healthcare hold enormous potential to increase the efficiency and quality of care. The Committee recognizes, and we agree that in order to achieve the twin goals of promoting innovation and protecting patient safety, we must create clear guidelines that distinguish applications that do not need to be regulated, from those that do, based on the potential risk that they pose to patients.

For example, software that monitors a person's activity as part of a weight management plan poses far less risk than software that monitor's insulin dosing to control blood sugar. The functional aspect of these software examples could be quite similar, but the patient risk is magnified in the insulin example. The Committee provision seems to recognize the need to differentiate based on patient risk, but does not quite make clear the actual delineation. As you know, ACS provided an expert witness in the committee hearing on this subject, and we would be pleased help refine this section, as appropriate, with the intent of assuring that robust innovation in healthcare software continues.

Expanded Access (Sections 1121-1125)

The primary goal of drug development should be the rapid evaluation of drug candidates so that safe and efficacious drugs are brought to market as quickly as possible. While full approval and broad access are the primary goals, in certain specific circumstances individual patients may benefit from an unapproved drug, yet be unable to access the drug via participation in a clinical trial for a variety of reasons, including ineligibility for trial participation. In such a case, an FDA expanded access protocol is in place for individual access to experimental drugs. This process requires obtaining physician and drug sponsor support of the proposed expanded access. For drug sponsors, providing such access can sometimes be challenging due to a number of concerns, including limited supplies of the drug and the potential for negative impact on the ongoing clinical trial.

FDA has recently proposed streamlining the process for single patient expanded access. The proposal contained in the 21st Century Cures draft would build upon the FDA proposal by providing drug sponsors new special approval pathway options in exchange for more transparency in their expanded access decision-making process. We support this proposal and look forward to further insight provided by the proposed GAO report and task force. We further recommend that both the GAO and the task force evaluate the effect of clinical trial inclusion/exclusion criteria on the need for expanded access and the impact of granting expanded access on the normal drug approval process.

Restricting Access to Pain Medication: (Sect. 4281 and 4284)

Prescription drug abuse and misuse continues to be a serious public health concern, and we share the goal of resolving this problem. However, it is important to approach changes in this policy area in a balanced way. Policies that address misuse and abuse must preserve patient access to effective medications for the millions of people who are debilitated by serious and/or chronic pain – many of whom are cancer patients. ACS CAN strongly opposes the policy changes proposed in Section 4281 of the draft. The language implies that every Medicare Part D beneficiary who uses a Schedule II, III, IV or V controlled substance would be identified for surveillance, and be required to participate in a safe pharmacy program. This policy change could create unintended consequences for patients who rely on these medications due to chronic and severe pain. It is a fact that localized shortages of opioid medications is an ongoing and increasing problem for cancer patients who have a legitimate need for pain control in order to work and to retain their independence. Limiting patient access, particularly for seniors who may be less able to travel to one particular pharmacy could cause cancer patients harm.

Additionally, the draft legislation would not allow coverage for a Part D schedule II, III, IV or V controlled substance unless the prescription is transmitted electronically in accordance with an electronic prescription drug program. This provision is concerning to us because the adoption of electronic prescription drug programs by pharmacies nationwide is extremely varied, and in most states only 60 to 80 percent of pharmacies are enabled. This provision would further restrict senior access to appropriate pain medication, and we would urge the Committee to adopt a phase-in period that would allow the nation's pharmacies time to successfully implement e-prescribing programs.

Modernizing Regulation of Diagnostics (Sec. 2161)

While no text was proposed for this section, we understand the Committee's interest in the issue of diagnostics regulation. ACS CAN provided extensive comments to the Committee in a letter dated January 5th, in which we expressed the importance of a regulatory framework that ensures patient safety regardless of where or how a test is developed and run.

Conclusion

Thank you again for the opportunity to contribute toward the 21st Century Cures initiative. Please do not hesitate to contact Dick Woodruff (dick.woodruff@cancer.org) or Keysha Brooks-Coley (Keysha.Brooks-Coley@cancer.org) if you have any questions. We look forward to continuing the discussion, and being of assistance in creating a final legislative product that meets the needs of cancer patients, survivors, and those who are helping them in the fight against the disease.

Sincerely,

A handwritten signature in black ink, appearing to read "Dick Woodruff", enclosed in a thin black rectangular border.

The American Cancer Society Cancer Action Network, the nonprofit, nonpartisan advocacy affiliate of the American Cancer Society, is the nation's leading cancer advocacy organization that works to make cancer issues a national priority.

Amendment to H.R. / S. _____

(21st Century Cures)

Insert at the appropriate place the following new section (and conform the table of contents accordingly):

SEC. __. IMPROVING PUBLIC ACCESS TO DATA ON PATIENT PARTICIPATION IN FDA PRODUCT SPONSOR MEETINGS AND FDA ADVISORY COMMITTEES.

(a) ANNUAL REPORTS ON PATIENT REPRESENTATION.—

(1) MEDICAL PRODUCT DISCUSSIONS.—Section 569C of the Federal Food, Drug and Cosmetic Act (21 U.S.C. 360bbb-8c) is amended by adding at the end the following new subsections:

“(f) ANNUAL REPORT.—

“(1) PATIENT PARTICIPATION.—Not later than February 1 of each year, the Secretary shall submit to the Committee on Appropriations and the Committee on Health, Education, Labor, and Pensions of the Senate, and the Committee on Appropriations and the Committee on Energy and Commerce of the House of Representatives, a report that provides information on participation of patient representatives in agency meetings during the fiscal year that ended on September 30 of the previous year, including the information described in paragraph (2), and recommendations described in paragraph (3). A report submitted under this subsection may be submitted in conjunction with the Secretary’s annual report on conflicts of interest required under section 712(e).

