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The 21st Century Cures Initiative: Summary of the House Energy & Commerce Discussion Document and the Senate HELP Committee Chairman's Innovation for Healthier Americans Report

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I. Introduction

On January 27, 2015, House Energy and Commerce Committee Chairman Fred Upton (R-MI) released a massive "discussion document" seeking feedback on draft legislative provisions that, based upon stakeholder feedback, will ultimately form the 21st Century Cures Initiative legislation that will be introduced in the coming months. The Initiative aims to "accelerate the discovery, development, and delivery of promising new treatments to patients."[1]

The 21st Century Cures Initiative has been a bipartisan collaboration between Chairman Upton, Diana DeGette (D-CO), and others. Over the past year, the Committee has convened eight hearings, requested comments, hosted over a dozen roundtables, and published numerous white papers to engage stakeholders on how to accelerate the pace of cures.

Chairman Upton's summary describes the discussion document as focusing on a wide range of issues, including provisions authored by both Republicans and Democrats that would: (1) better incorporate patient perspectives into the regulatory process and help address unmet medical needs; (2) streamline clinical trials; (3) accelerate the discovery, development, and delivery cycle and support continued innovation at Federal public health agencies; and (5) modernize medical product regulation.[2] The Committee also released a White Paper and section-by-section summary document.

In the Senate, Senator Lamar Alexander (R-TN), Chairman of the Senate Health, Education, Labor and Pensions (HELP) Committee, announced that he looks forward to working with Ranking Member Patty

[1] "Overview," *available at* http://energycommerce.house.gov/sites/republicans.energycommerce.house.gov/files/analysis/21stCenturyCures/20140501WhitePaper.pdf.

[2] "21st Century Cures Discussion Document Summary - January 27, 2015," *available at* http://energycommerce.house.gov/sites/republicans.energycommerce.house.gov/files/114/Analysis/Cures/20150127-Cures-Discussion-Document-Section-by-Section.pdf.

Murray (D-WA) and Chairman Upton to review the proposals. He explained that one of the committee's "top priorities this year will be modernizing the Food and Drug Administration, helping to get treatments and medicines to patients as quickly and safely as possible."[3] The HELP Committee has formed a bipartisan working group on these issues and will be holding a series of hearings in the coming months. On January 29, 2015, HELP Committee Chairman Alexander and Senator Burr (R-NC) also released a report outlining their ideas for legislation in this area and soliciting stakeholder feedback.

Although many in industry and some patient groups have hailed the proposals in the discussion document, not all initial reactions have been positive, and it is clear that the bipartisan nature of the initiative is in jeopardy, despite the inclusion of provisions authored by Democratic Members of the Committee. Representative Frank Pallone (D-NJ), Ranking Member of the House Energy and Commerce Committee, announced that he is "disappointed that the discussion document released today by Chairman Upton does not reflect true bipartisan collaboration . . . In its current form, I am concerned that the nearly 400 page draft could create more problems for our health care system than it solves." Representative Pallone also criticized the discussion document for not including "any real dollars to fund additional basic research."

The Generic Pharmaceutical Association (GPhA) also quickly objected to the draft, particularly provisions that would provide 15 years of exclusivity for a new class of products designated as dormant therapies, thereby delaying generic competition. AARP echoed similar concerns, noting that the legislation needs to achieve proper balance for ensuring treatments are safe and effective, but also affordable to consumers. However, in the White Paper released with the discussion document, Chairman Upton suggested that increased exclusivity periods is still an area open for discussion.

Given this opposition, it will be important for industry to provide comments as well as develop support from various parties -- including physician and patient groups -- to support this important Initiative. The Committee is accepting feedback at *cures@mail.house.gov*.

The following is a summary of the discussion document.

[3] *Available at* http://www.help.senate.gov/newsroom/press/release/?id=5e947998-4da4-47f8-a470-e7e1244e1fc5.

II. Title I--Putting Patients First By Incorporating Their Perspectives Into the Regulatory Process and Addressing Unmet Needs

Title I includes various proposals intended to empower patients, better incorporate patient experiences into the product approval process, and improve the regulatory and economic environments to encourage the development of therapies to address unmet medical needs, including new product classifications and approval pathways.

A. Subtitle A--Patient Focused Drug Development

The regulatory decision-making framework for drugs has been criticized for not adequately taking into consideration real world patient experiences with their disease or conditions. The Patient-Focused Drug Development Initiative, a commitment under the Prescription Drug User Fee Act (PDUFA V), aims to gather patients' perspectives on their diseases and conditions and available treatments through efforts including public meetings on specific disease areas. Building on the Patient-Focused Drug Development Initiative, Section 1001 would require the Food and Drug Administration (FDA) to establish a way to incorporate patient experience data (defined to include data collected by patients, parents, caregivers, patient advocacy organizations, disease research foundations, and medical researchers), into the risk-benefit framework within the new drug approval process. Section 1001 also would require the Secretary of the U.S. Department of Health and Humans Services (HHS)("Secretary") to convene workshops and a public meeting to obtain input for developing guidance, publish draft guidance within two years of the enactment of the Act, and report to Congress on the use of patient experience data not later than five years of enactment of the Act.

B. Subtitle B--Surrogate Endpoint Qualification and Utilization

Sections 1021-1024 are intended to enable manufacturers to sooner identify effective (or ineffective) compounds through the use of advancements in science and technology. These sections would require the Secretary to establish standards for the FDA's qualification and consideration of surrogate endpoints in its decision-making process. The Secretary would be required to issue draft guidance on the implementation of these sections within a year of the enactment of the Act (final guidance within 18 months). Additionally, Section 1021 would permit the Secretary to enter into public-private partnerships for the purpose of qualifying other biomarkers. Under Section 1024, the Secretary would be required to report to Congress on the number (and type) of surrogate endpoints requested for review, and the number qualified as such, within 18 months after final guidance is issued, and biannually thereafter.

C. Subtitle C--Approval of Breakthrough Therapies

Section 1041 would amend the Federal Food, Drug, and Cosmetic Act (FDCA) to make clear that FDA may approve a drug designated as a breakthrough therapy (under FDCA Section 506(a)) when "early clinical safety and effectiveness data" provides sufficient evidence for approval under the current safety and efficacy standards. Within one year, the Secretary would be required to publish draft guidance—developed in consultation with industry, academia, patient groups, disease research foundations, and other stakeholders—on policies and procedures for obtaining FDA approval of breakthrough therapies.

D. Subtitle D--Antibiotic Drug Development

Subtitle D builds on the Generating Antibiotics Incentives Now (GAIN) Act to attempt to stimulate greater industry investment in new infectious disease therapies in a manner similar to the Antibiotic Development to Advance Patient Treatment or "ADAPT" Act and Developing an Innovative Strategy for Antimicrobial Resistant Microorganisms or "DISARM" Act. Sections 1061-1063 would aim to foster the development of new antibiotics and antifungals by providing incentives including:

- A pathway for approval of products for use in limited populations;
- A new transferable extended *exclusivity* period program, similar to the transferable priority review voucher program for neglected tropical disease treatments and rare pediatric diseases. Recipients of transferred exclusivity vouchers would be required to make donations to the NIH and an independent patient assistance program.

Section 1062 would aim to ensure that susceptibility test interpretive criteria for antimicrobial drugs are updated and made publicly available. These sections also would require the Secretary to monitor the use of antibacterial and antifungals and changes in bacterial and fungal resistance to drugs and make its monitoring data publicly available.

Further, Section 1064 would aim to incentivize new drug development by improving Medicare payment for antibiotics for unmet medical needs. This section also would call for study of and reporting on the barriers that prevent the development of new antimicrobials, and identification of recommendations to overcome barriers, no later than one year after enactment.

E. Subtitle E--Priority Review for Breakthrough Devices

FDA's priority review program sets a goal for FDA to complete reviews of new drug applications within six months from the filing date (instead of ten) for drugs that could treat serious conditions and could provide

significant improvements in safety or effectiveness. Section 1081 would establish a similar process for the designation and expedited review of medical devices that represent breakthrough technologies and have the potential to address unmet medical needs. This section also would require FDA to issue guidance on this process.

Section 1082, which would require the Centers for Medicare and Medicaid (CMS) to provide coverage for breakthrough devices, is still under development.

F. Subtitle F--Accelerated Approval for Breakthrough Devices

FDA's accelerated approval program allows for earlier approval of drugs that treat serious diseases or conditions and generally provide a meaningful advantage over available therapies based on surrogate endpoints reasonably likely to predict clinical benefit. Section 1101 would establish an accelerated approval pathway for medical devices similar to the pathway that currently exists for drugs.

G. Subtitle G--Expanded Access

Sections 1121-1125 would impose certain transparency requirements on drug companies regarding their programs for patient access to certain investigational (unapproved) drugs. These sections would require regular reporting (by the Comptroller General) to Congress on the extent to which patients have access to investigational drugs, and recommendations for improving patient access. The draft would create an "Expanded Access Task Force" to make recommendations to Congress about further reforms to expand patient access. Within 180 days of the Task Force's establishment, the Secretary would be required to issue draft guidance on expanded access to investigational treatments.

H. Subtitle H--Facilitating Responsible Communication of Scientific and Medical Developments

Section 1141, which is expected to clarify and rationalize FDA's rules and policies for what drug and device manufacturers may say about their products, particularly with respect to off-label data, is still under development. This proposal is intended to address decades-old rules and policies governing what manufacturers may say so that scientific and medical developments can be shared with physicians, insurers, and researchers, with appropriate safeguards, to optimize patient care.

I. Subtitle I--Modernizing the Regulation of Social Media

Section 1161 would clarify communication rules about medical products on social media by requiring the Secretary to review and revise relevant regulations and guidance to "facilitate meaningful use . . . of the Internet, including Internet applications and social media for dissemination of truthful, non-misleading information about medical products."

J. Subtitle J--Streamlined Data Review

Section 1181 would facilitate labeling updates by requiring the Secretary to establish a process for the submission of "qualified data summaries" (rather than full data packages) in supplemental applications to add new indications to approved drug labels (initially for oncology indications). The FDA Commissioner would be required to issue guidance on the implementation of this section within 18 months of enactment and also to report to Congress twice on the implementation of this section and make recommendations for future use of data summaries in the application review process (first report due within 2 years of enactment).

K. Subtitle K--Cures Acceleration Network

The National Center for Advancing Translational Science (NCATS), was established at NIH in 2011 to "transform the translational science process so that new treatments and cures for disease can be delivered to patients faster."[4] Section 1201 would amend the Public Health Service Act (PHSA) to provide NCATS with more flexibility to enter into transactions to fund certain research projects. Section 1202 also would authorize additional funds for research on repurposing approved drugs for new uses.

L. Subtitle L--Dormant Therapies

Certain treatments and cures for complex diseases take longer to develop and thus may have insufficient patent protection. Sections 1221-1223, based on the Modernizing Our Drug & Diagnostics Evaluation and Regulatory Network (MODDERN) Cures Act (H.R. 3116), would aim to incentivize the development of these kinds of treatments through the creation of a new class of drugs or biologicals called "dormant therapies"[5] that address unmet medical needs (as defined by the FDA). These sections would provide a designated dormant therapy with a 15-year "protection period" upon FDA approval, during which the

[4] *See* "About NCATS," *available at* http://www.ncats.nih.gov/about/about.html.

