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H.R. 6: The 21st Century Cures Act

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Summary

On July 10, 2015, the House passed H.R. 6, the 21st Century Cures Act, on a vote of 344 to 77. Eight amendments were offered; five were approved by voice vote, two failed by recorded vote, and one was withdrawn. The House Energy and Commerce Committee, on May 21, 2015, unanimously ordered to be reported H.R. 6 and the House Committee on Rules published a committee print of the bill on July 2, 2015. On July 7, 2015, H.R. 6 was reported by the Committee on Energy and Commerce (H.Rept. 114-190), and the House Committee on Ways and Means was discharged from further consideration of the bill.

The bill would reauthorize the National Institutes of Health (NIH) through FY2018 and provide other funding to the agency through FY2020. In addition, the bill would promote and encourage more strategic planning for research conducted by NIH; change loan support for young, emerging scientists; promote pediatric research; and encourage more collaborative research activities. The bill also focuses on changes to the Food and Drug Administration's (FDA's) regulatory procedures for drugs and devices by requiring the issuance of more guidance and increasing regulatory flexibility in areas such as precision (or personalized) medicine, types of data that could serve as evidence of safety and effectiveness, antibiotic drug development, orphan drugs, and medical devices. The bill proposes additional funding for the FDA to support some of its efforts in certain specified areas.

H.R. 6 consists of four separate titles. Title I focuses on discovery-related issues and is concentrated on matters related to the NIH, including developing strategic plans, cultivating young scientists, and promoting more collaboration among NIH researchers, grant recipients, and institutions. Title II targets the development of new and more innovative drugs and medical devices and the regulatory processes in place to consider these products. Title III includes provisions related to the delivery of health care, including interoperability of electronic health information technology and the treatment of disposable medical technologies. Title IV includes Medicare and Medicaid changes being proposed to offset the costs of the NIH- and FDA-related changes in Titles I, II, and III. These proposed offsets include changes in prior authorization procedures for power mobility devices under Medicare, as well as a proposed drawdown in the nation's strategic petroleum reserve.

This report provides a brief summary of each provision of H.R. 6 as passed by the House on July 10, 2015. Each summary includes a brief description of current law and an explanation of how the bill would change current law. A list of the abbreviations used throughout this report appears in **Appendix B**.

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Overview

On July 10, 2015, the House passed H.R. 6, the 21st Century Cures Act, on a vote of 344 to 77. Eight amendments were offered; five were approved by voice vote, two failed by recorded vote, and one was withdrawn. On May 21, 2015, the House Energy and Commerce Committee unanimously ordered to be reported H.R. 6.[1] The House Committee on Rules published a committee print of the bill on July 2, 2015.[2] On July 7, 2015, H.R. 6 was reported by the Committee on Energy and Commerce (H.Rept. 114-190), and the House Committee on Ways and Means was discharged from further consideration of the bill.

Amendments to H.R. 6

Amendment 1 (Brat) to reform the NIH and Cures Innovation Fund to make it a discretionary program, **failed by recorded vote Y-141 N-281**.

Amendment 2 (Young) to create authority within NIH prize program to incentivize health innovation and create breakthrough research and technology, **approved by voice vote**.

Amendment 3 (Lee) to strike the provision that applies policy riders in appropriations bills to NIH & FDA funding in H.R. 6, **failed by recorded vote Y-176 N-245**.

Amendment 4 (Castro) to ensure that underrepresented individuals in the sciences (women and minorities) are included as a focus topic in the report on Supporting Young Emerging Scientists, **approved by voice vote**.

Amendment 5 (Slaughter) to direct CDC to conduct a study to determine how the additional payments for certain drugs are affecting usage practices and the development of drug resistance, **approved by voice vote**.

Amendment 6 (Fitzpatrick) to express a sense of Congress that recording Unique Device Identifiers at the point-of-care in electronic health record systems could significantly enhance the availability of medical device data for post-market surveillance purposes, **approved by voice vote**.

Amendment 7 (Polis) to direct FDA to issue a report on the risks and benefits associated with a two-tiered approval process that would permit certain medical devices to provisionally come to market if they have demonstrated safety but not efficacy, **withdrawn**.

Amendment 8 (Jackson Lee) to direct the HHS Secretary to conduct outreach to certain colleges, universities and other institutions to ensure that health professionals from underrepresented populations are aware of the research opportunities under this Act, **approved by voice vote**.

While consisting of many different provisions, the bill is primarily focused on efforts to increase strategic investments in medical research at the National Institutes of Health (NIH) and change some aspects of how the Food and Drug Administration (FDA) executes its regulatory oversight mission with regard to the review and approval of new drugs, biologics, and medical devices.

H.R. 6 is the result of a series of hearings and roundtable meetings hosted by the House Energy and Commerce Committee dating back to spring 2014.[3] The hearings and roundtables focused on a broad range of topics, including modernizing clinical trials, incorporating patient perspectives

[1] U.S. House of Representatives, Committee on Energy and Commerce, *Full Committee Vote on the 21st Century Cures Act*, May 19, 2015, http://energycommerce.house.gov/markup/full-committee-vote-21st-century-cures-act. The committee website links to more than one version of the bill and several amendments; the committee marked up a composite of a Committee Print dated May 19, 2015, named UPTON_005 (http://docs.house.gov/meetings/IF/IF00/20150519/103516/BILLS-1146ih.pdf) and a manager's amendment dated May 20, 2015, named UPTON_006 (http://docs.house.gov/meetings/IF/IF00/20150519/103516/BILLS-114-6-U000031-Amdt-3.pdf).

[2] Rules Committee Print 114-22, Text of H.R. 6, 21st Century Cures Act, based on H.R. 6 as ordered reported by the Committee on Energy and Commerce, July 2, 2015, http://docs.house.gov/billsthisweek/20150706/CPRT-114-HPRT-RU00-HR6.pdf.

[3] House Energy and Commerce Committee, 21st Century Cures Roundtable, http://energycommerce.house.gov/event/21st-century-cures-roundtable.

into medical research and regulatory processes, precision/personalized medicine, digital health care, and more. At present, no companion legislation to H.R. 6 has been introduced in the Senate. However, the Senate Health, Education, Labor, and Pensions (HELP) Committee has started work on issues related to medical innovation[4] and committee leadership has indicated they plan to continue this work in the 114th Congress.

The July 2, 2015, Rules Committee Print differs from the version that the Committee on Energy and Commerce ordered to be reported on May 21, 2015. Among the most notable changes is the reduction in proposed funding for the NIH Innovation Fund, from a level of $10 billion over five years in the version voted on by Energy and Commerce to a level of $8.75 billion. The other most significant changes to the bill are found in the proposals to offset the costs for the legislation. A provision in the May 21 version that would have delayed certain Medicare prepayments for prescription drug plans has been deleted from the current version of the bill. The latest version (July 2) includes a new provision to expand the use of prior authorization under Medicare for power mobility devices (wheelchairs and scooters) along with an expansion of the provision in the May 21 version for drawdowns from the Strategic Petroleum Reserve, among others.

Some of the key themes in the bill are innovation, flexibility, and transparency. H.R. 6 represents an effort to maintain or increase medical innovation as reflected in research conducted or funded by the NIH. The bill has numerous provisions related to increasing regulatory flexibility by FDA in its processes for reviewing and approving drugs, biologics, and medical devices. In particular, the bill increases the ability of industries subject to FDA regulation (e.g., pharmaceutical companies, device makers) and the individuals who are or may become patients who could benefit from future products, to have a greater voice in the regulatory process and to streamline the various ways in which FDA ensures safe and effective medications and medical devices enter the market. The bill attempts to improve the transparency of data—for researchers, consumers, and regulated entities—by helping to provide enhanced and timelier information for decisionmakers.

H.R. 6 would reauthorize the NIH through FY2018 and provide $8.75 billion in additional funding for an innovation fund through FY2020. The bill would promote and encourage more strategic planning for research conducted by NIH; change loan support for young, emerging scientists; promote pediatric research; and encourage more collaborative research activities. The bill focuses on changes to the FDA's regulatory procedures for drugs and devices by requiring the issuance of more guidance and increasing regulatory flexibility in areas such as precision (or personalized) medicine, antibiotic drug development, orphan drugs, and medical devices. The bill also proposes $550 million in additional funding over five years to support efforts in certain specified areas, mostly in FDA. FDA estimates it could cost more than $900 million to implement the legislation; "any unfunded mandates in the bill may require resources to be shifted from other activities."[5]

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[4] U.S. Senate Committee on Health, Education, Labor, and Pensions, Full Committee Hearing, "Continuing America's Leadership in Medical Innovation for Patient," March 10, 2015, http://www.help.senate.gov/hearings/continuing-americas-leadership-in-medical-innovation-for-patients. Statement by Senator Lamar Alexander, "Senate Health Committee Holds First Hearing on Innovation Initiative: How to Get Medical Devices, Drugs, Treatments from Discovery to the Medicine Cabinet," March 10, 2015, http://www.help.senate.gov/chair/newsroom/press/-senate-health-committee-holds-first-hearing-on-innovation-initiative-how-to-get-medical-devices-drugs-treatments-from-discovery-to-the-medicine-cabinet.

[5] Derrick Gingery, "FDA Program Cuts Loom if "Cures" Bill Isn't Fully Funded, Ostroff Warns," *The Pink Sheet Daily*, June 3, 2015. See **Appendix A** for a list of new requirements (e.g., regulations, guidance, reports, etc.) that would be created by H.R. 6.

Many interested groups (manufacturers, medical schools, disease and patient advocates, researchers, and past and present regulators) have opined publicly on specific provisions in various drafts of the bill, not all of which have been supportive.[6] This report provides summary descriptions of the bill's provisions, along with background to give context, not a full analysis of their impact. The Congressional Budget Office has published a cost estimate.[7]

Summary of Provisions

Section 2. NIH and Cures Innovation Fund

Background

The National Institutes of Health is the lead federal agency charged with performing and supporting biomedical and behavioral research. It also has major roles in training biomedical researchers and disseminating health information. Congress doubled the NIH budget from $13.65 billion to $27.1 billion in the five-year period from FY1998 to FY2003; during that period, annual increases in the 14%-15% range were the norm. Since then, increases from regular appropriations have been between 1.0% and 3.2% each year.[8] The growth rate of the NIH budget has been at or below the rate of inflation, which for biomedical research in FY2015 is estimated to be 2.2%.[9] NIH funding in FY2015 is 22% lower than the FY2003 level, the peak of the doubling period in constant 2012 dollars.[10]