“(2) INFORMATION REQUIRED.—Each report under paragraph (1) for a fiscal year shall provide detailed information on the following:

“(A) PARTICIPATION IN AGENCY SPONSOR MEETINGS.—The participation of patient representatives in agency meetings with medical product sponsors and investigators. Such information—

“(i) shall include the number of such agency meetings in which patient representatives participated during such fiscal year; and

“(ii) shall be presented in multiple formats, including by type of medical product, by the division or office of the Food and Drug Administration conducting the review, by disease area, and by type of meeting (as such meeting types are specified in the Food and Drug Administration guidance document entitled “Formal Meetings Between the FDA and Sponsors or Applicants”).

“(B) AGENCY STAFFING FOR PATIENT REPRESENTATION.—Staffing levels within the Food and Drug Administration specifically dedicated to facilitating patient participation in agency meetings and patient engagement. Such information—

“(i) shall be presented by division and office of the Food and Drug Administration;

“(ii) shall include specific information on the number of staff and the amount of staff time dedicated to facilitating patient participation in agency meetings; and

“(iii) shall exclude staff or staff time dedicated to public relations, marketing or dissemination of information to patient representatives.

[Discussion Draft]

“(3) FINDINGS AND RECOMMENDATIONS.—The Secretary shall include in each report under paragraph (1)—

“(A) findings as to the adequacy of patient participation in such agency meetings during the fiscal year involved;

“(B) an explanation for the findings made under subparagraph (A); and

“(C) such recommendations to increase patient participation in such agency meetings as the Secretary determines to be appropriate.

“(4) PUBLIC AVAILABILITY.—Not later than 30 days after submitting a report under paragraph (1), the Secretary shall make such report available to the public.

“(g) PUBLICATION OF DATA ON AGENCY WEB SITES.—The Secretary shall publish and regularly update the information described in subsection (f)(2) on an appropriate Internet Web site or sites of the Food and Drug Administration, such as the ‘FDA Track’ Internet Web site. Such information shall be updated periodically but in no case less frequently than once each calendar quarter. The Secretary shall also post each annual report required under subsection (f)(1) on such Internet Web site or sites.”.

(2) CONFLICTS OF INTEREST.—

(A) ANALYSIS OF BARRIERS TO PATIENT PARTICIPATION.—The Commissioner of Food and Drugs shall perform a full analysis of the process and guidance for conflicts of interest under section 712 of the Federal Food, Drug and Cosmetic Act (21 U.S.C. 379d-1) to determine whether such process and guidance presents barriers to patient participation on advisory committees of the Food and Drug Administration. Such analysis shall also include an examination of the staff of the Food and Drug Administration and staff time available to carry out the requirements of such section and such other provisions of such Act, including section 569C of such Act, that are intended to encourage patient participation on advisory committees and at agency meetings with medical product sponsors and investigators.

(B) REPORT TO CONGRESS.—Not later than one year after the date of the enactment of this Act, the Commissioner shall submit a report to the Committee on Appropriations and the Committee on Health, Education, Labor, and Pensions of the Senate, and the Committee on Appropriations and the Committee on Energy and Commerce of the House of Representatives that describes the Commissioner’s findings of the analysis performed under subparagraph (A) and includes such recommendations for legislative or regulatory changes to eliminate barriers to patient participation on such advisory committees and at such meetings.

(b) GAO REPORT.—

(1) STUDY.—The Comptroller General of the United States shall conduct a study of all programs at the Food and Drug Administration designed to facilitate patient representation or patient participation on advisory committees that provide advice or recommendations to the Commissioner of Food and Drugs on activities of the Food and Drug Administration or in the medical product development process, including under sections 569C and 712 of the Federal Food, Drug and Cosmetic Act (21 U.S.C. 360bbb-8c, 379d-1).

(2) SPECIFIC MATTERS STUDIED.—The study conducted under paragraph (1) shall include an analysis of the following:

(A) The organizational structure and mandates of the various offices of the Food and Drug Administration with duties relating to patient representation or patient participation in activities of the Food and Drug Administration.

[Discussion Draft]

(B) The adequacy of the office staffing to carry out such duties.

(C) Whether the office structure or reporting requirements present conflicts with the mandates for patient-focused duties.

(D) A detailed examination of the coordination of the various patient representation or patient participation duties within different offices.

(E) The conflict of interest process and guidance under section 712 of the Federal Food, Drug and Cosmetic Act, and the effectiveness of the process and guidance in screening patient representatives in a timely manner to permit patients to participate in medical product sponsor meetings or on advisory committees effectively.

(F) Existing barriers that impede patient engagement in the Food and Drug Administration drug and device approval processes.

(3) FEEDBACK FROM PATIENT ADVOCACY ORGANIZATION.—In carrying out the study under paragraph (1), the Comptroller General shall seek input from patient advocacy organizations on the following:

(A) The views and experiences of patient representatives who have served or sought to serve on FDA advisory committees.

(B) Specific suggestions to expand the opportunities for greater engagement of patient representatives in medical product sponsor meetings, on advisory committees effectively, or in other appropriate areas.

(4) REPORT.—Not later than one year after the date of the enactment of this Act, the Comptroller General shall submit to the Committee on Appropriations and the Committee on Health, Education, Labor, and Pensions of the Senate, and the Committee on Appropriations and the Committee on Energy and Commerce of the House of Representatives a report that sets forth the findings of the Comptroller General with respect to the study conducted under this subsection, and includes recommendations for better coordination of patient engagement activities across the Food and Drug Administration.