[5] Defined to mean a medicine designated as a dormant therapy under proposed section 1222(a).

Secretary would not approve any applications referencing or otherwise relying on the approval of the dormant therapy. Applicants for the designation, however, would have to waive the right to enforce any patent that may expire after the protection period. The Secretary (in consultation with the Secretary of Commerce) would be tasked with promulgating regulations and guidance implementing the dormant therapy program within 18 months of enactment.

M. Subtitle M--New Therapeutic Entities

Section 1241 would amend the FDCA to extend the exclusivity period for an additional two years for new drug applications and abbreviated new drug applications that represent significant improvements to existing molecules (e.g., products expected to promote greater patient adherence, reduce public health risks, reduce side effects).

N. Subtitle N--Orphan Product Extensions Now

Section 1261 would amend the FDCA to provide an additional six months of market exclusivity for drugs designated by the Secretary as approved for an indication to prevent, diagnose, or treat a rare disease or condition.

III. Title II--Building the Foundation For 21st Century Medicine, Including Helping Young Scientists

A. Subtitle A--21st Century Cures Consortium Act

Section 2001, led by Representative Cathy McMorris Rodgers (R-WA), would establish a public-private partnership-- the "21st Century Cures Consortium"--to accelerate the discovery, development, and delivery of innovative cures, treatments, and preventive measures for patients in the United States. The Consortium would award grants and contracts and provide other assistance to eligible entities for activities to accelerate the discovery, development, and delivery of innovative cures, treatments, and preventive measures for patients. Participants or recipients of grants or awards would have to match Consortium support with proportional private funds.

The Consortium would be led by a board composed of government leaders from NIH, FDA, and CMS, and leaders from medical device and pharmaceutical companies, academic research institutions, patient groups, health plans, and others. Although this Consortium is broader in scope, it is based on the success of the European Union's Innovative Medicines Initiative, Europe's largest public-private initiative aimed at accelerating the development of medicines. The Consortium would sunset in September 2021.

B. Subtitle B--Medical Product Innovation Advisory Commission

Section 2021 would create the Medical Product Innovation Advisory Commission to analyze medical product innovation in the U.S. (including a review of NIH, FDA, and CMS policies) and recommend to Congress policies to accelerate the discovery, development, and delivery of new medical products. This Commission, which is based on the Medicare Payment Advisory Commission (MedPAC),[6] would advise Congress on issues related to the discovery-development-delivery cycle.

C. Subtitle C--Regenerative Medicine

Section 2041 would require FDA to update its guidance on surrogate and intermediate endpoints for the accelerated approval of regenerative medicine products under Section 506(c) of the FDCA. The Secretary would be required to consult with stakeholders, which may include public hearings within one year, and would be required to issue the guidance not later than two years after the legislation is enacted.

D. Subtitle D--Genetically Targeted Platform Technologies for Rare Diseases

Section 2051 would clarify the accelerated approval pathway to enable FDA to rely on data from products that utilize a similar genetically-targeted therapeutic platform technology. Specifically, Section 2051 would permit the Secretary to approve an application for a product for a serious or life-threatening disease or condition, including a fast track product under Section 505(c) of the FDCA or 351(a) of the PHSA upon a determination that, taking into account the severity, rarity, or prevalence of the condition and the unavailability or lack of alternative treatments:

I. The product has an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit; or
II. The extrapolation of evidence is reasonably likely to predict clinical benefit of the product.

Evidence to support a finding that an endpoint is reasonably likely to predict clinical benefit, or that a product is reasonably likely to have a clinical benefit could include: (i) epidemiological, pathophysiological,

[6] MedPAC is an independent congressional agency that advises Congress on issues affecting Medicare.

therapeutic, pharmacologic, or other evidence, such as evidence from the use of biomarkers; or (ii) evidence derived from extrapolation[7] from adequate and well-controlled trials that have formed the basis for investigation on other products: (I) that utilize the same or a very similar underlying "genetically-targeted therapeutic platform technology"[8] as the product involved; (II) for which disease genomics are known; and (III) that possess the same or very similar drug-like characteristics as the product involved, including with respect to safety, distribution, and metabolism; or (iii) other scientific methods or tools. The Secretary's determination shall be based on the "totality of the evidence."

E. Subtitle E--Sensible Oversight for Technology Which Advances Regulatory Efficiency

Sections 2061-2063 include language from the recently released discussion draft based on H.R. 3303, the Sensible Oversight for Technology which Advances Regulatory Efficiency (SOFTWARE) Act, which was introduced by Full Committee Vice Chair Marsha Blackburn (R-TN), Health Subcommittee Ranking Member Gene Green (D-TX), and Representatives Greg Walden (R-OR), Diana DeGette (D-CO), and G.K. Butterfield (D-NC). These sections would help provide regulatory certainty for those developing apps and health information technologies by creating statutory definitions for "software,"[9] "medical software,"[10] "health software,"[11] "accessory,"[12] and "component."[13] Under these proposed definitions, the

[7] Defined in Section 2051 to include "extending a sponsor's information and conclusions available from studies in one or more subgroups of the patient population, with respect to related conditions or related medicinal products, to make inferences for another subgroup of the population, condition, or medicinal product, thus reducing the need to generate additional information to reach conclusions for the target subgroup, condition, or medicinal product."

[8] Defined in Section 2051 to mean "a therapy based on a nucleic acid or an analogous compound with a common or highly-similar chemistry that: (I) may be applied across multiple products; and (II) can result in the modulation (including suppression, upregulation, or activation) of the function of a gene or its associated gene product, causing an altered disease state."

[9] Defined to mean "a coded or operational product that contains programs, procedures, and rules that act upon data to process, store, transmit, analyze, present, or operationalize information."

[10] The discussion document proposes to define "medical software" as software that: (A) is not a component; (B) is not intended to provide a diagnosis; and (C) is intended to analyze patient-specific information and other information to recommend to health care professionals a single treatment or course of action: (i) without the need for such professionals to perform additional interpretation of, or to independently confirm the means for, such recommendation; and (ii) for the purpose of informing or influencing health care decisions in the prevention, diagnosis, prognosis, treatment, cure, or disease management related to any disease or condition in humans.

[11] Section 2061 would define "health software" to mean "software that is not medical software, is not a component, is intended to be used for or in support of a health care purpose, and: (A) is intended for use for administrative or operational support or the processing and maintenance of financial records; (B) is intended for use for clinical, laboratory, or administrative workflow and related record-keeping, including electronic health records; (C) is intended for use for aggregation, conversion, storage, management, retrieval, or transmission of data from a device or other thing; (D) is intended for use as a platform for a secondary software: (i) to run or act as a mechanism for connectivity; or (ii) to store data; (E) is intended for use to organize and present medical information for consumer health and wellness education or for use for maintaining health or wellness; (F) is intended for use by patients for self-

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SOFTWARE Act would only regulate those products meeting the definition of "medical software" by requiring the Secretary to promulgate final regulations within 24 months after the date of enactment to classify medical software and establish standards for the development, validation, and verification of medical software. Health software would not be subject to regulation under these sections, and Section 2063 proposes to specifically exclude from the definition of "device" "health software."

Section 2062 would not require medical software products to be classified and cleared or approved under sections 513, 510(k) and 515. This section also would permit sponsors that previously initiated the process for classification and clearance or approval of medical software to proceed with the classification process rather than seeking classification and review under the future medical software regulations (noted above), and would not later be required by the Secretary to seek review.

Additionally, under Section 2062, the future medical software regulations would be required to establish procedures for reviewing medical software, including modifications, manufacturing, quality systems, labeling, and postmarketing. Violations of future regulations for medical software for manufacture, distribution, or sale would result in the products being treated as adulterated under Section 501 and misbranded under Section 502 for any improper labeling. The Secretary would be required to convene workshops with relevant stakeholders, including patients, FDA officials, software developers, and health care software product developers.

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management or self-monitoring of a disease or condition, including management of medications; (G) is intended for use to collect patient reported outcomes data for use by a health care practitioner; (H) is intended for use to analyze patient-specific information or other information for purposes of presenting patient-specific recommended treatments or courses of action to inform health care professionals' decisions with respect to the prevention, diagnosis, prognosis, treatment, cure, or management of a particular disease or condition, with the opportunity for additional interpretation or an independent confirmation of the means for such treatments or courses of action; or (I) is intended for use to analyze patient-specific information or other medical information for the purpose of providing general information related to the prevention, diagnosis, prognosis, treatment, cure, monitoring, or management of a disease or condition.

[12] Defined as a product that: "(A) is intended by its manufacturer to be used together with a particular device or software product to extend that device's or software product's intended use or functionality; (B) is not a component and could, based on the intended use of the product, be considered medical software, health software, or a device; and (C) is a product in its own right and should be classified based on its own intended use, functionality, and risk, and not the product in conjunction with which it is used."

[13] Defined as "a product that is an integral part of a device necessary to support the intended use of the device."

F. Subtitle F--Building a 21st Century Data Sharing Framework

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

1. Part 1--Improving Clinical Trial Data Opportunities for Patients

Section 2081 would require NIH to ensure that the Clinical Trial Registry Data Bank can be used easily by the public and that entries in the registry and results data bank are easily comparable. Further, this section would require NIH to ensure that information submitted to the registry and results data bank, including recruitment information, is submitted in a standardized format employing comprehensive health care terminology that includes clinical trial inclusion and exclusion criteria, including: (i) criteria for primary disease or condition being studies; and (ii) eligibility criteria that allow: (I) electronic matching to diagnoses or procedure coding systems such as the International Classification of Diseases or the Current Procedural Terminology (CPT); and (II) integration into electronic health records. The Secretary would also be required to convene a meeting of stakeholders (patients, researchers, physician, industry, HIT providers, FDA, etc.) to provide advice on enhancements to the clinical trial registry data bank.

Section 2082 would require the Secretary to enter into a collaborative agreement -- the "Clinical Trial Data System Agreement"-- with one or more eligible entities to implement a system to make de-identified clinical trial data from "qualified clinical trials"[14] available for purposes of conducting further research. This section also would establish an application process for such entities to be considered and approved.

[14] Defined to mean a "clinical trial sponsored solely by an agency" of HHS with respect to a medical product that: (A) was approved or cleared under section 505, 510(k) or 515 or has an exemption for investigational use in effect under section 505 or 520(m) of the FDCA; or is licensed under section 351 of the PHSA or has an exemption for investigational use under this section; or (B) that is an investigational product for which the original development was discontinued and with respect to which: no additional work to support approval, licensure, or clearance of such medical product is being or is planned to be undertaken by the sponsor of the original development program, its successors, assigns, or collaborators; and (ii) the sponsor of the original investigational development program has provided its consent to the Secretary for inclusion of data regarding such product in the system established under this section.