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[6] See, for example, The Editorial Board, "How Not to Fix the FDA," *The New York Times*, July 20, 2015, http://www.nytimes.com/2015/07/20/opinion/how-not-to-fix-the-fda.html?_r=0; Rita F. Redberg and Sanket S. Dhruva, "The FDA's Medical Device Problem," *The New York Times*, July 17, 2015, http://www.nytimes.com/2015/07/17/opinion/the-fdas-medical-device-problem.html; Jerry Avorn and Aaron S. Kesselheim, "The 21st Century Cures Act—Will It Take Us Back in Time?" New England Journal of Medicine, June 3, 2015, http://www.nejm.org/doi/pdf/10.1056/NEJMp1506964; Michael Causey, "Congress Crawls Out of 20th Century to Push Bipartisan 'Cures' Legislation," Quality Digest, June 9, 2015, http://www.qualitydigest.com/inside/fda-compliance-article/060915-congress-crawls-out-20th-century-push-bipartisan-cures#; Editorial Board, "A breakthrough for biomedicine," May 29, 2015, Denver Post, May 29, 2015, http://www.denverpost.com/editorials/ci_28216439/breakthrough-biomedicine; Quardricos Bernard Driskell, "What will it take to cure psoriatic disease?" National Psoriasis Foundation blog, June 2, 2015, http://blog.psoriasis.org/blog/what-will-it-take-cure-psoriatic-disease; Newt Gingrich, "21st Century Cures is a Major Breakthrough," Gingrich Productions, June 3, 2015, http://www.gingrichproductions.com/2015/06/21st-century-cures-is-a-major-breakthrough/; Gregg Gonsalves, Mark Harrington, and David A. Kessler, "Don't Weaken the F.D.A.'s Drug Approval Process," New York Times, June 11, 2015, http://www.nytimes.com/2015/06/11/opinion/dont-weaken-the-fdas-drug-approval-process.html?ref=opinion; Bronwyn Mixter, "Watchdog, Consumer Groups Say 'Cures' Bill Could Lower Certain Drug Approval Standards," BNA, June 3, 2015, http://healthlawrc.bna.com/hlrc/4225/split_display.adp?fedfid=69846101&vname=hcenotallissues&fn=69846101&jd=69846101; Bernard Muller, "Beyond the Ice Bucket," The Hill, June 10, 2015, http://thehill.com/blogs/congress-blog/healthcare/244456-beyond-the-ice-bucket; and Ed Silverman, "Will the 21st Century Cures Bill Lower Standards for Some Drug Approvals?" Pharmalot blog in Wall Street Journal, May 29, 2015, http://blogs.wsj.com/pharmalot/search/will%20the%2021st%20century%20cure/?s=will+the+21st+century+cure.

[7] Congressional Budget Office, *H.R. 6, 21st Century Cures Act*, Cost Estimate for Rules Committee Print 114-22, July 7, 2015, http://www.cbo.gov/publication/50361.

[8] For further information, see CRS Report R43341, *NIH Funding: FY1994-FY2016*.

[9] The Biomedical Research and Development Price Index (BRDPI) is developed each year for NIH by the Bureau of Economic Analysis of the Department of Commerce. It reflects the increase in prices of the resources needed to conduct biomedical research—including personnel services, supplies, equipment—and indicates how much the NIH budget must change to maintain purchasing power. See http://officeofbudget.od.nih.gov/gbiPriceIndexes.html.

[10] For further information, see CRS Report R43341, *NIH Funding: FY1994-FY2016*.

A recent analysis of U.S. expenditures on biomedical research found that "U.S. government research funding declined from 57% (2004) to 50% (2012) of the global total, as did that of U.S. companies (50% to 41%), with the total U.S. (public plus private) share of global research funding declining from 57% to 44%. Asia, particularly China, tripled investment from $2.6 billion (2004) to $9.7 billion (2012) preferentially for education and personnel."[11] The United States continues to be the top supporter of both public and industry medical research.[12] However, some Members of Congress and many in the biomedical research community have expressed concern over the rapidly increasing investments being made by other countries in this area of research.

Many of those who are concerned over the U.S. global position in biomedical research investment have made frequent calls for increased support for research at the NIH. However, another recent analysis of U.S. biomedical research funding cautioned that the past pattern of rapid doubling of the NIH budget followed by slowdowns in federal funding "created an unsustainable hypercompetitive system that is discouraging even the most outstanding prospective students from entering our profession—and making it difficult for seasoned investigators to produce their best work."[13] Rather than short-term infusions of cash that disappear, the authors recommend that greater emphasis be placed on the predictable and stable growth of federal funds for the research enterprise.[14] In responding to questions raised by Senator Elizabeth Warren during a May 5, 2015, Senate hearing, NIH Director Francis Collins agreed that continued NIH budget increases—ranging from 3.7% annually to inflation plus 4% or 5%—would be preferred to a temporary larger investment that disappears.[15]

The Food and Drug Administration plays a central role in protecting the public health in the United States by regulating most of the food supply and vitally important medical products, including drugs, devices, and biologics that affect American lives on a daily basis. In performing this role, FDA regulates some of the most successful and innovative companies in the U.S. economy, such as those in the pharmaceutical and medical device industries. In recent years, some have argued that FDA is underfunded and at risk of being unable to fulfill all its statutory responsibilities assigned by Congress. Implementing new statutory provisions involves the development of new regulations and extensive communication with industry and the public; carrying out the new responsibilities requires additional FDA staff time as well as agency resources.[16]

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[11] Hamilton Moses, David H. M. Matheson, Sarah Cairns-Smith, et al., "The Anatomy of Medical Research: U.S. and International Comparisons," *Journal of the American Medical Association*, vol. 313, no. 2 (January 13, 2015), pp. 174-189.

[12] Overall medical research funding in the United States was $117.2 billion in 2011. See Figure 8 on page 181 in Hamilton Moses, David H. M. Matheson, Sarah Cairns-Smith, et al., "The Anatomy of Medical Research: U.S. and International Comparisons," *Journal of the American Medical Association*, vol. 313, no. 2 (January 13, 2015).

[13] Bruce Alberts, Marc W. Kirschner, Shirley Tilghman, and Harold Varmus, "Rescuing U.S. biomedical research from its systemic flaws," *Proceedings of the National Academy of Sciences*, vol. 111, no. 16 (April 22, 2014), pp. 5773-5777.

[14] Ibid., p. 5775.

[15] U.S. Congress, Senate Committee on Health, Education, Labor, and Pensions, *Continuing America's Leadership: Realizing the Promise of Precision Medicine for Patients*, 114th Cong., 1st sess., May 5, 2015.

[16] A recent example is implementation of the Food Safety Modernization Act (FSMA). The Congressional Budget Office indicated that FDA would require $580 million between 2011 through 2015 to carry out FSMA; so far Congress has appropriated less than half of this amount.

FDA's program level, the amount that FDA can spend, is composed of direct appropriations (also referred to as budget authority) and user fees collected from the regulated industry.[17] For FY2015, FDA's program level is $4.5 billion, of which 42.3% ($1.902 billion) comes from user fees. By statute and by five-year agreements between FDA and the regulated industries that pay the fees, FDA may use user fee revenue only for specified activities. This requirement, along with tasks that the Committee on Appropriations reports direct FDA to conduct, influences the priorities of the agency, potentially leaving other tasks inadequately addressed.

Provision

The provision would establish in the U.S. Treasury an NIH and Cures Innovation Fund and would provide the Fund with $1.86 billion in mandatory funds per year for FY2016 through FY2020.[18] The amounts appropriated to the Fund would be in addition to amounts otherwise made available to the Department of Health and Human Services (HHS).

Of the amounts made available to the NIH and Cures Innovation Fund for each fiscal year, $1.75 billion would be for "NIH Biomedical Research" and $110 million would be for "Cures Development." Of the amounts for NIH biomedical research for a fiscal year, not less than $500 million is for the Accelerating Advancement Program. Of the remaining funds in that fiscal year, not less than 20% is for high-risk, high-reward research, and not less than 35% is for early stage investigators (defined as the principal investigator of the proposed research, who has been awarded no more than one substantial, competing grant, and who is within 10 years of having completed a medical residency or terminal degree). Of the total amount made available for the NIH Innovation Fund in a fiscal year, not more than 10% is for intramural research.

The NIH and Cures Innovation Fund would not be subject to any transfer authority of the NIH Director or the Secretary of HHS, such as the PHS Evaluation Set-Aside, the Common Fund, the 1% transfer authority of the NIH Director, or the Nonrecurring Expenses Fund.[19]

The provision states that amounts in the NIH and Cures Innovation Fund that are allocated for "NIH biomedical research" would be used only to conduct or support certain biomedical research activities. The provision specifies some of these activities, such as research carried out by an early stage investigator, research carried out by a small business, the Accelerating Advancement Program, and development and implementation of the NIH research strategic plan.

The provision states that amounts in the NIH and Cures Innovation Fund that are allocated for "cures development" would only be used for activities of the following nine provisions:

- PHSA Section 229A, as added by Section 1123 (the natural history of diseases);
- Section 2001 and the amendments made by such section (development and use of patient experience data to enhance structured risk-benefit assessment framework);

[17] Beginning with the Prescription Drug User Fee Act (PDUFA, P.L. 102-571) in 1992, Congress has authorized FDA to collect fees from industry sponsors of certain FDA-regulated products and to use the revenue to support statutorily defined activities, such as the review of product marketing applications.

[18] During floor debate on H.R. 6, Amendment 1 (Brat) was defeated on a vote of Yea-141 Nay-281. Amendment 1 would have made the NIH and Cures Innovation Fund a discretionary spending program.

[19] Nonrecurring Expenses Fund (NEF) is an account within the Department of the Treasury. The HHS Secretary is authorized to transfer to the NEF unobligated balances of expired discretionary funds. NEF funds are available until expended for use by the HHS Secretary for capital acquisitions, including facility and information technology infrastructure. Congressional appropriators must be notified in advance of any planned use of NEF funds. NEF was created by Section 223 of Division G of the Consolidated Appropriations Act, 2008 (42 U.S.C. 3514a).

- Section 2021 and the amendments made by such section (qualification of drug development tools);
- Section 2062 and the amendments made by such section (utilizing evidence from clinical experience);
- Section 2161 (grants to study the process of continuous drug manufacturing);
- Section 2201 and the amendments made by such section (priority review for breakthrough devices);
- Section 2221 and the amendments made by such section (third-party quality system assessments);
- Sections 2241, 2242, and 2243 and the amendments made by such sections (health software); and
- FFDCA Section 513(j), as added by Section 2223 (training and oversight in least burdensome appropriate means concept).

Of the biomedical research funded under the provision, the NIH Director would ensure coordination among the various research institutes, centers, agencies, departments, offices of the federal government and minimize unnecessary duplication. This section requires the NIH Director to establish the Accelerating Advancement Program under which for every $1 of NIH Innovation Fund made available by the NIH Director to an NIH research Institute or Center, the Institute or Center contributes $1 of other funding to accomplish important biomedical research objectives. The scientifically based strategic plan would identify focus areas in which the resources of the NIH Innovation Fund can be used on biomedical research to expand knowledge, find more effective treatments, and address unmet needs in the United States. Focus areas include biomarkers, precision medicine, infectious diseases, and antibiotics. The strategic plan would include objectives for each strategic focus area and ensure that basic research remains a priority. The strategic plan would be updated not less than every 18 months.

The House and Senate Committees on Appropriation could provide for the transfer of funds in the NIH and Cures Innovation Fund for the authorized uses specified in the provision (NIH biomedical research and cures development).

Funds appropriated to the NIH and Cures Innovation Fund would be used to supplement, not supplant, the funds otherwise made available to HHS, are subject to the requirements and limitations of the most recently enacted regular or full-year continuing appropriation Act or resolution for NIH or FDA programs, and may be used only for the activities specified in the provision.[20]

[20] Amendment 1 (Brat) to H.R. 6 would have struck subsection (f) and all the terms and conditions listed here. Amendment 1 was defeated on a vote of Yea-141 Nay-281. Amendment 3 (Lee), defeated on a vote of Yea-176 Nay-245, would have struck paragraph (f)(2). Paragraph (f)(2) would require that the funds appropriated to the NIH and Cures Innovation Fund be subject to the requirements and limitations—also called policy riders—of the most recently enacted regular or full-year continuing appropriation Act or resolution for NIH or FDA programs.