2. Part 2--Improving Clinical Outcomes for Patients and Program Integrity Through CMS Data

Section 2085 would expand uses of Medicare data (beginning July 1, 2015) by authorizing qualified entities[15] to use the combined data described in paragraph (4)(B)(iii) of Section 1874(e) of the Social Security Act (42 U.S.C. § 1395kk(e))[16] to conduct additional nonpublic analyses (as determined appropriate by the Secretary) and provide or sell such analyses to authorized users[17] for nonpublic use (including for the purposes of assisting providers of services[18] and suppliers[19] to develop and participate in quality and patient care improvement activities, including developing new models of care). Any analyses provided or sold under this section to an employer[20] may only be used by such employer for purposes of providing health insurance to employees and retirees of the employer. A qualified entity would not be allowed to provide or sell an analysis to a health insurance issuer described in paragraph (9)(A)(iv) unless the issuer is providing the qualified entity with data under Section 1874(e)(4)(B)(iii) of the Social Security Act (42 U.S.C. 1395kk(e)(4)(B)(iii)).[21]

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[15] This section defines "qualified entity" to have the meaning given such term in section 1874(e)(2) of the Social Security Act (42 U.S.C. § 1395kk(e)).

[16] This includes standardized extracts of claims data under parts A, B, and D for items and services furnished under such parts for one or more specified geographic areas and time periods requested by a qualified entity.

[17] This section defines this term to mean: (i) a provider of services; (ii) a supplier; (iii) an employer (as defined in section 3(5) of the Employee Retirement Insurance Security Act of 1974); (iv) a health insurance issuer (as defined in section 2791 of the PHSA); (v) a medical society or hospital association; and (vi) any entity not described that is approved by the Secretary.

[18] This section defines "provider of services" to have the meaning given such term in section 1861(u) of the Social Security Act (42 U.S.C. § 1395x(u)).

[19] This section defines "supplier" to have the meaning given such term in section 1861(d) of the Social Security Act (42 U.S.C. § 1395x(d)).

[20] As defined in paragraph (9)(A)(iii).

[21] Beginning July 1, 2015, a qualified entity would be allowed to provide or sell the combined data described in paragraph (4)(B)(iii) to users described in clauses (9)(A)(i), (ii), and (v) for nonpublic use, including for the purpose of assisting providers of services and suppliers in developing and participating in quality and patient care improvement activities, including developing new models of care. Qualified entities also would be able to provide the same users Medicare claims data for nonpublic uses, but would not be able to charge a fee for providing such data. Analyses that use such data would not be able to contain information that individually identifies a patient. An analysis that is or data that are provided or sold to a provider of services or supplier could contain information that individually identifies a patient of *such* provider or supplier, including with respect to items and services furnished to the patient by other providers of services or suppliers. Authorized users of such data would be prohibited from using any analysis or data provided for marketing purposes.

A qualified entity and an authorized user would be required to enter into an agreement regarding the use of any data that the qualified entity is providing or selling to the authorized user. Such agreement would be required to describe the requirements for privacy and security of the data and, as determined appropriate by the Secretary, any prohibitions on using such data to link to other individually identifiable sources of information. If the authorized user is not a covered entity under the Health Insurance Portability and Accountability Act (HIPAA), the agreement would be required to identify the relevant regulations, as determined by the Secretary, that the user must comply with as if it were acting in the capacity of such a covered entity.[22] Section 2085 also provides for the assessment of up to a $100 penalty per individual for breaches under the data use agreement. Qualified entities that provide or sell an analysis or data under this section would be required to submit a detailed report to the Secretary describing the uses and sales of such data and analyses.

Section 2085(b) would require the Secretary, at the request of a qualified clinical data registry under Section 1848(m)(3)(E) of the Social Security Act (42 U.S.C. § 1395w-4(m)(3)(E)), to provide the data described above (in a form and manner determined appropriate) to such qualified clinical data registry for the purposes of linking such data with clinical outcomes data and performing risk-adjusted, scientifically valid analyses and research to support quality improvement or patient safety, provided that any public reporting of such analyses or research that identifies a provider of services or supplier must only be conducted with the opportunity of such provider or supplier to appeal and correct errors. This data would include claims data under the Medicare program, and, if the Secretary determines appropriate, claims data under the Medicaid program and the State Children's Health Insurance Program (CHIP). Data could be provided to a qualified clinical data registry at a fee equal to the cost of providing such data.

Section 2085(c) would allow the Secretary, if deemed appropriate, to provide qualified entities, beginning July 1, 2015, standardized extracts (as determined by the Secretary) of claims data under Medicare and Medicaid for assistance provided under such titles for one or more specified geographic areas and time periods requested by a qualified entity.

Section 2086 would require the Secretary to promulgate an interim final regulation to permit certain entities to obtain the data described above in Section 2085. An entity that is permitted to obtain such

[22] An authorized user that is provided or sold an analysis or data would not be permitted to re-disclose or make public such analysis or data or any analysis using such data. However, a provider of services or supplier that is provided or sold an analysis or data would, as determined by the Secretary, be able to re-disclose such analysis or data for the purposes of performance improvement and care coordination activities but would not be able to make public such analysis or data or any analysis using such data. This section also provides an opportunity for providers of services and suppliers to review data before it is provided to appeal and correct errors.

information would include a State or a qualified researcher[23] who submits to the Secretary an application with certain information. [24] Section 2087 would require the Secretary to establish an exception that would allow clinical data registries to comply with HIPAA privacy and security law, (Section 3009 of the PHSA) in lieu of complying with the privacy and security provisions of the Common Rule (45 C.F.R. Part 46, subpart A). The Secretary would be required to propose regulations and guidance, as may be necessary, within 12 months after enactment, to implement this exception.

Section 2088 would give the Secretary the discretion to allow access in real time to Medicare claims data by third parties certified by the Secretary or the Commissioner, as applicable, for purposes of fraud prevention.

3. Part 3--Building a 21st Century Clinical Data Sharing System

Section 2091 would require the Secretary to establish within HHS the "Commission on Data Sharing for Research Development," headed by a director appointed by the Speaker of the House of Representatives. The Commission would consist of 15 appointed members, with members being appointed by the Secretary, the Speaker, and the Senate Majority Leader. The Commission would be required to develop: (A) methods to enable data obtained from individuals participating in a public health program, including Medicare, Medicaid, and CHIP, and an Exchange established under title I of the Patient Protection and Affordable Care Act (Public Law 111–148), to be shared with a qualified entity; (B) uniform standards for the sharing by such a qualified entity or other entity of data so obtained; and (C) other recommendations for the collection and dissemination of such data, as appropriate.

With respect to the collection and dissemination of clinical data in a clinical data registry, the Commission would be required to develop: (A) processes and procedures to ensure that only valid data are entered into a clinical data registry, including processes and procedures for the development of standardized data definitions for use by health care providers (specific to each specialty) to enable real-time data migration between electronic health records used by such providers and such a registry; (B) appropriate data

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[23] The term "qualified researcher" means an individual with the education and experience necessary to design and conduct research properly, as determined by the Secretary, regardless of the individual's commercial or institutional affiliation."

[24] The application would be required to contain: (A) a description of the purposes for which the entity intends to use the data; (B) a demonstration that the entity is qualified to perform the tasks necessary to achieve the purposes described by the entity; and (C) an attestation by the entity that the entity will adhere to all requirements promulgated by the Secretary with respect to the use of the data. This data would not contain individually identifiable health information; would be the minimum amount of data necessary for the entity to accomplish its described purposes; and would relate to files designed by CMS as research-identifiable files.

integrity and security standards to ensure that the validity of the data in a clinical data registry is maintained both during the active phase of the clinical data registry and after closure of any special activities carried out by the registry; (C) appropriate processes for adverse event adjudication with respect to the use of data from a clinical data registry; (D) best practices to support audit practices necessary to ensure the integrity of the data in a clinical data registry; and (E) rules governing the review and access to data in such a registry, including rules establishing: (i) the review and acceptance process for requests and analysis of such data, taking into consideration informed consent restrictions, if any, and the objective of the initial clinical data registry activity; (ii) controlled processes for the access and release of such data that take into account: (I) data privacy, data integrity and traceability concerns; and (II) the effect that such access and release has on the market approvals and patent exclusivity periods for drugs, biological products, and devices and patent exclusivity periods; (iii) guidelines for data transparency; (iv) a process for the sharing of such data that relates to a specific drug, biological product, or device, including how such data are shared with the sponsor of the drug, biological product, or device; and (v) a process for sharing such data with qualified scientific and medical researchers for purposes benefitting public health or patient care.

Lastly, the Commission would be required to develop, for purposes of clinical research and clinical development, a process to enable a qualified entity or another entity approved by the Secretary to: (A) search across databases maintaining such data for de-identified information satisfying characteristics specified by such entity; and (B) receive such de-identified information satisfying such characteristics, whether or not data relating to such characteristics were included or specified in such a database using standardized or uniform terminology.

Section 2092 would require the Secretary within one year of enactment to make recommendations for the development and use, when appropriate, of clinical data registries that are integrated with clinical practice guidelines and best practices or standards of care, including registries designed to minimize duplication and burden on those operating or reporting to such registries, for the improvement of patient care. The Secretary must make such recommendations available to the public by posting them on a public HHS website.[25]

[25] The recommendations, with respect to such registries, would be required to include: (1) recommendations for a set of standards that, if adopted by such registries, would allow for the bidirectional, interoperable exchange of information between the electronic health records of the reporting clinicians and such registries; (2) recommendations on how clinical registries, including outcomes-based registries, may be developed and then used to evaluate various care models and methods, including improved clinical care coordination, and the impact of such models and methods on the management of diseases as measured by appropriate care parameters based on clinical practice guidelines and best practices (such as A1C, blood pressure, and cholesterol levels in the case of diabetes); (3) recommendations on how such registries should be structured to facilitate the recording and reporting of postmarket

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The Secretary would be required to consult with national medical specialty societies and drug and device manufacturers in the development of such recommendations as they relate to the diseases that they (or their manufactured drugs or devices) manage and treat (such as with endocrinologists with respect to recommendations relating to diabetes and prediabetes conditions). Congress requested input on other ideas for supporting the use of data to support new cures and increase the quality of patient care.

G. Subtitle G--Utilizing Real-World Evidence

Section 2101, led by Representative Michael C. Burgess, M.D. (R-TX), would authorize FDA to utilize real world evidence and require FDA to issue guidance on collecting such evidence.[26] Section 2101 would define "real-world evidence" to mean "data about the usage, benefits, or risks of a drug derived from sources other than randomized clinical trials, including from observational studies and registries, used to establish safety or effectiveness under section 505(d)." Under this provision, the Secretary would be required to establish a program for sponsors to submit real-world evidence for purposes including: (1) to support the approval of the use of a drug for a new indication; and (2) to support or satisfy post-approval study requirements.