Title I—Discovery

Subtitle A—National Institutes of Health Funding

Section 1001. National Institutes of Health Reauthorization

Background

NIH derives its statutory authority from the Public Health Service Act of 1944 (PHSA), as amended.[21] Section 301 of the PHSA grants the Secretary of HHS broad permanent authority to conduct and sponsor research.[22] In addition, Title IV of the PHSA, "National Research Institutes," authorizes in greater detail various activities, functions, and responsibilities of the NIH Director and the institutes and centers.[23] The last major NIH reauthorization was the NIH Reform Act of 2006.[24] The NIH Reform Act authorized total funding levels for NIH appropriations for FY2007 ($30,331,309,000), FY2008 ($32,831,309,000), and such sums as necessary for FY2009. Overall NIH authorization expired at the end of FY2009 and has not been extended by Congress. Annual appropriations, together with Section 301 of the PHSA, provide authority for NIH programs to continue from FY2009 to the present.

Provision

The provision would authorize appropriations for NIH in FY2016 ($31,811,000,000), FY2017 ($33,331,000,000), and FY2018 ($34,851,000,000).

Section 1002. Prize Competitions

Background

Section 105 of the America COMPETES Reauthorization Act of 2010 (P.L. 111-358) provided federal agencies with broad authority to carry out programs designed to stimulate innovation through prize competitions.[25] Before passage of P.L. 111-358, only certain federal agencies had the authority to initiate prize competitions. The White House Office of Science and Technology Policy (OSTP) has published annual reports on the implementation of Section 105 as required by P.L. 111-358. Currently a number of federal government agencies, including NIH, sponsor challenges or prize competitions in science and medical research. A current list of such challenges can be found at a federal government website.[26] A search of the website on July 15, 2015, resulted in seven competitions conducted by the "National Institutes of Health." Examples of research topics covered in the various challenges: breast cancer genetics, antimicrobial resistance, and drug abuse and addiction research.

[21] 42 U.S.C. §§201-300mm-61.

[22] 42 U.S.C. §241.

[23] 42 U.S.C. §§281-290b.

[24] P.L. 109-482

[25] For more information, see CRS Report R43880, *The America COMPETES Acts: An Overview*.

[26] https://www.challenge.gov/list/.

Provision

The provision would amend the PHSA by adding a new Section 409K that would require the Director of NIH to establish an Innovation Prizes Program.[27] The goal(s) of the NIH prize program would be "identifying and funding areas of biomedical science that could realize significant advancement through the creation of a prize competition," and/or "improving health outcomes, particularly with respect to human diseases and conditions for which the public and private investment in research is disproportionately small relative to federal government expenditures on prevention and treatment activities."

Within six months of enactment, the Director of NIH would be required to: (1) design the prize competitions; (2) ensure the design is realistic (given the funds to be awarded), does not reflect any bias (concerning which innovations would be the best solution), allows any person to participate; and (3) submit a report to Congress on the design of the competitions.

The Director of NIH would be required to establish the "I-Prize Board" composed of 9 board members who would be appointed by the NIH Director and certain specified Members of Congress. The I-Prize Board would provide advice on identifying areas of biomedical science per the goals of the prize program and make recommendations on establishing the criteria for the prize competitions as well as how to conduct the prize competition. Board members would be appointed within 120 days of enactment, each for a 5-year term.

The provision specifies restrictions on financial conflict of interest that would be imposed on the members of the I-Prize board and any other NIH officer or employee involved in carrying out the prize competition. The provision also would require that the NIH Director, "with respect to an innovation," not award a prize "to any individual or entity that has a vested financial interest in any product or procedure that is likely to be developed or marketed because of such innovation."

The provision would allow for one or more contracts to be awarded by the NIH Director to perform a simulation of the prize competitions and use the simulation to assess the effectiveness of the competition design; a report to Congress on the simulation results would be submitted within 4 months of awarding such a contract. The provision would allow the NIH Director to enter into an agreement with one or more "tax exempt" entities to implement the prize competition. However, no more than 15% of funds or other assistance "shall be for administration of the prize competition and not less than 85% of such assistance shall be for activities in direct support of competitors."

The Director of NIH would be required to collect information on the medical efficacy of innovations funded via the prize program as well as the actual and potential effect on federal expenditures and submit reports to Congress as specified in the provision.

The provision would prohibit the federal government from acquiring the intellectual property rights from a participant in a prize competition without his or her written consent. The provision would allow the federal government to negotiate a license for the use of such intellectual property.

[27] Amendment 2 (Young), agreed to by voice vote during floor debate on H.R. 6, added this provision.

Subtitle B—National Institutes of Health Planning and Administration

Section 1021. NIH Research Strategic Plan

Background

Section 402(b)(5) of the PHSA specifies that the NIH Director "shall ensure that scientifically based strategic planning is implemented in support of research priorities as determined by the agencies of the National Institutes of Health." NIH provides access to many of its strategic plans on the agency's website.[28]

The focus of NIH research, and to some extent its organizational structure, have been criticized by some in the academic literature.[29] A point often made is that the United States spends more on health care than any of the other 30 countries that make up the Organization for Economic Cooperation and Development (OECD)—in fact, U.S. health care spending is more than 2.5 times the OECD average—and yet, the health of the U.S. populace, as measured by life expectancy, is ranked 24th of the 30 countries.[30] "Despite its name, NIH's mission has not generally been current health, per se, but rather research for tomorrow's health.... An agency devoted to current health would do well to focus on tobacco control, exercise, nutrition, sanitation, and more cost-effective delivery of health care—prevention and efficiency, rather than research on diseases currently not treatable."[31] Questioning or making changes to the focus of NIH research (whether basic, clinical, prevention, health care delivery, or patient-centered outcomes research) is perhaps "especially pertinent in light of the nation's continually mediocre public health outcomes, and their stark contrast to the sophistication and productivity of the biomedical research enterprise."[32]

Provision

The provision would add a new subsection (m) to Section 402 of the PHSA, which describes in further detail a Research Strategic Plan for NIH. Every five years, beginning in 2016, the NIH Director, along with the directors of the national research Institutes and Centers, as well as researchers, patient advocacy groups, and industry leaders, would be required to develop and maintain a biomedical research strategic plan. The strategic plan would be used to identify research opportunities and develop individual strategic plans for the research activities of each of the NIH Institutes and Centers. The Institute and Center (IC) plans would have a common template and identify strategic focus areas. The IC plans would consider and identify the return on investment to the U.S. public of such biomedical research and identify contributions to improving U.S. public health through biomedical research. Overarching and trans-NIH focus areas—or Mission Priority Focus Areas—would be identified that best serve the goals of preventing or eliminating the burden of a disease or condition and scientifically merit enhanced and focused research over the next five years. Rare and pediatric diseases would remain a priority. In developing the strategic plan, the NIH Director would be required to ensure that

[28] See for example http://report.nih.gov/strategicplans/#tab2.

[29] See for example Michael M. Crow, "Time to rethink NIH," *Nature*, vol. 471 (March 31, 2011), pp. 569-571; and, Robert Cook-Deegan, "Has NIH lost its halo?," *Issues in Science and Technology*, Winter 2015, pp. 37-47.

[30] Michael M. Crow, "Time to rethink NIH," *Nature*, vol. 471 (March 31, 2011), p. 570.

[31] Robert Cook-Deegan, "Has NIH lost its halo?," *Issues in Science and Technology*, Winter 2015, p. 43.

[32] Ibid.

maintaining the biomedical workforce, including the participation of scientists from traditionally underrepresented groups, would remain a priority. The initial strategic plan would be completed not later than 270 days after enactment. The NIH Director, in consultation with the directors of the national research Institutes and Centers, would be required to conduct annual progress reviews for each strategic focus area in the IC plans. The plans would be reviewed and updated every five years.

Section 1022. Increasing Accountability at the National Institutes of Health

Background

Section 405 of the PHSA specifies that the Director of the National Cancer Institute is appointed by the President and the Directors of the other NIH Institutes are appointed by the Secretary. Each NIH Institute Director reports directly to the NIH Director.

Section 202 of the Labor/HHS/ED Appropriations Act, 1993, states at the end of the section that the payment of compensation to consultants or individual scientists appointed for limited periods of time is "not to exceed the per diem rate equivalent to the maximum rate payable for senior-level positions," which is "not less than 120% of the minimum rate of basic pay payable for GS–15 of the General Schedule; and ... not greater than the rate of basic pay payable for level III of the Executive Schedule."[33]

Provision

The provision would amend Section 405 of the PHSA with regard to the appointment and terms of the Director of the National Cancer Institute and the directors of other NIH Institutes and Centers (ICs). It would require that directors of ICs be appointed by the NIH Director, with the exception of the Director of the National Cancer Institute (who would continue to be appointed by the President). It would add a new requirement that the term of office for the director of an IC be five years and authorize the NIH Director to remove an IC Director prior to the end of a five-year term. It would permit the director of an IC to be reappointed at the end of a five-year term, with no limit to the number of terms served. It would require that, if the office of a director of an IC becomes vacant before the end of a five-year term, the director appointed to fill the vacancy begin a new five-year term (as opposed to finishing the five-year term of the previous director). Each current IC Director would be deemed to be appointed for a five-year term as of the date of enactment.

The provision would remove compensation limitations for consultants and individual scientists as stipulated by Section 202 of the Labor/HHS/ED Appropriations Act, 1993.[34]

The provision would add a new requirement that before a new research grant is made, the IC Director will review and approve the award, taking into consideration the mission of the IC, the scientific priorities identified in the strategic plan, and "whether other agencies are funding programs or projects to accomplish the same goal."

The provision would require the Secretary to enter into an arrangement with the Institute of Medicine[35] (or other appropriate entity) to complete a study, not later than two years following

[33] P.L. 102-394 and 5 U.S.C. 5376.

[34] Ibid.

[35] In April 2015, the Institute of Medicine of the National Academies announced that, effective July 1, 2015, it would change its name to the National Academy of Medicine. Institute of Medicine of the National Academies, "Institute of

enactment, "on the extent to which biomedical research supported by the federal government is duplicative" and would require a report be submitted to Congress including recommendations on how to prevent such duplication.

Section 1023. Reducing Administrative Burdens of Researchers

Background

The Federal Demonstration Partnership (FDP) is "a cooperative initiative among 10 federal agencies and 119 institutional recipients of federal funds, sponsored by the National Academies, with a purpose of reducing the administrative burdens associated with federal research grants and contracts."[36] In 2005 and 2012, FDP conducted surveys of principal investigators of federally funded projects to determine the impact of federal regulations and requirements on the research process. In both surveys, researchers reported spending 57% of their time engaged in research and 42% of their time in completing pre- and post-award requirements. "The most commonly experienced administrative responsibilities included those related to federal project finances, personnel, and effort reporting. These were also among the most time-consuming responsibilities. For researchers engaged in projects that required human or animal subjects, the related Institutional Review Board (IRB) and Institutional Animal Care and Use Committee (IACUC) requirements were by far the most time-consuming. Other areas viewed as particularly time-consuming were those involving clinical trials, subcontracts, and cross-agency differences."[37]

Provision

The provision would require the NIH Director to implement measures to reduce the administrative burden of NIH-funded researchers, taking into account the recommendations of the NIH Scientific Management Review Board, the National Academy of Sciences, the Faculty Burden Survey conducted by the Federal Demonstration Partnership, and the Research Business Models Working Group. Not later than two years following enactment, the NIH Director would be required to submit a report to Congress on the measures that have been implemented to reduce the administrative burden of NIH-funded researchers.