The real-world evidence guidance would be required to address: (A) the appropriate standards and methodologies for the collection and analysis of real-world evidence submitted for the purposes noted above; and (B) the circumstances under which sponsors of drugs and the Secretary may rely on real-world evidence for such purposes. The Secretary also would be required to submit a report to the House

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data for the purposes of monitoring safety and efficacy of FDA-approved devices and drugs, reporting relevant clinical data to satisfy attestation requirements for coverage of prescribed devices and drugs, and better defining appropriate clinical use in support of evidence development for the Medicare program (such as improving patient access to safe and effective glucose monitoring systems and future glucose monitoring technologies); (4) recommendations on how data from such registries may be used to inform physicians and other health care professionals regarding clinical practices for the prevention of diseases (such as diabetes and the precursor conditions of diabetes) and appropriate methods for the dissemination of clinical practice support tools and other educational resources that may be derived from registry data; and (5) recommendations for how registries can be used to promote preventive health benefits such as screenings and the Medicare annual wellness visits that may reduce the risk of chronic diseases (such as obesity, osteoporosis, cardiovascular disease, cancer, diabetes, and their complications)

[26] The guidance must be issued not later than 18 months after the date of enactment, and the Secretary must consult with industry, academy, patients, and others in developing such guidance.

Energy and Commerce Committee and the Senate HELP Committee, and report on the implementation of the real-world evidence program within four years of enactment.[27]

H. Subtitle H--Coverage With Evidence Development

Section 2121, led by Representative John Shimkus (R-IL), would address the long and sometimes costly process that new technology developers must go through to secure CMS coverage, while allowing Medicare beneficiaries to secure coverage from the program for products that are the subject of the clinical trial in which they participate. This section provides that an item or service is considered to be for coverage with evidence development (CED) if: (A) the item or service is furnished to individuals as part of a clinical study performed to determine whether the furnishing of such item or service improves the health outcomes of such individuals; and (B) the furnishing of the item or service to the individual is determined by the Secretary to be reasonable and necessary to the carrying out of such clinical study.

Section 2121 would establish that the determination of whether the furnishing to individuals of items or services improves the health outcomes of such individuals would be determined by assessing whether the furnishing of such items or services improves the: (A) diagnosis or treatment of illnesses or injuries of such individuals (as compared to the diagnosis or treatment of illnesses or injuries of comparable individuals who are not so furnished such items or services); or (B) functioning of malformed body members of such individuals (as compared to the functioning of malformed body members of comparable individuals who are not so furnished such items or services).

I. Subtitle I--Combination Products

Sections 2141-2142, led by Representative Gus Bilirakis (R-FL), would require FDA to set forth additional guidance on the review process for products that include both a drug and device. This section would establish that the FDA center with primary jurisdiction for the premarket review of a combination product would be the sole point of contact for the sponsor of the product, and that such center would coordinate communications to and from any consulting agency center involved in such premarket review. Agency communications and commitments from the center with primary jurisdiction would be binding on all other centers involved in the review. Within one year after enactment, the Secretary would be required to issue final guidance that describes the responsibilities of each agency center regarding its review of combination products, including each center's role in evaluating labeling, product usability assessments,

[27] The report must address: (1) how the program has been utilized by drug sponsors; (2) how the program has impacted regulatory decision-making, including 'substantial evidence' determinations under section 505(d); and (3) how the program could be expanded for the use of real-world evidence for additional purposes.

and human factors testing. The guidance would need to be updated at least biannually, after soliciting public comment, and specify in such updated guidance the reasons for updates. Section 2142 would require the Government Accountability Office (GAO) to submit a detailed report to Congress regarding FDA's regulation of combination products over an 11-year period (2003-2013), which addresses a number of specific issues.

J. Subtitle J--Modernizing Regulation of Diagnostics

Section 2161 includes placeholder language.

K. Subtitle K--Interoperability

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

L. Subtitle L--NIH Federal Data Sharing

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

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

Section 2221 would amend the privacy-related provisions of the Health Information Technology for Economic and Clinical Health (HITECH) Act to help realize the research potential of health data siloed in health care facilities across the country. Under the current privacy rules promulgated pursuant to the HITECH Act and the HIPAA, health care providers and health plans (covered entities) are strictly limited in the extent to which they may use and disclose personal (protected) health information (PHI) for research purposes. Section 2221 would require the Secretary to relax those rules, while at the same time providing for attendant new safeguards to protect the privacy rights of individuals.

Under the privacy rules as amended pursuant to Section 2221, disclosures of PHI for research purposes would not require a written authorization from the individual to whom the PHI pertains, provided that the disclosures were made: (A) to another covered entity for health care operations purposes; (B) to a business associate that has entered into a contract with the disclosing covered entity to perform health care operations; or (C) to a business associate for the purpose of data aggregation. Additionally, the amended privacy rules would permit a covered entity to receive payment in exchange for sharing of PHI for research purposes, subject to the current requirements for obtaining individual authorizations or

waivers of those requirements by an IRB or Privacy Board. Section 2221 also would allow covered entities to disclose PHI to a person subject to the jurisdiction of FDA for purposes of research, including comparative effectiveness research, with respect to an FDA-regulated product or activity for which that person has responsibility.

This provision also would relax the current privacy rules' restrictions on access to PHI by researchers for reviews preparatory to research, in order to permit remote electronic access from a portal or other access point outside of the covered entity so long as: (1) appropriate security and privacy safeguards are maintained by the covered entity; and (2) the protected health information is not copied or otherwise retained by the researcher. For uses and disclosures of PHI for research purposes where an individual authorization is obtained, Section 2221 would permit the authorization to apply to *all* future research purposes (rather than just specifically referenced research) and to cover uses and disclosures of PHI collected after the date of such authorization. To be valid, any such "one-time" authorization would have to: (1) sufficiently explain that the PHI will be used and disclosed for future research; (2) state that the authorization will remain valid unless and until it is withdrawn by the individual; and (3) afford the individual the option, and clearly describe how to exercise the option. to withdraw, the authorization at any time.

Finally, to enhance the privacy protections of individuals whose PHI has been either fully de-identified or stripped of all identifiers other than those permissible in a limited data set, Section 2221 would make it criminal offense for any person who has received or been granted access to the information either to: (A) knowingly identify or contact, or attempt to identify or contact, those individuals; or (B) knowingly permit or authorize a third party to knowingly identify or contact, or attempt to identify or contact, those individuals. This prohibition would not apply, however, to a HIPAA-regulated "business associate" whose authorized activities pursuant to a valid business associate agreement with a covered entity include identifying or contacting those individuals on the covered entity's behalf.

The new regulatory provisions required by Section 2221 would have to be promulgated by HHS within 12 months after enactment.

N. Subtitle N--21st Century Chronic Disease Initiative Act

Section 2241 would require the Secretary of HHS (in consultation with the Director of NIH) to develop a plan to carry out a longitudinal study designed to improve the outcomes of patients with chronic disease through better understanding of risk, transition from wellness to disease, disease progression, diagnosis, and other factors related to chronic disease, including by identifying potential targets for preventive or

therapeutic intervention. The plan would need to be designed and executed to support the goal of improving the outcomes of patients with a chronic disease and would need to: (1) address the roles of researchers, patients, experts, health care providers, etc., in developing and implementing the longitudinal study; (2) identify existing and ongoing studies that are relevant; (3) describe how patient cohorts will be utilized, coordinated, and expanded to ensure sufficient enrollment; and (4) describe how researchers and investigators will interact and coordinate with other chronic disease research efforts (e.g., National Alzheimer's Project Act).

O. Subtitle O--Helping Young Emerging Scientists

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

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

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

Q. Subtitle Q--Precision Medicine

Section 2301 includes placeholder language.

IV. Title III--Modernizing Clinical Trials

A. Subtitle A--Clinical Research Modernization

Sections 3001-3002, led by Representatives Cathy McMorris Rodgers (R-WA) and Diana DeGette (D-CO), would help streamline the institutional review board (IRB) process, particularly for clinical trials conducted at multiple sites, by minimizing regulatory duplication and unnecessary delays. This provision provides that human subject research that is subject to the FDCA or Section 351 of the PHSA would not be subject to the Basic HHS Policy for Protection of Human Research Subjects (45 C.F.R. Part 46, Subpart A). This section also would permit human subject research that is "cooperative research" (as defined in 45 C.F.R. § 46.114) to: (i) use joint or shared review; (ii) rely upon the review of: (I) an independent IRB; or (II) an IRB of an entity other than the sponsor of the research; or (iii) use similar arrangements to avoid duplication of effort. Within one year after enactment, the Secretary (in consultation with OHRP and FDA), would be required to issue regulations that, among other things,

delineate the roles of IRBs in multisite or cooperative, multisite studies where one or more local IRBs are relied upon or similar arrangements are used.

B. Subtitle B--Broader Application of Bayesian Statistics and Adaptive Trial Designs

Section 3021, led by Representative Chris Collins (R-NY), would require the Secretary to establish and implement a framework through which sponsors of drugs, biological products, or devices may submit to FDA a proposal for the incorporation of adaptive trial designs, Bayesian methods, or other alternative statistical methods into proposed clinical protocols and marketing applications for drugs, biological products, or devices. The Secretary, acting through FDA, also would be required to issue guidance addressing the use of adaptive trial design and the use of Bayesian methods in the development and regulatory review and approval, licensure, or clearance of drugs, biological products, and devices. The Secretary would be required to hold a Gpublic meeting regarding these proposals within one year of enactment and review and revise (as appropriate) the guidance not later than four years after enactment.

C. Subtitle C--Postapproval Studies and Clinical Trials

Section 3031, sponsored by Representative Chris Collins (R-NY), would ensure that FDA and sponsors periodically evaluate whether post-approval studies or clinical trials to determine whether: (I) the trial or study is no longer scientifically warranted; or (II) the design, or the timelines applicable to the completion of the study or trial should be renegotiated because of changes in medical practice or the standard of care. If a determination is made that a post-approval study or clinical trial is no longer scientifically warranted, the Secretary would no longer require the responsible person to conduct the study or trial. If a determination is made that the design or the timelines applicable to the completion of a post-approval study or clinical trial should be renegotiated, the Secretary would be required to enter into negotiations with the responsible person to make such changes as may be necessary to such design or timelines as the Secretary determines are necessary. The Secretary would be required to issue draft guidance to implement this section within one year and must finalize such guidance not later than two years after enactment, respectively.

D. Subtitle D--Pediatric Research Network Improvement

Section 3041, led by Representative Cathy McMorris Rodgers (R-WA), would require NIH to implement the National Pediatric Research Network Act, which was established as part of the Prematurity Research Expansion and Education for Mothers who deliver Infants Early (PREEMIE) Reauthorization Act (P.L. 113-55), legislation that reauthorized federal research, education and intervention activities related to preterm birth and infant mortality.

E. Subtitle E--Global Pediatric Clinical Trial

Section 3061, led by Health Subcommittee Chairman Joe Pitts (R-PA), would set forth a sense of Congress that NIH and FDA should work with European Union, industry, and others to establish a global pediatric clinical trial network. Under this section, NIH would be required to support the global pediatric clinical trial network through the allocation of grants to supplement the salaries of young researchers who participate in the global pediatric clinical trial network.

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

A. Subtitle A--National Institutes of Health

Section 4001, based on the work of Representative Andy Harris (R-MD), would require NIH to issue a 5-year biomedical research strategic investment plan, starting in fiscal year 2016, in consultation with directors of the national research institutes and centers, researchers, patient advocacy groups, and industry leaders. The plan would need to be designed to increase the efficient and effective focus of biomedical research in a manner that leverages the best scientific opportunities through a deliberative planning process; identify areas, to be known as strategic focus areas, in which the resources of NIH can best contribute to the goal of expanding knowledge on human health in the United States through biomedical research; and include measurable objectives for each such strategic focus area. Under the strategic plan, at least 55% of funds used to support extramural research for any fiscal year would need to be used to support basic biomedical extramural research. The NIH Director would be required to identify up to 10 strategic focus areas which best serve the goals of preventing or eliminating the burden of a disease or condition and scientifically merit an enhanced and focused research engagement campaign over the next 5 years. The plan would need to continue to ensure that rare and pediatric diseases and conditions remain a priority.

Section 4002 would establish the “Biomedical Research Working Group,” composed of NIH and relevant stakeholders, to provide recommendations on how to streamline the grant process for researchers by reducing administrative burdens. This would include making recommendations to the NIH director about restructuring, streamlining or simplifying the NIH grant proposal submission and progress report requirements and improving replicability of NIH-funded research.

Section 4003 – NIH travel--Section 4003 contains a placeholder.

Section 4004, based on the work of Chairman Emeritus Joe Barton (R-TX) and Representative Andy Harris (R-MD), would provide the NIH Director with more authority over the institutes and centers at NIH. Under this section, the President would appoint the Director of the National Cancer Institute and the other directors of the other national research institutes and centers would be appointed by the NIH Director. Terms for such directors, who would report directly to the NIH Director, would be for four years and could be removed or reappointed, with no term limits.

This section would also require that the director of a national research institute or center personally review and approve an award made by such institute or center for a grant for a research program or project (commonly referred to as an 'R-series grant'), other than an award constituting a renewal of such a grant. The director would be required to take into consideration: (i) whether the goals of the research program or project are a national priority and have public support; (ii) whether other agencies are funding programs or projects to accomplish the same goal; and (iii) whether the monetary investment is worth the potential scientific discovery.

Sections 4004-4005 would require GAO to conduct three studies not later than 270 days after enactment that address: (1) the extent to which biomedical research conducted or supported by federal agencies is duplicative; (2) the extent to which there is waste, fraud, and lack of consistency with the mission of NIH in the conduct and support of research by NIH; and (3) how amounts reserved under the NIH Common Fund have been used and the impact of that funding on each of the areas that received funding.[28] GAO would be required to submit the results of such studies or reports to Congress, including any recommendations on how to prevent duplicative research.

Section 4006, led by Representative Leonard Lance (R-NJ), would exempt certain NIH research activities from the Paperwork Reduction Act.

Section 4007 would authorize additional funding for the NIH Common Fund, and Section 4008, based on the work of Representative Tim Murphy (R-PA), would authorize funding for NIH's Brain Research through Advancing Innovative Technologies (BRAIN) initiative.

Section 4009 would remove the restriction on National Center for Advancing Translational Sciences' (NCATS) funding of phase IIB clinical trials, and instead, apply such restrictions to phase III clinical trials.

[28] Authored by Chairman Emeritus Joe Barton (R-TX).

B. Subtitle B--Advancing Research for Neurological Diseases

Section 4021, led by Representatives Michael C. Burgess, M.D. (R-TX) and Chris Van Hollen (D-MD), would require the Centers for Disease Control and Prevention (CDC) to set up a surveillance system to track the epidemiology of neurological diseases, including multiple sclerosis and Parkinson's disease, and incorporate information obtained through such activities into a statistically sound, scientifically credible, integrated surveillance system, to be known as the "National Neurological Diseases Surveillance System." The Secretary would need to ensure that this System is designed in a manner that facilitates further research on neurological diseases.[29] The System would need to collect and store information on neurological diseases such as: (A) demographics and other information associated or possibly associated with neurological diseases, such as age, race, ethnicity, sex, geographic location, and family history; (B) risk factors associated or possibly associated with neurological diseases, including genetic and environmental risk factors; and (C) diagnosis and progression markers.[30] The Secretary would be required to consult with relevant stakeholders in carrying out this section, including public health experts, relevant patient advocacy groups, health IT experts, and clinicians and researchers with relevant experience. The System would be made available to the public, including researchers, but would be required to ensure privacy and security protections are in place (e.g., HIPAA).

C. Subtitle C--Vaccine Access, Certainty, and Innovation (Also Addresses Priority Review Vouchers)

Sections 4041-4048, 4061-4063, led by Representative Renee Ellmers (R-NC), would provide certainty and transparency with respect to the regulation of vaccines, including with respect to CDC and CMS.

1. Part 1--Development, Licensure, and Recommendations

Sections 4041- 4048 would require the Director of the National Vaccine Program to establish standard timelines during which the Advisory Committee on Immunization Practices (ACIP) should consider and

[29] The Secretary would also be able to also award grants or enter into agreements to carry out these activities.

[30] The System could also include the collection and storage of information concerning: (A) the epidemiology of the diseases; (B) the natural history of the diseases; (C) the prevention of the diseases; (D) the detection, management, and treatment approaches for the diseases; and (E) the development of outcomes measures. The Secretary also would be required to make information in this System available to FDA, CMS, AHRQ, VA, DOD, and other appropriate agencies or federal departments. The Secretary also would be required to submit a report to Congress regarding the implementation of this section within four years after enactment.

make recommendations with respect to the route of administration, dosage, and frequency of administration of vaccines for specified populations. If the Advisory Committee does not make a recommendation within 120 calendar days after the licensure of the vaccine under section 351, the Advisory Committee, at the request of the sponsor of the vaccine, would be required to make such recommendations within 60 calendar days of the Advisory Committee's receipt of the request. If a vaccine is designated as a breakthrough therapy under section 506 of the FDCA, the Advisory Committee would be required to make the recommendations on an expedited basis.

Section 4042 would require the CDC Director to conduct a review of the transparency and consistency of the process used by the ACIP in formulating and issuing recommendations pertaining to vaccines.[31] This review would include an assessment of: (1) the criteria used to evaluate new and existing vaccines; (2) the Grading of Recommendations, Assessment, Development, and Evaluation (GRADE) approach to the review and analysis of scientific and economic data, including the scientific basis for such approach; and (3) the extent to which the processes used by the working groups of the ACIP are transparent and consistent.

Section 4043 would require the Secretary to issue final guidance, within two years after enactment, to facilitate the use of accelerated and expedited pathways for the development and licensure of vaccines to prevent: (1) emerging, re-emerging, or rare infectious diseases with respect to which the low prevalence or nature of the disease may render the existence or collection of clinical outcome data unlikely or impractical; and (2) infectious diseases with respect to which currently available vaccines are not addressing the full scope of public health needs.[32]

Section 4044 would require that CDC meet with vaccine developers, upon the submission of a written request, for the purpose of informing the vaccine developer's understanding of public health needs and priorities, and to provide the perspectives of the CDC and other relevant Federal agencies regarding: (i)

[31] The CDC Director would also be required to solicit input from vaccine stakeholders in carrying out this review. The CDC Director also would be required to submit a report to Congress regarding this section, including any recommendations on improving the transparency and consistency of the ACIP process, and make such report publicly available.

[32] In developing the guidance, the Secretary would be required to consider issues relating to clinical development strategies for diseases described in this section, including the development and acceptability of novel clinical and surrogate endpoints, the use of novel or accelerated study designs, the use of observational real-world data, the use of novel adjuvants, the use of new technologies or approaches to collecting and monitoring patient-level data, and the demonstration of efficacy through studies in healthy volunteers for the purpose of licensure.

public health needs, epidemiology, and implementation considerations with regard to a vaccine developer's potential vaccine profile; and (ii) potential implications of such perspectives for the vaccine developer's vaccine research and development planning.[33] Such meetings would be required to take place no later than 90 calendar days after receipt of the request.

Upon the submission of a written request by a vaccine developer, the Secretary, acting through the CDC Director, would be required to provide to the vaccine developer, within 90 calendar days after the receipt of such request, any age-based disease epidemiological analyses or data that: (i) are specified in the request; (ii) have been published; (iii) have been performed by or are in the possession of CDC; and (iv) are not a trade secret or otherwise confidential information. The Secretary would be required to promptly notify a vaccine developer if the Secretary becomes aware of any change to information that was shared by the Secretary to the vaccine developer during their meeting or provided by the Secretary to the vaccine developer in one or more analyses described above; and whether the change may have implications for the vaccine developer's vaccine research and development.

Section 4045 would modify the priority review voucher program for tropical diseases (21 U.S.C. § 360n) by requiring the Secretary to establish a process to designate infectious diseases other than those currently specified to be tropical diseases, using a methodology that is made available to the public on FDA's website. The Secretary would be required to publish on FDA's website a complete, updated list of the diseases that are tropical diseases.

In designating an infectious disease as a tropical disease, Section 4045 would require the Secretary to: (i) consider the potential impact of the disease on the public health due to: (I) the potential rate of spread of the disease; and (II) the potential severity of the disease in terms of human morbidity and mortality; and consult with experts in tropical infectious diseases, including the CDC, FDA, medical professionals, the clinical research community, and the World Health Organization. Every five years, or more frequently as determined necessary by the Secretary, the Secretary would be required to review, provide modifications to, and republish the published list noted above and any revisions made to the methodology for designation of diseases.

Section 4045 also would require each person to whom a priority review voucher is transferred to notify the Secretary of such change in ownership of the voucher not later than 30 calendar days after such transfer.

[33] The meeting must include experts in immunization programs, epidemiology, and other relevant areas, including such experts from FDA and the National Vaccine Program.

A sponsor of a human drug application that provides notification of intent to transfer would be permitted to transfer the voucher after such notification is provided, if such sponsor has not yet submitted the human drug application described in the notification. Upon such a transfer, the sponsor would not remain legally committed to pay a user fee because of the sponsor's notification of intent.

Section 4046 would require the Secretary to issue within two years after enactment a guidance that addresses changes to approved biological products. The guidance would be required to address changes in a licensed biological product or the labeling, production process, quality controls, equipment, facilities, or responsible personnel for such a product established in the application for the product that was approved under Section 351.[34] The guidance would not be able to address changes for specified biotechnology or specified synthetic biological products listed in 21 C.F.R. § 601.2(c).

Section 4047 would amend Section 801(e)(4) of the FDCA to require the Secretary to issue an export certificate for vaccines within 10 business days of the receipt of a request for export certification.