Section 1024. Exemption for the National Institutes of Health from the Paperwork Reduction Act Requirements

Background

The Paperwork Reduction Act (PRA, 44 U.S.C. Chapter 35), enacted in 1980 and amended in 1995, established the Office of Information and Regulatory Affairs (OIRA) in the Office of Management and Budget (OMB). Congress required that agencies seek OIRA permission before collecting information from the public. The first of 11 stated purposes was to "minimize the paperwork burden for individuals ... and other persons resulting from the collection of

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Medicine to Become National Academy of Medicine," press release, April 28, 2015, http://www.iom.edu/Global/News%20Announcements/IOM-to-become-NAM-Press-Release.aspx.

[36] Sandra L. Schneider et al., *Federal Demonstration Partnership (FDP) 2012 Faculty Workload Survey: Executive Summary,* April 2014.

[37] http://sites.nationalacademies.org/cs/groups/pgasite/documents/webpage/pga_087823.pdf; http://sites.nationalacademies.org/PGA/fdp/PGA_055749.

information by and for the Federal Government."[38] The PRA requires that federal agencies receive clearance from OIRA before requesting most types of information from the public.[39] PRA clearance is required when standardized information is collected from 10 or more respondents within a 12-month period.[40] PRA does not apply to certain types of scientific research, including collections that are neither sponsored nor conducted by the agency and those that are subject to a clinical exception.[41]

Provision

The provision would amend 44 U.S.C. Chapter 35 to exempt NIH research from the requirements of the PRA.

Section 1025. NIH Travel

Background

Following allegations of misspent funds during a 2010 General Services Administration meeting held in Las Vegas, the Office of Management and Budget imposed restrictions on conference travel for federal employees in a May 11, 2012, memorandum.[42] The memorandum directed agencies, beginning in FY2013, to spend at least 30% less than what was spent in FY2010 on travel expenses, and stated that agencies "must maintain this reduced level of spending each year through FY 2016." Senior level agency approval is required for all conferences sponsored by an agency where the conference expenses to the agency are over $100,000. Agencies are prohibited from spending more than $500,000 on a single conference. However, this restriction may be waived if the agency head "determines that exceptional circumstances exist whereby spending in excess of $500,000 on a single conference is the most cost-effective option to achieve a compelling purpose."[43]

Provision

The provision would express the sense of Congress that "participation in or sponsorship of scientific conferences and meetings is essential to the mission of the National Institutes of Health."

[38] 44 U.S.C. §3501.

[39] For further information about the PRA, see CRS Report RL30590, *Paperwork Reduction Act Reauthorization and Government Information Management Issues*, and CRS Report RL32397, *Federal Rulemaking: The Role of the Office of Information and Regulatory Affairs*.

[40] See NIH, Office of Science Policy, Genetics, Health and Society, *What is the Paperwork Reduction Act?*, at http://osp.od.nih.gov/faq/what-paperwork-reduction-act; and HHS, *Frequently Asked Questions About PRA / Information Collection*, at http://www.hhs.gov/ocio/policy/collection/infocollectfaq.html.

[41] Cass R. Sunstein, *Facilitating Scientific Research by Streamlining the Paperwork Reduction Act Process*, Executive Office of the President, Office of Management and Budget, December 9, 2010, https://www.whitehouse.gov/sites/default/files/omb/memoranda/2011/m11-07.pdf.

[42] Promoting Efficient Spending to Support Agency Operations, http://www.whitehouse.gov/sites/default/files/omb/memoranda/2012/m-12-12.pdf.

[43] Ibid.

Section 1026. Other Transactions Authority

Background

Section 480 of the PHSA establishes the Cures Acceleration Network (CAN). The purpose of the CAN is to support revolutionary advances in basic research and facilitate FDA review of CAN-funded high-need cures. A high-need cure is a drug, biological product, or device that, as determined by the Director of the NIH National Center for Advancing Translational Sciences (NCATS), "is a priority to diagnose, mitigate, prevent, or treat harm from any disease or condition [and] for which the incentives of the commercial market are unlikely to result in its adequate or timely development."[44]

Under current law, if the Director of NCATS determines that the goals and objectives of this section cannot be adequately carried out through a contract, grant, or cooperative agreement, then the Director has "flexible research authority to use other transactions to fund projects in accordance with the terms and conditions of this section. Awards made under such flexible research authority for a fiscal year shall not exceed 20 percent of the total funds appropriated" for a fiscal year.[45] Other transaction (OT) authority is a special vehicle used by certain federal agencies for obtaining or advancing research and development (R&D).[46] Generally, OT authority is created because the government needs to obtain leading-edge R&D from commercial sources, but some companies (and other entities) are unwilling or unable to comply with the government's procurement regulations.

Current law stipulates that any "grant, cooperative agreement, or contract awarded under this section shall be awarded on a competitive basis."[47]

Provision

The provision would replace the current subparagraph on other transactions authority with a new subparagraph that would provide other transactions authority with fewer restrictions. The OT authority would not be conditional on a determination that the goals and objectives of this section cannot be adequately carried out through a contract, grant, or cooperative agreement. The provision would not limit OTs to 20% of the total funds appropriated.

The provision would also delete the requirement that grants, contracts, and cooperative agreements be awarded on a competitive basis.

Section 1027. NCATS Phase IIB Restriction

Background

Prior to FDA approval, medical products are tested in a clinical trial using human volunteers to see how the products compare to standard treatments or to no treatment. FDA uses the data from clinical trials to determine whether to approve a manufacturer's application for marketing a medical product. Clinical trials are conducted in phases, which are described by FDA in the

[44] PHS Act §480(a)(3).

[45] PHS Act §480(e)(3)(C).

[46] An OT is not a contract, grant, or cooperative agreement, and there is no statutory or regulatory definition of "other transaction." Only those agencies that have been provided OT authority may engage in other transactions. For further information, see CRS Report RL34760, *Other Transaction (OT) Authority*.

[47] PHS Act §480(f).

paragraphs below. Sometimes Phase II clinical trials are divided into Phase IIA (to assess dosing requirements) and Phase IIB (to study efficacy).

> **Phase I** trials try to determine dosing, document how a drug is metabolized and excreted, and identify acute side effects. Usually, a small number of healthy volunteers (between 20 and 80) are used in Phase I trials.
>
> **Phase II** trials include more participants (about 100-300) who have the disease or condition that the product potentially could treat. In Phase II trials, researchers seek to gather further safety data and preliminary evidence of the drug's beneficial effects (efficacy), and they develop and refine research methods for future trials with this drug. If the Phase II trials indicate that the drug may be effective—and the risks are considered acceptable, given the observed efficacy and the severity of the disease—the drug moves to Phase III.
>
> In **Phase III** trials, the drug is studied in a larger number of participants with the disease (approximately 1,000-3,000). This phase further tests the product's effectiveness, monitors side effects and, in some cases, compares the product's effects to a standard treatment, if one is already available. As more and more participants are tested over longer periods of time, the less common side effects are more likely to be revealed.[48]

Under current law, although NCATS may develop and provide infrastructure and resources for all phases of clinical trials research, it may support clinical trial activities only through the end of Phase IIA, with one exception. NCATS may support clinical trial activities through the end of Phase IIB for a treatment for a rare disease or condition if (1) it gives public notice for a period of at least 120 days of NCATS intention to support the clinical trial activities in Phase IIB; (2) no public or private organization provides credible written intent to NCATS that the organization has timely plans to further the clinical trial activities or conduct clinical trials of a similar nature beyond Phase IIA; and (3) NCATS ensures that support of the clinical trial activities in Phase IIB will not increase the federal government's liability beyond the award value of the center's support.

Provision

The provision would extend NCATS's authority to support clinical trial activities through the end of Phase IIB (instead of Phase IIA), and extend the exception for treatment of a rare disease or condition through the end of Phase III (instead of Phase IIB).

Section 1028. High-Risk, High-Reward Research

Background

The NIH Common Fund, within the Office of the NIH Director, supports research in emerging areas of scientific opportunity, public health challenges, and knowledge gaps. These are often large, complex research efforts that involve the collaboration of two or more research institutes or centers. The Common Fund also supports the High-Risk, High-Reward Research Program, which has "four unique funding opportunities for exceptionally creative scientists who propose highly innovative approaches to major challenges in biomedical research."[49] These awards are intended "to encourage creative, outside-the-box thinkers to pursue exciting and innovative ideas about biomedical research." The four funding opportunities are (1) the NIH Director's Pioneer Award,

[48] FDA, Inside Clinical Trials: Testing Medical Products in People, What Happens in a Clinical Trial?, at http://www.fda.gov/Drugs/ResourcesForYou/Consumers/ucm143531.htm.

[49] NIH, Office of Strategic Coordination, The Common Fund, High-Risk Research, at https://commonfund.nih.gov/highrisk/index.

(2) the New Innovator Award, (3) the Transformative Research Award, and (4) the NIH Director's Early Independence Award. This last award was created in FY2011 "to support exceptional early career scientists who possess the intellect, scientific creativity, drive, and maturity to flourish independently immediately following their graduate training, eliminating the need for traditional post-doctoral training."[50] NIH announced 78 High-Risk, High-Reward awards in FY2013.[51] A total of 85 such awards were made in FY2014.[52]

Provision

The provision would add a new Section 409K to the PHSA that would require the Director of each NIH institute to "establish programs to conduct or support research projects that pursue innovative approaches to major contemporary challenges in biomedical research that involve inherent high risk, but have the potential to lead to breakthroughs." The NIH Director would determine a specific percentage of funding for each institute for such projects.

Section 1029. Sense of Congress on Increased Inclusion of Underrepresented Communities in Clinical Trials

Background

Minorities have been underrepresented in clinical trials. For example, according to a 2011 report from an FDA-sponsored conference, "African Americans represent 12% of the U.S. population but only 5% of clinical trial participants and Hispanics make up 16% of the population but only 1% of clinical trial participants."[53] There can be biological differences in how people process or respond to medical products. For example, genetic differences can make a treatment less effective or perhaps even more toxic in one particular ethnic group. Therefore, it is important to study in clinical trials the safety and effectiveness of medical products in all people who will use the products following FDA approval.

Provision

The provision would express the sense of Congress that the NIH National Institute on Minority Health and Health Disparities "should include within its strategic plan ways to increase representation of underrepresented communities in clinical trials."

[50] NIH, Office of Strategic Coordination, The Common Fund, High-Risk Research, at http://commonfund.nih.gov/highrisk/overview.

[51] NIH, News Releases, at http://www.nih.gov/news/health/sep2013/od-30.htm.

[52] NIH, News Releases, at http://www.nih.gov/news/health/oct2014/od-06.htm.

[53] FDA, For Consumers, Clinical Trials Shed Light on Minority Health, at http://www.fda.gov/ForConsumers/ConsumerUpdates/ucm349063.htm.