Section 4048 would require the NIH Director to conduct or support translational science, research, and research training to advance the development of vaccines for the prevention of diseases, including the advancement of vaccine development programs into clinical trials. Within one year of enactment, the NIH Director would be required to conduct a review of vaccine research being conducted or supported by NIH and publish a report on the results of such review.[35]

2. Part 2--Medicare, Medicaid, and Other Provisions

Section 4061 would require the Secretary to determine whether to update policies with respect to Medicare coverage of vaccines or immunizations not later than 60 calendar days after the date on which the ACIP issues a recommendation or update a recommendation for a vaccine that the Secretary is using under title XVIII of the Social Security Act (42 U.S.C. § 1395 et seq.).

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[34] This guidance would be required to update and supersede the guidance entitled "Changes to an Approved Application: Biological Products," that was issued by FDA in July 1997.

[35] The report must: (A) describe intramural and extramural vaccine research and development programs that are being conducted or supported by NIH, including those that are translational or clinical phase studies; (B) provide a summary of funding allocations made to conduct or support the matters described in section 447D of the Public Health Service Act, as added by subsection (a), and identify projected funding needs with regard to future research or support with regard to these matters; and (C) identify funding and collaborations with the private sector through: (i) the Small Business Innovation Research and Small Business Technology Transfer programs; and (ii) cooperative research and development agreements.

Section 4062 provides that for plan years beginning on or after the date of enactment of the Vaccine Access, Certainty, and Innovation Act of 2015, activities that improve health care quality would be required to include programs to increase adult immunization. Not later than December 31, 2016, the Secretary would be required to establish standardized methodologies, including definitions, for which activities, and in what regard such activities, constitute programs to increase adult immunization. The Secretary would be required to consult relevant stakeholders in establishing such methodologies. This section also would require that for purposes of calculating the minimum medical loss ratio, for plan years beginning at least 12 months after the date of enactment, the numerator must include any expenditures on programs to increase adult immunization.

D. Subtitle D--Reagan-Udall Improvements Bill

Section 4081, led by Full Committee Vice Chair Marsha Blackburn (R-TN), would improve the Reagan-Udall Foundation (a non-profit created by Congress to advance the mission of the FDA) by providing additional members, including certain federal employees, staggered terms for members, and increasing the executive director's compensation.

E. Subtitle E--FDA Hiring, Travel, and Training

Section 4101 contains placeholder language.

F. Subtitle F--FDA Succession Planning

Sections 4121-4122, led by Health Subcommittee Vice Chair Brett Guthrie (R-KY), would ensure that FDA staff has the ability to continue to improve their expertise and that FDA develops a succession plan for management positions. Section 4121 would require the Secretary to enhance the professional development of technical and scientific staff at FDA by requiring staff to attend technical and scientific conferences, meetings, and working groups that provide training and education on emerging technology and science relevant to the development and regulation of products under the jurisdiction of FDA. Section 4122 would require the Secretary to develop and implement a formal succession plan for management positions within FDA at or higher than the level of a director of a center.

G. Subtitle G--Disposable Medical Technologies

Section 4141, led by Representative Renee Ellmers (R-NC), would reform the coverage requirements under the Medicare program for certain non-durable disposable medical technologies that CMS classifies

as substitutes for durable medical equipment (DME). These products would be exempt from the DME competitive bidding program. CMS would be required to set a single payment amount for: (1) "substitute disposable medical technologies"[36]; and (2) "any services and supplies used in conjunction with such technology." Payment would be a lump sum payment for 95% of the cost of the DME for which the disposable product substitutes, plus the cost of associated services and supplies. The section, however, does not specify whether the "supplies" it covers include drugs that are used in conjunction with a "substitute disposable medical technology."

H. Subtitle H--Local and National Coverage Decision Reforms

Section 4161, led by Health Subcommittee Vice Chair Brett Guthrie (R-KY), would reform the Medicare local coverage determination (LCD) process. It also includes a request for additional ideas. This section would require a 45-day public comment period for proposed new or significantly revised LCDs (60 days if the proposal would limit or preclude coverage of an item or service). The Medicare Administrative Contractor (MAC) also would be required to meet upon request with providers, beneficiary representatives, or manufacturers or sponsors affected by the proposal; hold a meeting of its Carrier Advisory Committee if the proposed LCD would limit or preclude coverage of an item/service; and respond to comments received and describe the evidence considered in developing the LCD when the final LCD is released. These requirements would apply even if the MAC is proposing to adopt an LCD from another jurisdiction.

Section 4161 also would allow a MAC to adopt for its jurisdiction an LCD proposed or adopted for another jurisdiction only if it undertakes the process as described above in its jurisdiction with respect to such determination, including an opportunity for comment and meetings in its jurisdiction on such determination. MACs would be permitted to issue a revised LCD without the meeting or comment requirements above if the determination is: (I) a clarification that does not restrict coverage; (II) a change for a compelling clinical, safety, or technical reason, such as prevention of harm to individuals (subject to the approval of the Secretary); (III) a change for coding, coverage, or payment updates over which the MAC does not have discretion; (IV) a discretionary coding update that does not restrict coverage; (V) a change to effectuate a decision of an administrative law judge on a challenge under Section 1869(f) of the Social Security Act; or (VI) another type of change that the Secretary may specify in regulations.

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[36] This term is defined as "medical equipment that: (1) is primarily and customarily used to serve a medical purpose; (2) would otherwise be covered as durable medical equipment under this title but for the fact that such equipment is not durable (as defined by the Secretary for purposes of coverage of durable medical equipment under this title); and (3) the Secretary determines substitutes for durable medical equipment."

I. Subtitle I--Telemedicine

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

Within four years after enactment, CMS would be required to develop and implement a methodology to cover and pay for certain telehealth services (or episodes of services) "to the same extent and in the same manner" as if the supplier and the Medicare beneficiary receiving the services were at the same location. Subject to certain conditions, CMS would be allowed to waive otherwise-applicable Medicare telehealth requirements on what qualifies as a telehealth "originating site," any geographic limitation (e.g., that the beneficiary must be in a rural or underserved area), or any limitation on the "type of health care provider who may furnish such services." The payment methodology that CMS develops would not be able to increase projected Medicare expenditures.

CMS would select telehealth services and episodes to be covered by the new payment methodology through rulemaking. CMS's Chief Actuary would be required to certify that the payment methodology for the services and episodes would reduce or not increase net program spending. CMS also would be required to consider for inclusion in the new payment methodology telehealth services/episodes that meet "unmet needs" (as defined by CMS); are substitutes for an in-person visit; are proven to reduce readmissions; or enable a patient to move to a lower level of care. CMS could include telehealth services in any Medicare demonstration program, without applying otherwise-applicable telehealth requirements on what qualifies as an originating site, geographic limits, or limitations on the type of provider who may furnish services.

For purposes of demonstrations, the "telehealth"[37] definition would be expanded to include "store and forward" technology (currently telehealth services generally must involve real-time audio and video communication). "Store and forward" would mean technologies allowing for "electronic transmission of medical information, such as digital images, documents, and prerecorded videos through secure email transmission"; input is requested on this definition. Finally, Section 4181 provides that it is the sense of Congress that states should collaborate to create common licensure requirements for providing telehealth

[37] Telehealth services has the meaning given under section 1834(m)(4)(F) of the Social Security Act.

services in order to facilitate multistate practices and allow for health care providers to provide such services across state lines.

J. Subtitle J--Revise IPPS New Technology Add-On Payment (NTAP)

Section 4201 would provide for greater transparency regarding the new technology add-on payment (NTAP) reimbursement process. This section would permit administrative appeals of CMS denials of New Technology Add-On Payment (NTAP) applications, which would have to be decided within 90 days. Within two years of the date of enactment, CMS would be required to "eliminate the use of HCPCS Level II codes for drugs and biologicals for purposes of coverage, coding and reimbursement under [Part B]."

HCPCS codes would be replaced by a group of drug products within NDCs "qualified as therapeutically equivalent by the [FDA] in the [Orange Book]." Section 4201 would make very detailed changes to Social Security Act § 1847A (on ASP-based payments for Part B drugs). These changes would move away from Part B drug payments based on broadly-described HCPCS codes, and instead, would only allow payments to be calculated based on the volume-weighted ASP of drugs rated as therapeutically equivalent by FDA in its Orange Book.

Section 4201 provides that it is the sense of Congress that "novel emerging therapies will offer a major step forward in the treatment and curing of diseases, as many of these new and emerging therapies, such as regenerative medicines, novel gene therapies, and new stem cell therapies will be used . . . . [CMS is] urged to begin the development of appropriate billing and payment coding regimes to anticipate the use of . . . new technologies . . . ."

K. Subtitle K--Lowering Medicare Patients Out-of-Pocket (OOP) Costs

Section 4221, led by Representative Gus Bilirakis (R-FL), would allow seniors to better identify the out of pocket costs they might face for a given treatment or service and pick the service that is right for them and their budget. Under this provision, the National Institute for Standards and Technology (NIST) and HHS would be required to establish a searchable database that provides Medicare beneficiaries with information for specified zip codes (and Medicare Advantage plans, if applicable), for certain services that may be furnished at different types of sites of service, on (among other things) the maximum out-of-pocket cost to an individual if the service were furnished at such a site and the total payment rate.

L. Subtitle L--Global Surgery Services Rule

Section 4241, led by Representative Larry Bucshon, M.D. (R-IN), would prevent the implementation of the global surgery services rule, which does away with bundled payments for surgeons. This section would prohibit CMS from implementing the provisions in its final MPFS rule for 2015 on transitioning 10-day and 90-day global surgery services to 0-day global periods. CMS's final rule provided for "unbundling" follow-up services from the surgery itself and thus waiving to "0-day" payments for the surgery and any services provided on the day of the surgery. CMS adopted this approach because CMS found that to set correct payments it needed better information on what services were actually being performed during the 10-day or 90-day post-surgical period. An OIG report found that the global payment rates were too high given the number of services actually performed during the period following surgery. The new CMS approach, however, has been criticized by some groups representing surgeons as being administratively unworkable due to requiring many small bills to patients for services following their surgeries.

M. Subtitle M--Providers Consolidation and Medicare Payments Examined Through Evaluation

Section 4261, led by Representative Michael C. Burgess, M.D. (R-TX), would require CMS to analyze and seek public input on how proposed Medicare payment policies would affect the consolidation of providers and payers. Beginning in 2016, as part of its annual rulemaking processes on Medicare payment system changes, CMS would be required to seek public comment on, and evaluate how payment system changes will likely affect, provider consolidation. "Consolidation" includes vertical integration among providers and suppliers, "including professional practices, health care services and ancillary services by any entity (such as a health system, group practice, or health insurer)."

N. Subtitle N--Medicare Part D Patient Safety and Drug Abuse Prevention

Sections 4281-4284, led by Representatives Gus Bilirakis (R-FL), Ed Whitfield (R-KY), Billy Long (R-MO) and Ben Ray Lujan (D-NM), seeks to prevent high-risk Medicare Part D beneficiaries from abusing controlled substances. Part D plans would be required to adopt procedures designed "to identify an individual who has obtained coverage for a covered Part D drug that is a frequently abused schedule II, III, IV, or V controlled substance, as determined in accordance with utilization guidelines developed by [CMS and the plan sponsor] and to notify such individuals that they have been so identified." The identified beneficiary would then be limited to filling prescriptions for schedule II-V controlled substances for some or all Part D drug classes at a specified pharmacy or pharmacies with a "safe pharmacy

network" meeting certain criteria. "Identified" beneficiaries could appeal or petition for termination of this status. When an "identified" beneficiary disenrolled from a Part D plan and enrolled in a new plan, CMS would be required to share information with the beneficiary's new plan about the pharmacy lock-in requirements that apply to the beneficiary.

Section 4282 would permit Part D plans to suspend payments to a pharmacy pending an investigation of a "credible allegation of fraud," which is defined to include: (I) a complaint made on the Medicare fraud hotline; (II) detection of potential fraud through the analysis of claims data; (III) detection of potential fraud through identification of inappropriate dispensing through audits, civil false claims cases, and law enforcement investigations; and (IV) claims referred to Medicare drug integrity contractors (MEDICs). Section 4283 would allow MEDICS to obtain prescription and medical records directly from entities such as pharmacies, PDPs, and physicians. CMS would be required to develop uniform criteria to assess MEDICS' performance.

Section 4284 would generally require Part D plans to cover a Schedule II-V controlled substance only if the prescription was transmitted electronically in accordance with Part D e-prescribing program standards.

O. Subtitle O--Accelerating Innovation in Medicine

Section 4301, led by Representative Erik Paulsen (R-MN), would establish a program that allows for patients to access medical device treatments sooner than otherwise would be available. This section would require CMS to maintain an "Accelerating Innovation in Medicine (AIM) List of Medical Devices Voluntarily Excluded from Coverage." No Medicare payment (direct or capitated) could be made for the listed devices and the patient receiving the device would be responsible for payment (and would have to be advised of this). Device manufacturers could only request AIM listing within the first 30 days of the device's launch. A device could stay on the AIM list for 3 years. To keep the device on the list for subsequent 3-year periods, the manufacturer would be required to provide CMS with published or publicly available data or clinical studies completed during the last three-year period. This provision is analogous to the Medicare "private contracting" provision for physicians, providing a way to opt out of the Medicare payment system. This section would not require the Secretary to promulgate regulations.

If a physician or other entity furnishes a medical device included on the AIM list to an individual and fails to obtain, before furnishing the device, an appropriate informed consent under which the individual is informed of and accepts liability for payment for the device (and related services), the physician or other

entity will be deemed to have agreed not to impose any charges under this title for such device (and for services related to furnishing the device).

P. Subtitle P--Medicare Pharmaceutical and Technology Ombudsman

Section 4321, led by Representative Susan Brooks (R-IN), would establish an ombudsman at CMS, within 12 months after enactment, to allow medical device and pharmaceutical companies to file complaints, appeal decisions, and better understand the reasoning behind Medicare coverage, coding, or payment decisions for their products.

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

Section 4341 would ensure that local MACs review all data before making coverage decisions on Category III codes.

R. Subtitle R--Advancing Care for Exception Kids

Sections 4361-436, led by Chairman Emeritus Joe Barton (R-TX) and Representative Kathy Castor (D-FL), would establish a Medicaid and CHIP Care Coordination program for children with medically complex conditions.

S. Subtitle S--Continuing Medical Education Sunshine Exemption

Section 4381, based on H.R. 293, which was introduced by Representatives Michael C. Burgess, M.D. (R-TX) and Peter DeFazio (D-OR), would clarify that peer-reviewed journals, journal reprints, journal supplements, and medical textbooks, as well as payments or transfers of value intended solely for purposes providing continuing medical education (CME), are excluded from the reporting requirement under the Physician Payments Sunshine Act.

T. Subtitle T--Medical Testing Availability

Section 4401, based on H.R. 298, which was introduced by Representatives Michael C. Burgess, M.D. (R-TX) and Jackie Speier (D-CA), would clarify the law regarding Research Use Only (RUO) labeled tests. This section provides that a product whose labeling bears “Research Use Only” (as required in 21 C.F.R. § 809.10(c)(2)(i)), would not be deemed adulterated or misbranded under the FDCA on the basis that the manufacturer or distributor of the product: (A) sells the product to an end user who uses the

product in a manner inconsistent with such statement; or (B) engages in business communications[38] regarding the product with an end user of the product.

VI. Title V--Modernizing Medical Product Regulation

A. Subtitle A--Manufacturing Incentives

Section 5001, led by Health Subcommittee Vice Chair Brett Guthrie (R-KY), aims to incentivize the manufacturer of generic drugs in the U.S. by requiring the Secretary to designate drugs (or biologicals) as American-manufactured drugs for purposes of granting exclusivity extensions if certain requirements are met.[39] The Committee specifically requests comment on what additional or different requirements should a manufacturer have to meet in order to receive the designation as an American-manufactured drug.

Drugs designated as American-manufactured would receive an extension on the 180-day period of exclusivity under 505(j)(5)(B)(iv). However, Congress did not propose a time frame for such extended exclusivity for drugs or biologicals. Only drugs approved on or after the enactment of the legislation would be eligible for designation.[40]

B. Subtitle B--21st Century Manufacturing

Section 5021, led by Health Subcommittee Vice Chair Brett Guthrie (R-KY), would require FDA to issue final regulations and guidance to update relevant cGMP regulations and guidance (FDA's main regulatory standard for ensuring product quality) to reflect novel manufacturing techniques.

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[38] Business communications are defined as: (A) "oral, written, or electronic contact between a manufacturer or distributor of such product and an end user regarding the functioning of such product; and (B) includes any such contact consisting of technical support, customer service, assistance with the installation of such product, communication relating to ensuring the performance of the product, and other similar contacts." This provision would sunset after five years.

[39] Requirements include: (1) an application is submitted for approval or licensure of such drug under section 505(j) or section 351(k); (2) the manufacturer or the sponsor of the drug includes in such application a request for designation of the drug as an American-manufactured drug; and (3) the request demonstrates to the Secretary's satisfaction that all quantities of the drug intended to be marketed in the United States will be manufactured, prepared, propagated, compounded, and processed, as applicable, in the United States.

[40] This section would not apply to a supplement to an application under 505(j) or 351(k) for which an extension is in effect or has expired; or a subsequent application filed with respect to a drug approved under section 505(j) or biological product under 351(k) for a change that results in a new route of administration, dosing schedule, dosage form, delivery system, delivery device, or strength.

C. Subtitle C--Controlled Substance Manufacturing and Exports

Section 5041, led by Health Subcommittee Chairman Joe Pitts (R-PA), aims to provide U.S. pharmaceutical companies with a level-playing field regarding controlled substances exports. This section would amend 21 U.S.C. § 953(f) to permit a controlled substance to be exported from a second country to another country that is a member of the European Economic Area. Such controlled substances could continue to be exported from any country that is a member of the European Economic Area to any other such country provided that certain conditions are met by each subsequent country to which the controlled substance is exported. Under Section 5041, the U.S. Attorney General would be prohibited from promulgating and enforcing any regulation, subregulatory guidance, or enforcement policy which impedes re-exportation among European Economic Area countries.

D. Subtitle D--Medical Device Reforms

Section 5061, led by Representative John Shimkus (R-IL), would amend the FDCA to allow FDA to rely on third party accredited bodies to certify minor manufacturing changes. Such accredited persons would be required to assess and certify a facility's capability to evaluate and implement: (A) technology changes to devices that were found to be substantially equivalent to a predicate device for purposes of classification under Section 513(f); (B) changes in the manufacturing of a device; (C) changes that do not alter a device's fundamental technology; and (D) labeling changes described in 21 C.F.R. § 814.39(d) (or any successor regulations). The Committee is requesting comments for more specificity on what types of changes would be appropriate under this section (e.g., technology, manufacturing, modifications that do not alter a device's fundamental technology, and labeling), and which types of changes would not.

An assessment pursuant to this section must assess the facility in which a device is manufactured or designed and determine whether the facility's quality system, including the facility's design controls, is capable of evaluating, on a continuing basis, the types of changes noted above so as to provide a reasonable assurance of safety and effectiveness. FDA would accredit and renew accreditation of such persons or entities consistent with Section 523 of the FDCA (21 U.S.C. § 360m).[41] The person who registers and lists the device under Section 510 must be the person who initiates the use of one or more accredited persons who makes the facility assessment.

[41] The provisions of subsections (a)(2), (a)(3), and (c) of section 523 would not apply for purposes of Section 524.

Under Section 5061, if a facility is determined by an accredited person to have a quality system as described above, then the facility need not submit a premarket notification under Section 510(k) nor a supplement under Section 515(d)(6) with respect to any change listed in subsection (a)(1), so long as the accredited person determines, in writing, that the change is in compliance with the requirements of this Act and the regulations thereunder, including 21 C.F.R. Part 820 (or any successor regulations). This determination would last for two years from the date of such determination and may be renewed through the same processes, and will continue to apply with respect to changes made during such two-year period, irrespective of whether such determination is renewed after such two-year period.

Section 5062, led by Representative John Shimkus (R-IL), would clarify that "valid scientific evidence," for the purposes of 21 U.S.C. § 360c, includes "well-documented case histories, including registry data, that are collected and monitored under an acceptable protocol, and studies published in peer-reviewed journals that are internationally recognized as authoritative sources of information." Such data may include data collected in countries outside the U.S. so long as it otherwise meets the criteria previously stated. This section would establish that such data are "presumed valid based on the peer-review process that supports publication of the studies, and the Secretary may not require submission of the data for the Secretary's review."

Section 5063, led by Representative John Shimkus (R-IL), would ensure that FDA reviewers involved in the review of premarket submissions under Section 515 or 510(k), including supervisors, receive training regarding the meaning and implementation of the least burdensome means concept in the context of the use of the term in 21 U.S.C. §§ 360c(a)(3)(D) and (i)(1)(D).[42] Such training must include when advisory panels are appropriate and necessary to review premarket submissions under Sections 515 or 510(k). Such employees must receive re-training on an annual basis. Under this section, the Secretary must also ensure that adequate guidance documents exist to describe the least burdensome concept and are available to FDA personnel involved in premarket submission review. The guidance, which must be published within 12 months after enactment, must include tools that such persons may use to ensure adherence to the least burdensome means concept, such as an evidentiary matrix based on a device type's benefits and risks. The CDRH Ombudsman would be required to conduct an audit to determine

[42] In October 2002, CDRH issued guidance defining the term "least burdensome means" as "a successful means of addressing a premarket issue that involves the most appropriate investment of time, effort, and resources on the part of industry and FDA." FDA, The Least Burdensome Provisions of the FDA Modernization Act of 1997: Concept and Principles; Final Guidance for FDA and Industry (Oct. 2002) *available at* http://www.fda.gov/downloads/MedicalDevices/DeviceRegulationandGuidance/GuidanceDocuments/ucm085999.pdf.

whether the responsible unit within the center is implementing the least burdensome means concept, and must include interviews with a representative sample of persons from industry regarding their experience in the device premarket review process.