Subtitle C—Supporting Young Emerging Scientists

Section 1041. Improvement of Loan Repayment Programs of National Institutes of Health

Background

NIH funds seven loan repayment programs for researchers.[54] Three of these are intramural programs that provide loan repayment to researchers in exchange for undertaking research while employed by NIH. Intramural loan repayment programs support researchers from disadvantaged backgrounds, those who are investigating AIDS, and those undertaking general research (including general research by physicians during their fellowship training). NIH also funds four programs to repay the loans of extramural researchers. These funds are awarded competitively to researchers who are employed by a qualifying educational institution. Specific programs are available to extramural researchers investigating health disparities, undertaking contraception and infertility research, engaging in clinical research, and examining pediatric-related topics. Researchers may receive up to $35,000 per year in loan repayment under each of these programs, and the NIH loan repayment follows (where not inconsistent with the specific program) the regulations that govern the National Health Service Corps Loan Repayment Program[55] with regard to participant eligibility, application procedures, selection criteria, loan repayment contract terms, tax liability for payments received, service obligation, and penalties for breach of contract.

Provision

The provision would authorize a new NIH loan repayment program by adding a new PHSA Section 487H "Loan Repayment Program." The new section would require the Secretary to establish a new extramural loan repayment program for the NIH, based on the agency's scientific and workforce needs, under which the federal government would pay not more than $50,000 per year on the principal and educational loans of health professionals who engage in research. Beginning in FY2017, the provision would allow amounts repaid under this new program to be adjusted annually for inflation. Individuals eligible for loan repayment must have a substantial amount of educational loans relative to income and must complete at least two years of research service. The provision would also require that the new program, except where inconsistent with the program's purpose, be subject to the regulations that govern the National Health Service Corps Loan Repayment Program[56] with regard to participant eligibility, application procedures, selection criteria, loan repayment contract terms, tax liability for payments received, service obligation, and penalties for breach of contract. The provision would also allow amounts appropriated for new loan repayment contracts to remain available until the end of the second fiscal year after they are appropriated.

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[54] For description of these programs, see Appendix A of CRS Report R43571, *Federal Student Loan Forgiveness and Loan Repayment Programs*.

[55] For program description, see CRS Report R43920, *National Health Service Corps: Changes in Funding and Impact on Recruitment*. For program regulations, see U.S. Department of Health and Human Services, Health Resources and Services Administration, "National Health Service Corps: Loan repayment Program," http://nhsc.hrsa.gov/downloads/lrpapplicationguidance.pdf.

[56] Ibid.

Finally, the provision would amend the existing NIH loan repayment programs by increasing annual loan repayment limits from $35,000 to $50,000 and by permitting an annual adjustment of loan repayment amounts for inflation, beginning in FY2017.

Section 1042. Report

Provision

The provision would require the NIH Director to submit to Congress a report, not later than 18 months following enactment, on NIH efforts "to attract, retain and develop emerging scientists, including underrepresented individuals in the sciences, such as women and other minorities."[57]

Subtitle D—Capstone Grant Program

Section 1061. Capstone Award

Background

In February 2015, the NIH Deputy Director for Extramural Research, Sally Rockey, posted a description of a new NIH emeritus award that would "help senior investigators who wish to transition out of a position that relies on funding from NIH research grants, and facilitate the transfer of their work, knowledge and resources to junior colleagues."[58] According to *Science,* "most of the more than 120 comments" responding to Sally Rockey's blog were critical of the emeritus award.[59] At the same time, NIH also published a formal request for public comment—also known as a Request for Information (RFI)—on the emeritus award.[60] Jeremy Berg, former director of the NIH National Institute of General Medical Sciences stated that he is "skeptical that it would have the desired impact," that it may become "an entitlement for senior investigators," and that "[t]here is absolutely no need to create a new mechanism."[61]

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[57] Amendment 4 (Castro), agreed to by voice vote during floor debate on H.R. 6, added the language ensuring that underrepresented individuals in the sciences (women and minorities) would be included as a focus topic in the NIH report to Congress.

[58] Sally Rockey, *Rock Talk*, "Seeking Your Input on Sustaining the Workforce Through an Emeritus Award," February 3, 2015, at http://nexus.od.nih.gov/all/2015/02/03/emeritus-rfi/. The Federation of American Societies for Experimental Biology (FASEB) states that the award "reflects an idea initially suggested during a meeting of the NIH Advisory Committee to the Director." Yvette Seger, "NIH requests feedback on potential emeritus award," *The Washington Update*, February 11, 2015, http://washingtonupdate.faseb.org/?p=1225. The transition award idea is also a recommendation in a January 2015 FASEB report, *Sustaining Discovery in Biological and Medical Sciences: A Framework for Discussion*. See recommendation 2.10 on page 64 of the FASEB report, *Sustaining Discovery in Biological and Medical Sciences: A Framework for Discussion*, at http://www.faseb.org/pdfviewer.aspx?loadthis=http%3A%2F%2Fwww.faseb.org%2FPortals%2F2%2FPDFs%2Fopa%2F2015%2FSustaining%2520Discovery%2520Report%2520Final.pdf.

[59] Jocelyn Kaiser, NIH proposal to create grant for aging scientists hits a nerve, *ScienceInsider*, February 6, 2015, at http://news.sciencemag.org/funding/2015/02/nih-proposal-create-grant-aging-scientists-hits-nerve.

[60] http://grants.nih.gov/grants/guide/notice-files/NOT-OD-15-064.html; NIH uses RFIs as a mechanism to gather community feedback on proposed ideas; the draft concepts described in RFIs are subject to change as a result of public input and internal NIH discussions.

[61] Kaiser, "NIH proposal to create grant for aging scientists hits a nerve," *ScienceInsider*.

Provision

The provision would add a new Section 490 to the PHSA creating a capstone award to support outstanding scientists who have received NIH funding. The purpose of the award would be to "facilitate the successful transition or conclusion of research programs." The duration and amount of each award would be determined by the NIH Director in consultation with the IC Directors. Individuals who have received a capstone award would not be eligible to be the principal investigator on subsequent NIH awards.

Subtitle E—Promoting Pediatric Research Through the National Institutes of Health

Background

Section 409D(d) of the PHSA authorized in 2013 the establishment within NIH of a Pediatric Research Network "in order to more effectively support pediatric research and optimize the use of Federal resources."[62]

Section 1081. National Pediatric Research Network

Provision

The provision would require the establishment of the National Pediatric Research Network (NPRN). In establishing the NPRN, the provision would (1) eliminate language telling the NIH Director to consult with the Director of the Eunice Kennedy Shriver National Institute of Child Health and Human Development but (2) retain language telling the NIH Director to collaborate with the ICs that carry out pediatric research. The provision would allow that the NPRN "may be comprised of, as appropriate, the pediatric research consortia" that are receiving grants under this section of the PHSA, and deletes that the NPRN may be comprised of other consortia, centers or networks focused on pediatric research. The provision would now require the NIH Director to award funding to support the pediatric research consortia; the duration of such support "shall be for a period not to exceed 5 years." Each consortium receiving an award under this section of the PHSA would be required to "provide assistance to [CDC] for activities related to patient registries and other surveillance systems."

Section 1082. Global Pediatric Clinical Study Network Sense of Congress

Provision

The provision would express the sense of Congress that NIH "should encourage a global pediatric clinical study network through the allocation of grants, contracts, or cooperative agreements to supplement the salaries of new and early investigators who participate in the global pediatric clinical study network."

[62] Section 409D(d) was added to the PHS Act by P.L. 113-55, the Prematurity Research Expansion and Education for Mothers who deliver Infants Early Reauthorization Act, or the PREEMIE Reauthorization Act, which was signed into law on November 27, 2013.

The provision would express the sense of Congress that NIH "grants, contracts, or cooperative agreements should be awarded, solely for the purpose of supplementing the salaries of new and early investigators, to entities that participate in the global pediatric clinical study network."

The provision would express the sense of Congress that FDA "should engage the European Medicines Agency and other foreign regulatory entities during the formation of the global pediatric clinical study network to encourage their participation."

The provision would express the sense of Congress that "once a global pediatric clinical study network is established and becomes operational, [FDA] should continue to engage the European Medicines Agency and other foreign regulatory entities to encourage and facilitate their participation in the network with the goal of enhancing the global reach of the network."

Section 1083. Appropriate Age Groupings in Clinical Research

Provision

The provision would require the NIH Director, within 180 days of enactment, to convene a workshop of experts on pediatrics and geriatrics and then publish guidelines regarding "appropriate age groupings to be included in research studies." The Director would also be required to make available to the public, within 180 days after the end of the workshop, the findings and conclusions of the workshop. At least every other year, the Director would be required to disclose to the public the number of children included in NIH-supported research, "disaggregated by developmentally appropriate age group, race, and gender."

Subtitle F—Advancement of National Institutes of Health Research and Data Access

Section 1101. Standardization of Data in Clinical Trial Registry Data Bank on Eligibility for Clinical Trials

Background

Sponsors of clinical trials for drugs, biologics, and devices regulated by the FDA are required to submit registration and summary results information to ClinicalTrials.gov, the clinical trial registry and results data bank operated by NIH's National Library of Medicine (NLM) pursuant to Sections 402(i)-(j) of the PHS Act. Subparagraph 402(j)(2)(B) requires the NIH Director to ensure that the public may, in addition to key-word searching, search the entries in the data bank by various specified criteria, including the disease or condition being studied, the name of the drug or device under investigation, and the location of the clinical trial. The NIH Director is instructed to add search categories as deemed necessary and to ensure that the data bank is easy to use, and that its entries are easily compared.

Provision

The provision would add new language to Section 402(j) of the PHS Act ("Expanded Clinical Trial Registry Data Bank") requiring the NIH Director to ensure that (1) the registry and results data bank is easily used by the public; (2) the registry and results data bank entries are easily compared; (3) information is submitted to the registry and results data bank in a standardized format, including certain specified data; and (4) standard terminologies and code sets are used, to

the extent possible, to facilitate electronic data matching. The provision would strike subparagraph 402(j)(2)(B).

Within 90 days of enactment, the Secretary would be required to seek the advice of relevant stakeholders and experts on enhancements to the clinical trial registry data bank that are necessary to implement the provision. The Secretary would have to begin implementation of the provision within 18 months of enactment.

Subtitle G—Facilitating Collaborative Research

Section 1121. Clinical Trial Data System

Background

Sponsors of clinical trials for drugs, biologics, and devices regulated by the FDA are required to submit registration and summary results information to ClinicalTrials.gov, the clinical trial registry and results data bank operated by NIH's National Library of Medicine (NLM). Under Section 402(j) of the PHS Act, those responsible for specified clinical trials of FDA-regulated products have been required to submit registration information to ClinicalTrials.gov since December 2007, submit summary results information for clinical trials of approved products since September 2008, and submit adverse events information since September 2009. The Secretary is required, by rulemaking, to expand the requirements for submission of summary results information, and authorized to use rulemaking to make other changes in the requirements for submission of registration and results information. In November 2014, HHS published a proposed rule to clarify and expand requirements for the submission of clinical trial registration and results information to ClinicalTrials.gov.[63]

Provision

The provision would instruct the Secretary to enter into a seven-year cooperative agreement, contract, or grant—the Clinical Trial Data System Agreement—with one or more eligible entities (i.e., tax-exempt academic institutions) to implement a pilot program to enable registered users to conduct further research on reported clinical trial data. Eligible entities seeking funding would have to submit an application that contains certain specified information including, among other things, (1) information demonstrating that the eligible entity can compile clinical trial data in standardized formats; (2) a description of the system the eligible entity will use to store and maintain such data; (3) a certification that the eligible entity will allow only registered users to access and use de-identified clinical trial data; (4) evidence demonstrating the ability of the eligible entity to ensure that registered users disseminate the results of their research; and (5) evidence demonstrating that the eligible entity has a proven track record of protecting confidential data.