Section 5064, authored by Representative John Shimkus (R-IL), would improve the process of government recognition of appropriate standards set by the medical community. Specifically, this section would give the Secretary discretion (instead of a requirement) to recognize performance standards under 21 U.S.C. § 360d. Under this section, the Secretary would be required to make a decision with respect to an applicable standard within 60 days after the date on which the applicable standard development organization makes such standard available, and if the Secretary chooses not to recognize a standard, the Secretary must publish in the Federal Register the basis for refusing to recognize the standard. This section would also require FDA employees who review premarket submissions for devices to undergo training on the concept and use of recognized standards to facilitate the premarket review of devices and to provide reasonable assurance of safety and effectiveness, including standards relevant to an employee's area of device review.

Section 5065, led by Health Subcommittee Vice Chair Brett Guthrie (R-KY), would streamline the process for marketing of Class I medical devices. This section would allow a Class I device to satisfy the requirement of the report required under 21 U.S.C. § 360(k)(report preceding introduction of devices into interstate commerce) by submitting a notification to the Secretary that contains a written determination by a person accredited under Section 523 that the methods used in, or the facilities and controls used for, the manufacture, processing, packing, or installation of such device conforms with the requirements of Section 520(f) and that is received by the Secretary not less than 5 business days before the Class I device is introduced, or delivered for introduction, into interstate commerce.

Section 5066, led by Representative John Shimkus (R-IL), would streamline the 510(k) process for medical devices by prohibiting FDA from: (I) refusing to accept an indication for use statement for a device to the extent the predicate for such device has the same indication statement; or (II) requiring from the person submitting the report information or data related to an indication other than the proposed indication in the report.

Section 5067, led by Representative Leonard Lance (R-NJ), would allow FDA the authority to apply the Humanitarian Device Exemption (HDE) to conditions that impact more than 4,000 patients in the U.S. annually, where the applicant demonstrates that the severity of the disease or condition is such that: (i) the public health requires a greater availability of the device to treat or diagnose such patients; and (ii) no satisfactory alternative is available for such treatment or diagnosis. The current language in 21 U.S.C. §§

360j(m)(1) and (2)(A) requires that the device be designed to treat or diagnose a disease or condition that affects *fewer* than 4,000 individuals in the U.S.[43] The Secretary must issue a guidance document within six months after enactment that defines the criteria for establishing "probable benefit" as that term is used in Section 520(m)(2)(C) of the FDCA.

Section 5068, led by Representative John Shimkus (R-IL), would streamline the FDA medical device classification panel process under 21 U.S.C. §360c(b)(5). Specifically, the Secretary would be required to convene classification panels within 60 days after the matters to be considered by the panel are ready. Within 30 days before such meeting, the Secretary must provide the panel and person whose device is subject to review any material on the matters to be considered during such meeting. No later than 14 calendar days before the date of the meeting, the Secretary would be required to make any material that is made available to the panel members available to the public (redacting any trade secret or confidential information. The Secretary must consult with the submitter regarding the person's recommendations on the expertise needed among the voting panel members to ensure that "adequate expertise"[44] is represented to assess: (I) the disease or condition for which the device is intended to cure, treat, mitigate, prevent, or diagnose; and (II) the technology of the device.

Under this provision, any meeting of a classification panel with respect to the review of a device must provide adequate time for initial presentations by the person whose device is specifically the subject of such review, the Secretary, or any other interested party, and for a response to any differing views by such person, and must encourage free and open participation by all interested persons. Any initial presentation made by the person whose device is specifically the subject of such review would be made before the Secretary's initial presentation. Such a meeting would be required to provide such person with

[43] Under this section, an order granting a request for an exemption does not in any way limit the number of devices that are subject to the exemption if such devices are determined by the Secretary to be medically necessary to treat, diagnose, or monitor individuals with diseases or conditions.

[44] This section defines this term to mean that the membership of the classification panel includes: (i) two or more voting members who are specialists or have other expertise in the disease or condition for which the device is under review; and (ii) an equal number of voting members who are knowledgeable about the technology of the device.

adequate time to respond to the Secretary's initial presentation. Following the initial presentations and responses, the panel would have a period of time the panel considers appropriate to pose to the person whose device is the subject of the review questions that: (I) have been provided by the Secretary to the panel for purposes of the panel's review of the device; and (II) have been agreed upon by the Secretary and such person for such purposes.

E. Subtitle E--Supply Chain Security for Devices

Sections 5081-5088, also referred to as the "Device Distribution Licensing Act of 2015", led by Representative Bob Latta (R-OH), would establish a national framework for the licensure of medical device wholesalers and third-party logistics providers, similar to what Congress enacted for prescription drugs with the Drug Quality and Security Act (DQSA)(P.L. 113-54). These sections would largely mirror Title II of the DQSA--the Drug Supply Chain Security Act (DSCSA)--and include many of the same definitions of key terms and requirements, with some variation to account for the differences between manufacturing or distributing prescription medical devices[45] versus drugs. These sections, however, would not impose any new "track and trace" requirements on medical device distributors or other medical device trading partners.

Section 5083 would require that not later than January 1, 2016, medical device manufacturers[46] (defined to included importers[47]) wholesale distributors, third-party logistics providers (3PL)[48], and dispensers[49]

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[45] The term "prescription device" means a restricted device, as defined in section 520(e)(1): (A) that is intended for use by humans; (B) which, because of any potentiality for harmful effect, the method of its use, or the collateral measures necessary to its use is not safe except under the supervision of a practitioner licensed by law to direct the use of such device; (C) for which the Secretary has determined adequate directions for use cannot be prepared; and (D) that is required to carry on its label 'Rx', 'Rx only', a designation for physician-use or dentist-use only, or a statement that Federal law restricts the device to sale by or on the order of a licensed health care practitioner.

[46] The term "manufacturer" means the person who manufactures, prepares, propagates, compounds, assembles, or processes a prescription device by chemical, physical, biological, or other procedure. The term includes any person who: (A) repackages or otherwise changes the container, wrapper, or labeling of a prescription device in furtherance of the distribution of the prescription device from the original place of manufacture; (B) initiates specifications for prescription devices that are manufactured by a second party for subsequent distribution by the person initiating the specifications; (C) manufactures components or accessories that are prescription devices that are ready to be used and are intended to be commercially distributed and intended to be used as is, or are processed by a licensed practitioner or other qualified person to meet the needs of a particular patient; (D) reprocesses a single-use prescription device[46] that has previously been used on a patient; (E) is an importer; or (F) is the United States agent of a foreign manufacturer.

[47] The term "importer" means any person who imports a prescription device into the United States and who furthers the marketing of the prescription device from the original place of manufacture to the person who makes final delivery or sale to the ultimate user, but who does not repackage or otherwise change the container, wrapper, or labeling of the prescription device or prescription device package.

use only authorized trading partners--similar to the requirements now applicable to drugs under the DSCSA.[50]

Section 5084 would prohibit any person from engaging in the wholesale distribution of a prescription device in any state unless validly licensed under state law or Section 586B of the FDCA. Within one year after enactment, FDA would be required to establish regulations for the licensing of wholesale distributors of prescription medical devices, including the revocation, reissuance, and renewal of such license. Section 5084 outlines the specific areas and topics that these regulations must address (e.g., personnel, security and storage, inspection, etc.).

3PLs also would be required to obtain a license under Section 5084 or their applicable state, but may not be required by states to obtain a license as a wholesale distributor if the entity never assumes an ownership interest in the prescription device it handles. Section 5085 would require FDA within one year of enactment to issue regulations regarding the standards for licensing 3PLs, addressing specified requirements such as a third-party accreditation program, warehouse and facility requirements, inspection, and policies and procedures.

Section 5086 would require the Secretary to establish a process through guidance that would allow wholesale distributors and 3PLs to request a waiver from any of the requirements set forth in Sections 586B or 586C. Section 5087 would preempt states from establishing or continuing any standards, requirements or regulations for device distribution or 3PLs that are inconsistent with, different than, or in addition to the federal standards and requirements. Further, this section would prohibit states from requiring licensure as a wholesale device distributor or 3PL for any activity related to the manufacture, distribution, delivery, or dispensing of a device for which licensure is not required under the federal

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[48] The term "third-party logistics provider" means an entity that provides or coordinates warehousing of, or other logistics services with respect to, a prescription device in interstate commerce on behalf of a manufacturer, wholesale distributor, or dispenser of a prescription device, but does not take ownership of the prescription device, nor have responsibility to direct the sale or disposition of the prescription device.

[49] Means any person who makes final delivery or sale of a prescription device to the ultimate user, but who does not repackage or otherwise change the container, wrapper, or labeling, including: (A) a retail pharmacy, hospital pharmacy, or group of chain pharmacies under common ownership and control that do not act as a wholesale distributor; (B) a hospital, ambulatory surgical facility, nursing home, outpatient diagnostic facility, or outpatient treatment facility; and '(C) a physician or other health care provider authorized by applicable law to prescribe and administer prescription devices.

[50] 3PLs must have a valid license under State law or section 586C; dispensers must have a valid license under state law.

provisions. States, however, could continue to enforce wholesale distributor and 3PL licensure requirements until FDA finalized the implementing regulations.

Section 5088 would make failure to comply with any requirement under Sections 586A, 586B, or 586C a prohibited act under the FDCA (21 U.S.C. § 331).

VII. Senate Innovation for Healthier Americans Report

On January 29, 2015, Senate HELP Committee Chairman Lamar Alexander (R-TN) and Senator Richard Burr (R-NC) released a report outlining their ideas to improve and accelerate drug discovery and development processes.[51] The report, titled "Innovation for Healthier Americans: Identifying Opportunities for Meaningful Reform to Our Nation's Medical Product Discovery and Development," does not include specific draft legislation, but the proposals address many of the same problems targeted in the 21st Century Cures discussion document. Specifically, the report identifies "five themes" in its effort:

- It costs too much to bring medical products through the pipeline to patients.
- As science and technology advance, the discovery and development process takes too long for medical products to make their way to patients.
- FDA's responsibilities have grown to include many activities unrelated to the core function of regulating medical products to advance the public health.
- The disparity in scientific knowledge at FDA and the fast pace of biomedical innovation are slowing, and in some cases, stifling, innovation in American medicine.
- A working FDA is essential to continuing biomedical innovation in the United States and maintaining America's global leadership in medical innovation.

Among the ideas discussed in the Senate's report are:

- Potential ways to modernize FDA's clinical trial process and review standards and policies;
- Ways to incentivize medical products development, including market exclusivity periods; and
- Potential changes to FDA's use of guidance

[51] *Available at* http://www.help.senate.gov/newsroom/press/release/?id=c9abb2c5-58a3-4c8f-b9b3-ae689b109042&groups=Chair.

Chairman Alexander and Senator Burr are soliciting feedback on its report, which the HELP Committee will use to inform the creation of a "bipartisan legislative package" on this subject. The Committee is accepting feedback at Innovation@help.senate.gov no later than February 23, 2015.

If you have any questions about any of the topics discussed in this advisory, please contact your Arnold & Porter attorney or any of the following attorneys:

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