Within six years of establishing the pilot program, the Comptroller General would have to study and report to the Secretary and Congress on the impact and effectiveness of the program, including recommendations for improving it. Among other things, the report would have to include information on new discoveries, research inquiries, or clinical trials that resulted from having access to clinical trial data under the pilot program, as well as an analysis of whether the program had helped reduce adverse events in clinical trials. The Secretary would be able to

[63] Department of Health and Human Services, National Institutes of Health, "Clinical Trials Registration and Results Submission," 79 *Federal Register* 69566, November 21, 2014.

extend (including permanently), expand, or terminate the pilot program, in whole or in part, after the seven-year period expires.

Section 1122. National Neurological Diseases Surveillance System

Background

The PHSA does not explicitly authorize or require surveillance of neurological diseases in general, although the Secretary may conduct such activities under general authorities in PHSA Title III. Surveillance is explicitly authorized for certain specified neurological disorders (e.g., amyotrophic lateral sclerosis[64] and autism spectrum disorder).[65]

Provision

The provision would add a new PHSA Section 399V-6, "Surveillance of Neurological Diseases." It would require the Secretary, acting through the Director of the Centers for Disease Control and Prevention (CDC)—in consultation with specified stakeholders, and coordinated with other agencies—to establish a National Neurological Diseases Surveillance System, to include surveillance of multiple sclerosis and Parkinson's disease. Required system elements would include demographic information and risk factors associated or possibly associated with neurological diseases, and information about diagnosis and progression markers.[66] Optional system elements would include information about the epidemiology, natural history, prevention, detection, management, and treatment approaches for the diseases; the development of outcomes measures; and any additional matters identified by stakeholders.

The provision also would authorize the Secretary to furnish grants, contracts, or cooperative agreements with public or private nonprofit entities to implement this provision. The Secretary would be required to make information and analysis obtained from the system available to other federal health agencies (as listed) and state and local agencies, and, subject to HIPAA privacy and security protections, to the public, including researchers. The Secretary would be required to report to Congress regarding the system within four years of enactment. The provision would authorize the appropriation of $5 million for each of fiscal years FY2016 through FY2020.

Section 1123. Data on Natural History of Diseases

Background

The natural history of a disease is its course over time from inception to its eventual end in full recovery or death. Natural history encompasses exposure or another inciting event, onset and types of symptoms, and any resulting disability, among other things.

Provision

The provision would express the sense of Congress, in subsection (a), that "studies on the natural history of diseases can help facilitate and expedite the development of medical products for such

[64] PHSA Section 399S; 42 U.S.C. §280g-7.

[65] PHSA Section 399AA; 42 U.S.C. §280i.

[66] A *disease marker* is a substance or other measurable parameter that can be used to identify the presence or severity of a health condition. A *progression marker* is one that could indicate worsening or improvement in the condition over time.

diseases." Subsection (b) would establish a new PHSA Section 229A. It would authorize the Secretary to engage in public-private partnerships and award grants in order to gather, analyze, and interpret data on the natural history of diseases, with a focus on rare diseases. It also would authorize the Secretary, through these public-private partnerships, to support disease registries and a secure, flexible information management system, and to provide advice to researchers, advocacy groups, and others on matters regarding disease studies.

The new PHSA Section 229A also would require the Secretary to make data obtained or maintained pursuant to this section available to the public (including patient advocacy groups, researchers, and drug developers), consistent with federal and state privacy laws, in order to facilitate medical product development. The Secretary would be required to follow applicable laws protecting privileged or confidential trade secret, commercial, or financial information. Finally, the provision would authorize the appropriation of $5 million for each of fiscal years FY2016 through FY2020 to implement this section.

Section 1124. Accessing, Sharing, and Using Health Data for Research Purposes

Background

The Health Information Portability and Accountability Act (HIPAA) privacy rule describes the circumstances under which HIPAA-covered entities such as health plans and health care providers are permitted to use or disclose individually identifiable health information (i.e., protected health information, or PHI) without an individual's written authorization.[67] In general, covered entities may use or disclose PHI for the purposes of treatment, payment, and other routine health care operations with few restrictions.[68] Covered entities also may disclose PHI for certain public health purposes, including disclosing information about an FDA-regulated product or activity to an individual subject to FDA's jurisdiction.[69] However, the privacy rule's definition of health care operations excludes using or disclosing PHI for the primary purpose of conducting research (i.e., systematic investigation designed to develop or contribute to generalizable knowledge).[70]

The disclosure of PHI to researchers generally requires an individual's authorization unless an Institutional Review Board (or equivalent Privacy Board) waives the authorization.[71] A covered entity may, however, allow researchers access to PHI to prepare a research protocol, provided the PHI is not removed from the covered entity. The privacy rule traditionally has required authorizations to be study-specific; authorizations for future research were prohibited. In a January 2013 final rule, HHS permitted authorizations for future research if a sufficiently clear description of the future research is provided.[72] While covered entities may not sell PHI to

[67] The HIPAA privacy rule is codified at 45 C.F.R. Part 164, Subpart E.

[68] 45 C.F.R. §164.506.

[69] 45 C.F.R. §164.512(b)(1)(iii).

[70] 45 C.F.R. §164.501.

[71] 45 C.F.R. §164.512(i)(1)(i).

[72] Department of Health and Human Services, Office of the Secretary, "Modifications to the HIPAA Privacy, Security, Enforcement, and Breach Notification Rules Under the Health Information Technology for Economic and Clinical Health Act and the Genetic Information Nondiscrimination Act; Other Modifications to the HIPAA Rules; Final Rule," 78 *Federal Register* 5566, 5611, January 25, 2013.

researchers, they are permitted to charge researchers a cost-based fee to cover the preparation and transmission of the information.[73]

Provision

This provision would add a new Part 4 to Subtitle D of the HITECH Act instructing the Secretary to make several revisions or clarifications to the HIPAA privacy rule within 12 months of enactment. These changes are intended to ease some of the rule's restrictions on accessing, sharing, and using health information for research purposes.

First, the Secretary would be required to revise or clarify the privacy rule to allow the use or disclosure of PHI by a covered entity for research purposes to be treated as health care operations. However, such disclosures would be treated as health care operations only if they were made to another covered entity (or to a business associate under contract with the disclosing covered entity) to perform health care operations, or to a business associate under contract to perform data aggregation.

Second, the Secretary would be required to revise or clarify the privacy rule to permit the sale of PHI for research purposes by removing the provision that limits the remuneration to an amount that covers the cost of preparing and transmitting the information. The Secretary would be required to further amend the rule so that research on the quality, safety, or effectiveness of FDA-regulated products is treated as a public health activity for the purpose of disclosing PHI to an individual subject to FDA's jurisdiction.

Third, the Secretary would be required to revise or clarify the privacy rule's provision that prohibits researchers from removing PHI during preparation of a research protocol to permit remote access to PHI by researchers, provided appropriate security and privacy safeguards are in place and the PHI is not copied or retained by the researchers.

Finally, the Secretary would be required to revise or clarify the privacy rule to allow an authorization for the use or disclosure of PHI for future research purposes, provided the authorization (1) sufficiently describes the purposes such that it would be reasonable for an individual to expect that the PHI could be used or disclosed for future research; (2) states that the authorization will expire on a particular date or on the occurrence of a particular event; and (3) states that the authorization will remain valid unless revoked, and provides revocation instructions.

Subtitle H—Council for 21st Century Cures

Section 1141. Council for 21st Century Cures

Provision

The provision would add to Title II of the PHSA a new Part E, "Council for 21st Century Cures." The council would be a non-profit public-private partnership. The purpose of the council would be to "accelerate the discovery, development, and delivery in the United States of innovative cures, treatments, and preventive measures for patients." To accomplish its purpose, the council would foster collaboration and coordination of those "engaged in the cycle of discovery, development and delivery of life-saving and health-enhancing innovative interventions." Other duties of the council would include communication and dissemination activities; establishing a

[73] 45 C.F.R. §164.502(a)(5)(ii)(B)(*2*).

strategic agenda to accelerate the discovery, development, and delivery of cures, treatments, and preventive interventions; developing recommendations based on the identification of gaps and opportunities within the discovery, development, and delivery cycle; and identifying opportunities to work with other entities within the United States as well as internationally, such as the Innovative Medicines Initiative of the European Union.

The council would have a Board of Directors composed of eight ex officio members and 17 appointed members. The ex officio members would consist of the NIH Director, the FDA Commissioner, the CMS Administrator, and "the heads of five other federal agencies deemed by the Secretary to be engaged in biomedical research and development." Within six months of enactment, the Comptroller General would select the appointed board members. From a list of nominations made by the leading trade associations, the Comptroller General would select four representatives of the biopharmaceutical industry, two from the medical device industry, and two from the information and digital technology industry. In addition, the Comptroller General would select two academic researchers, three patient representatives, two representatives of health care providers, and two representatives of health care plans and insurers. The term of appointed members would be five years. Within 90 days of incorporation of the council and board appointment, the members of the board would select a Chair, establish the by-laws and policies for the council, and issue an agenda outlining how it will achieve its purpose. This agenda would be reviewed and updated annually. The board would be required to meet quarterly, and its minutes would be publically available and submitted to Congress. The day-to-day management of the council would be the responsibility of the Executive Director, whose specific duties would be established by the Board of Directors.

The council would be required to terminate on September 30, 2023. For each fiscal year, FY2016 through FY2023, the provision would authorize appropriations of $10 million to the council. The council would also be able to "accept financial or in-kind support from participating entities or private foundations or organizations when such support is deemed appropriate."

Title II—Development

Subtitle A—Patient-Focused Drug Development

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

Background

FFDCA Section 505(d), in its instructions on new drug applications, requires the Secretary to

> implement a structured risk-benefit assessment framework in the new drug approval process to facilitate the balanced consideration of benefits and risks, a consistent and systematic approach to the discussion and regulatory decision making, and the communication of the benefits and risks of new drugs. Nothing in the preceding sentence shall alter the criteria for evaluating an application for premarket approval of a drug.

Provision

The provision would amend FFDCA Section 505 by deleting a clause from Section 505(d) and adding new subsections (x) and (y). The new 505(x) would restate the deleted 505(d) requirement for the Secretary to "implement a structured risk-benefit assessment framework in the new drug

approval process." The new 505(y) would require the Secretary to "establish and implement processes under which" entities "seeking to develop patient experience data" could submit ideas and data to the Secretary and the Secretary could request materials from those entities, which could include the manufacturer and nonmanufacturer groups. This provision would define "patient experience data" as

> data collected by patients, parents, caregivers, patient advocacy organizations, disease research foundations, medical researchers, research sponsors, or other parties determined appropriate by the Secretary that is intended to facilitate or enhance the Secretary's risk-benefit assessments, including information about the impact of a disease or a therapy on patients' lives.

The new subsection would also require the Secretary to issue implementation guidance after holding several methodological workshops and a public meeting.

Subtitle B—Qualification and Use of Drug Development Tools

Section 2021. Qualification of Drug Development Tools

Background

The pharmaceutical industry claims the cost of drug discovery and development is high, estimated at $1.3 billion to $1.6 billion to bring a drug to market for use in humans.[74] Others have criticized these estimates, claiming they are "false and built on seriously flawed methods" and that the "true cost is likely to be below $100 million."[75] Lengthy clinical trials have been blamed as one factor contributing to the high cost of drug development.

Surrogate endpoints—based on the measurement of biomarkers—may be used to determine the clinical benefit of a product instead of using clinical endpoints. This is because surrogates "enable smaller, faster, and thus cheaper clinical trials. In addition, pharmaceutical companies argue that using surrogates means that fewer patients are exposed during testing, and beneficial new medications reach the market faster. Their main disadvantage is that favorable effects on surrogates do not automatically translate into benefits to health."[76] For example, Avastin "delayed tumor progression in advanced breast cancer but was not shown to benefit patients."[77] Likewise Avandia lowered a biomarker level in patients with diabetes but also increased their risk of heart attack. A number of drugs have been approved on the basis of surrogate endpoint data and, after

[74] Stephen Whitehead, "Making medicines evergreen," (rapid response), BMJ, December 17, 2012.

[75] Peter C. Gotzsche, "Making medicines evergreen," (rapid response), *BMJ*, December 17, 2012. See also: Arnold S. Relman and Marcia Angell, "America's other drug problem: how the drug industry distorts medicine and politics," *The New Republic*, December 16, 2002, pp. 27-41; Marcia Angell, "How much does the pharmaceutical industry really spend on R&D?," in The truth about the drug companies: How they deceive us and what to do about it (New York: Random House, 2004), pp. 37-41; Merrill Goozner, The $800 million pill: The truth behind the cost of new drugs (Berkeley: University of California Press, 2005); and, Donald W. Light, "Misleading Congress about Drug Development," *Journal of Health Politics, Policy and Law*, vol. 32, no. 5 (October 2007), pp. 895-913. One recent estimate states that the "median costs were a third less than the average, or $60 million. Deconstructing other inflators would lower the estimate of costs even further." Donald W. Light and Joel R. Lexchin, "Pharmaceutical research and development: what do we get for all that money?," *BMJ*, August 7, 2012.

[76] Staffan Svensson, David B. Menkes, and Joel Lexchin, "Surrogate Outcomes in Clinical Trials—A Cautionary Tale," *JAMA Internal Medicine*, vol. 173, no. 8 (April 22, 2013), pp. 611-612.

[77] Jerry Avorn and Aaron S. Kesselheim, "The 21st Century Cures Act—Will It Take Us Back in Time?," *The New England Journal of Medicine*, June 3, 2015, http://www.nejm.org/doi/full/10.1056/NEJMp1506964.

adoption into medical practice, have been shown to be harmful through clinical trials or other subsequent analysis.[78] The FDA uses surrogate endpoints in about half of new drug approvals.[79]

The Institute of Medicine defines a clinical endpoint as "a characteristic or variable that reflects how a patient [or consumer] feels, functions, or survives. Death is one example of a clinical endpoint."[80] IOM defines "surrogate endpoint" in the following way:

> a biomarker that is intended to substitute for a clinical endpoint. A surrogate endpoint is expected to predict clinical benefit (or harm or lack of benefit or harm) based on epidemiologic, therapeutic, pathophysiologic, or other scientific evidence. For example, blood pressure has served as a surrogate endpoint for morbidity and mortality due to cardiovascular disease in trials of several classes of antihypertensive drugs. A surrogate endpoint represents a special use of a biomarker, in which the biomarker substitutes for a clinical endpoint.[81]

The Food and Drug Administration Safety and Innovation Act (FDASIA, P.L. 112-144) amended FFDCA Section 506 by adding the following: "The Secretary shall ... establish a program to encourage the development of surrogate and clinical endpoints, including biomarkers, and other scientific methods and tools that can assist the Secretary in determining whether the evidence submitted in an application is reasonably likely to predict clinical benefit for serious or life-threatening conditions for which significant unmet medical needs exist."[82]

Provision

The provision would add a new FFDCA Section 507, "Qualification of Drug Development Tools," which would require the Secretary to establish a process for the qualification of drug development tools. A drug development tool would be defined to include (1) a biomarker; (2) a clinical outcome assessment; and (3) any other method, material, or measure that the Secretary determines aids drug development and regulatory review.

Under new FFDCA Section 507, the Secretary would be allowed to accept a qualification submission based on factors that include its scientific merit or the available resources of the FDA to review the submission, and the Secretary would be allowed to prioritize review of a qualification submission based on factors including, for example, the severity, rarity, or prevalence of the disease being targeted or the availability or lack of an alternative treatment. The Secretary would be allowed, through grants or other specified mechanisms, to consult with biomedical research consortia and may consider the consortia's recommendations in review of the qualification submission. "Biomedical research consortia" would be defined as collaborative groups that may take the form of public-private partnerships and may include, among others, government agencies, institutions of higher education, patient advocacy groups, industry representatives, clinical and scientific experts, and other relevant individuals.[83] The Secretary

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[78] Staffan Svensson, David B. Menkes, and Joel Lexchin, "Surrogate Outcomes in Clinical Trials—A Cautionary Tale," *JAMA Internal Medicine*, vol. 173, no. 8 (April 22, 2013), Supplementary Online eTable.

[79] Jerry Avorn and Aaron S. Kesselheim, "The 21st Century Cures Act—Will It Take Us Back in Time?," *The New England Journal of Medicine*, June 3, 2015, http://www.nejm.org/doi/full/10.1056/NEJMp1506964.

[80] IOM, *Perspectives on Biomarker and Surrogate Endpoint Evaluation: Discussion Forum Summary*, January 18, 2011, p.6.

[81] Ibid.

[82] FFDCA §506(d)(2).

[83] The provision includes a finding that these consortia can play a valuable role in helping develop and qualify drug development tools, and a sense of Congress stating that an entity seeking to qualify a drug development tool should be encouraged to consult with these consortia.

would be required to carry out a full review of the qualification package and to determine if the drug development tool at issue is qualified for its proposed context of use.

A qualified drug development tool would be allowed to be used to obtain approval or licensure of a drug or biologic or to support the product's investigational use. The Secretary would be allowed to rescind or modify the granted qualification if she determines the drug development tool is not appropriate for the proposed context, and, if the Secretary does this, the requestor would be granted a meeting with the Secretary to discuss the basis of the decision.

New FFDCA Section 507 would require the Secretary to make public, and update at least biannually, certain information, including, for example, information about the qualification submissions, the Secretary's determinations in response to the submissions, and any subsequent modifications to the Secretary's determinations. It also specifies that nothing in this section would be construed to allow the Secretary to release any information contained in an application for approval or licensure of a drug or biologic that is confidential commercial or trade secret information; in addition, nothing in the section would be allowed to be construed as altering the standards of evidence for approval or licensure of a drug or biologic or to limit the Secretary's authority to approve or license such products.

New FFDCA Section 507 would authorize to be appropriated $10 million for each of fiscal years FY2016 through FY2020.

The provision would also require the Secretary, not later than 24 months after enactment, to publish draft guidance to implement new FFDCA Section 507, in consultation with the biomedical research consortia and other interested parties through a collaborative public process. The guidance would be required to, for example, make recommendations for demonstrating that a surrogate endpoint is reasonably likely to predict clinical benefit for the purpose of supporting accelerated approval of a drug. The Secretary would be required to issue final guidance not later than six months after the comment period for the draft guidance closes. In order to inform the guidance, the Secretary would be required, in consultation with the biomedical research consortia, to develop a taxonomy for the classification of biomarkers for use in drug development. The Secretary would be required to make this publicly available not later than 12 months after enactment and finalize the taxonomy not later than 12 months after the public comment period closes.

The provision would require the Secretary, not later than 12 months after enactment, to convene a public meeting regarding the qualification process under new FFDCA Section 507. The Secretary would also be required to publish a report on FDA's website, not later than five years after enactment, to include information, as specified.

Funds from the Cures Innovation Fund, as would be established by Section 4041 of this Act, would be allowed to be made available to be used to carry out the activities in this provision and amendments made by this provision.

Section 2022. Accelerated Approval Development Plan

Background

For drugs that address unmet needs or serious or life-threatening conditions, have the potential to offer better outcomes or fewer side effects, or meet other criteria associated with improved public

health, FDA uses several formal mechanisms to expedite development or review processes.[84] These include "priority review," "breakthrough therapy," and "fast track" designations, and the accelerated approval pathway.[85] FFDCA Section 506(c) authorizes accelerated approval for a product to treat a serious or life-threatening disease or condition; this pathway allows the Secretary to approve an application based on the product's effect on a "surrogate endpoint that is reasonably likely to predict clinical benefit" or on a clinical endpoint meeting specified criteria. The Institute of Medicine defined "surrogate endpoint" as

> a biomarker that is intended to substitute for a clinical endpoint. A surrogate endpoint is expected to predict clinical benefit (or harm or lack of benefit or harm) based on epidemiologic, therapeutic, pathophysiologic, or other scientific evidence. For example, blood pressure has served as a surrogate endpoint for morbidity and mortality due to cardiovascular disease in trials of several classes of antihypertensive drugs. A surrogate endpoint represents a special use of a biomarker, in which the biomarker substitutes for a clinical endpoint.[86]

Provision

The provision would add to FFDCA Section 506 a new subsection (g), which would allow the sponsor of a drug or biological product to request that the Secretary agree to an accelerated approval development plan if an application for investigation of the product has been submitted under FFDCA Section 505(i) or PHSA Section 351(a)(3) and the Secretary determines that the product may be eligible for accelerated approval under FFDCA 506(c). An accelerated approval development plan would be defined as a plan agreed on by the Secretary and the sponsor that contains study parameters for the use of a surrogate endpoint that is reasonably likely to predict clinical benefit and is intended to be the basis of the accelerated approval of a product under FFDCA 506(c).

An accelerated approval development plan would include, among other things, agreement on the surrogate endpoint to be assessed and the design of the study that would utilize the surrogate endpoint. The provision would authorize the Secretary to require the product sponsor to modify or terminate the plan if data indicate that the plan is no longer sufficient to demonstrate the safety of the drug involved or the drug is no longer eligible for accelerated approval; in this case, the sponsor would be granted a request for a meeting to discuss the basis of the Secretary's decision.

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[84] FDA, "DRAFT Guidance for Industry: Expedited Programs for Serious Conditions—Drugs and Biologics," Center for Drug Evaluation and Research and Center for Biologics Evaluation and Research, June 2013, http://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM358301.pdf; and FDA, "Review Designation Policy: Priority (P) and Standard (S)," MAPP 6020.3 Rev. 2, Manual of Policies and Procedures, Center for Drug Evaluation and Research, Office of New Drugs, June 25, 2013, http://www.fda.gov/downloads/AboutFDA/CentersOffices/OfficeofMedicalProductsandTobacco/CDER/ManualofPoliciesProcedures/UCM082000.pdf.

[85] See FDA, "DRAFT Guidance for Industry: Expedited Programs for Serious Conditions—Drugs and Biologics" table, pp. 7-8.

[86] Institute of Medicine, 2011, "Perspectives on Biomarker and Surrogate Endpoint Evaluation: Discussion Forum Summary," p. 6, http://www.iom.edu/Reports/2011/Perspectives-on-Biomarker-and-Surrogate-Endpoint-Evaluation.aspx.

Subtitle C—FDA Advancement of Precision Medicine

Section 2041. Precision Medicine Guidance and Other Programs of Food and Drug Administration

Background

Precision medicine is a relatively new term for what has traditionally been called personalized medicine, the idea of providing health care to individuals based on specific patient characteristics. This approach relies on companion diagnostics to target drugs and biological products to specific subsets of patients.

Precision drugs and biologicals, because they may be treating small subsets of patients, sometimes qualify as "orphan drugs." Such drugs are called orphan drugs because firms may lack the financial incentives to sponsor products to treat small patient populations. Orphan drugs receive their designation pursuant to FFDCA Section 526(a),[87] a designation that was created by the Orphan Drug Act[88] to encourage firms to develop pharmaceuticals to treat rare diseases and conditions by providing an extended period of market exclusivity.

For drugs that address unmet needs or serious or life-threatening conditions, have the potential to offer better outcomes or fewer side effects, or meet other criteria associated with improved public health, FDA uses several formal mechanisms to expedite development or review processes.[89] These include "priority review," "breakthrough therapy," and "fast track" designations, and the accelerated approval pathway.[90]

Provision

The provision would add a new Subchapter J, Precision Medicine, to Chapter V of the FFDCA; this subchapter would include two new sections: (1) Section 591, "General agency guidance on precision medicine," and (2) Section 592, "Precision medicine regarding orphan-drug and expedited-approval programs." New FFDCA Section 591 would require the Secretary, not later than 18 months after enactment, to issue and periodically update guidance to help sponsors develop a precision drug or biological product. The guidance would have to, among other things, define the term "precision drug or biologic product," and address topics such as the evidence needed to support the use of biomarkers to identify subsets of patients for streamlining clinical trials, the design of studies to demonstrate a biomarker's validity, and considerations for inclusion of biomarker information in prescription drug or biological labeling.

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[87] FFDCA Section 526, "Designation of Drugs for Rare Diseases or Conditions"; 21 U.S.C. 360bb.

[88] P.L. 97-414, 96 Stat. 2049 (1982).

[89] FDA, "DRAFT Guidance for Industry: Expedited Programs for Serious Conditions—Drugs and Biologics," Center for Drug Evaluation and Research and Center for Biologics Evaluation and Research, June 2013, http://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM358301.pdf; and FDA, "Review Designation Policy: Priority (P) and Standard (S)," MAPP 6020.3 Rev. 2, Manual of Policies and Procedures, Center for Drug Evaluation and Research, Office of New Drugs, June 25, 2013, http://www.fda.gov/downloads/AboutFDA/CentersOffices/OfficeofMedicalProductsandTobacco/CDER/ManualofPoliciesProcedures/UCM082000.pdf.

[90] The fast track and breakthrough therapy designations and the accelerated approval pathway are authorized under FFDCA Section 506, "Expedited approval of drugs for serious or life-threatening diseases or conditions"; 21 U.S.C. 356. See FDA, "DRAFT Guidance for Industry: Expedited Programs for Serious Conditions—Drugs and Biologics," table, pp. 7-8.

For a precision drug or biological product application where the product is for the treatment of a serious or life-threatening disease or condition and has been designated as an orphan drug under FFDCA Section 526, the new FFDCA Section 592 would allow the Secretary to do two things. First, the Secretary would be allowed to rely on information about the drug or biological product that has been previously submitted, either by the same or a different sponsor (with permission), in approval of an application. This may be for either a new product, or for a different indication for an existing product. Second, it would allow the Secretary to consider the application for expedited review programs, including accelerated approval. New Section 592 should not be construed to limit the Secretary's product approval authorities, or to entitle sponsors to obtain information in another sponsor's application without permission of the other sponsor.

Subtitle D—Modern Trial Design and Evidence Development

Section 2061. Broader Application of Bayesian Statistics and Adaptive Trial Designs

Background

The traditional approach to clinical trials for drugs has focused on a design planned in advance that includes specific treatments and doses and durations, specified decision rules for patient/subject assignment to treatment groups, and prespecified statistical analysis to test a prespecified qualitative and quantitative hypothesis. Because the analytic plan is set in advance, it does not lend itself to unintentional (or intentional) bias as data are reviewed. A researcher may feel strongly about a hypothesis and hope that the results will confirm an idea, but he or she must carry out the analysis so the results can be understood and replicated by others. A drawback to trials with this kind of static design is that they tend to take a long time and cannot adapt to new information learned during the trial. In recent years, some clinical and methodological researchers have looked to *adaptive* trial designs and statistical analyses using techniques (such as Bayesian statistics) that can provide mid-course feedback. Because a mistaken finding of effectiveness or safety could put a dangerous drug on the market or delay the approval of a useful drug, FDA has acted cautiously in accepting alternative trial designs. In 2010, FDA published draft guidance on the use of adaptive trial design.[91]

Provision

The provision would require the Secretary to (1) update and finalize the draft guidance and (2) "issue draft guidance on the use of Bayesian methods in the development and regulatory review and approval or licensure of drugs and biological products." It would require that the guidances address the use of adaptive designs and Bayesian methods to meet the "substantial evidence" standard in FDA's review of safety and effectiveness data in marketing applications, technical feedback to drug sponsors, "the types of quantitative and qualitative information that should be submitted for review," and "recommended analysis methodologies." The provision would require the Secretary to conduct a public meeting of stakeholders before "updating or developing" these two guidances. The provision would require the Secretary to publish the first guidance no later

[91] FDA, "DRAFT Guidance for Industry: Adaptive Design Clinical Trials for Drugs and Biologics," Center for Drug Evaluation and Research and Center for Biologics Evaluation and Research, February 2010, http://www.fda.gov/RegulatoryInformation/Guidances/default.htm.

than 18 months after the date of the public meeting, and the second guidance no later than 48 months after the date of the public meeting.

Section 2062. Utilizing Evidence from Clinical Experience

Background

To approve a new drug for marketing in the United States, FDA reviews the sponsor's new drug application (NDA) to assess, among other things, whether the drug is safe and effective for its intended purpose. FFDCA Section 505(d) refers to "substantial evidence," which it defines as

> evidence consisting of adequate and well-controlled investigations, including clinical investigations, by experts qualified by scientific training and experience to evaluate the effectiveness of the drug involved, on the basis of which it could fairly and responsibly be concluded by such experts that the drug will have the effect it purports or is represented to have under the conditions of use prescribed, recommended, or suggested in the labeling or proposed labeling thereof. If the Secretary determines, based on relevant science, that data from one adequate and well-controlled clinical investigation and confirmatory evidence (obtained prior to or after such investigation) are sufficient to establish effectiveness, the Secretary may consider such data and evidence to constitute substantial evidence for purposes of the preceding sentence. The Secretary shall implement a structured risk-benefit assessment framework in the new drug approval process to facilitate the balanced consideration of benefits and risks, a consistent and systematic approach to the discussion and regulatory decisionmaking, and the communication of the benefits and risks of new drugs. Nothing in the preceding sentence shall alter the criteria for evaluating an application for premarket approval of a drug.

The associated rules (21 C.F.R. 314.126) describe characteristics of "adequate and well-controlled studies," which include a statement of objectives, an analytic plan, a control group, quantification of treatment duration and timing, and method of sample size determination. The study design would lead to the identification of appropriate research subjects and include methods to minimize bias in the assignment of subjects to treatment groups as well as in data analysis.

These characteristics basically describe a controlled (often randomized) clinical trial. The rule, however, places these characteristics in the context of having "been developed over a period of years and are recognized by the scientific community as the essentials of an adequate and well-controlled clinical investigation." The rule states that FDA "consider" these characteristics in its determination of effectiveness claims.

Provision

The provision would add a new Section 505F to the FFDCA, requiring the Secretary to "establish a program to evaluate the potential use of evidence from clinical experience to help support the approval of a new indication for a drug approved under Section 505(b) and to help support or satisfy postapproval study requirements." The provision would define "evidence from clinical experience" as "data regarding the usage, or the potential benefits or risks, of a drug derived from sources other than randomized clinical trials, including from observational studies, registries, and therapeutic use." It would require the Secretary to establish a draft framework for implementing the program to include specified content. Required consultation with interested parties could be done via a public-private partnership or a contract, grant, or other appropriate arrangement. The Secretary would be required to use the new "program to evaluate the potential use of evidence from clinical experience" to "inform" the development of guidance for industry. The provision would also state that "[t]this section shall not be construed to alter the standards of evidence

under" Section 505(c) or (d) of the FFDCA, including the substantial evidence standard; Section 351(a) of the PHSA; or "the Secretary's authority to require postapproval studies or clinical trials, or the standards of evidence under which studies or trials are evaluated."

This provision would add a new Section 505G to the FFDCA, requiring the Secretary to design and implement pilot demonstrations to use "data captured through the Sentinel System[92] surveillance infrastructure" to generate evidence of drugs' risks or benefits, protect the public health, and advance patient-centered care. The provision would allow the Secretary to "make strategic linkages with sources of complementary public health data and infrastructure." Regarding these pilots, the Secretary would be required to "(A) consult with regulated industry, academia, medical professional organizations, representatives of patient advocacy organizations, disease research foundations, and other interested parties through a public process; and (B) develop a framework to promote appropriate transparency and dialogue about research."

In addition, the new FFDCA Section 505G would allow the Secretary to deem such pilot demonstrations to be "public health activities" for purposes of permitting the use and disclosure of protected health information (as specified)[93] and placing them "outside the scope of 'research'"[94] for purposes of human subjects research protections (as specified).

The provision would also authorize to be appropriated $3 million for each of fiscal years FY2016 through FY2020.

Section 2063. Streamlined Data Review Program

Background

FFDCA Section 505 and accompanying regulations provide the framework for FDA's approval of sponsors' drug marketing applications. For a drug whose active ingredient has never been FDA-approved, the law requires the sponsor to submit a new drug application that includes data to provide evidence of the drug's safety and effectiveness for its intended use, information about the manufacturing process, and the drug labeling. Once a product has an approved NDA, FDA requires that the manufacturer submit a supplemental NDA each time the manufacturer wants to change the labeling, the manufacturing process, or the dosing, or when it wants to add a new indication (a new intended use) of the drug. Regulations at 21 C.F.R. Sections 314.50 and 314.54 describe the required contents of those applications. Regarding clinical data, the regulations direct the applicant to submit, in addition to descriptions and analysis of controlled and uncontrolled clinical studies,

> (iv) A description and analysis of any other data or information relevant to an evaluation of the safety and effectiveness of the drug product obtained or otherwise received by the applicant from any source, foreign or domestic, including information derived from clinical investigations, including controlled and uncontrolled studies of uses of the drug other than those proposed in the application, commercial marketing experience, reports in the scientific literature, and unpublished scientific papers. (21 C.F.R. 314.50(d)(5)(iv))

[92] In May 2008, FDA launched the Sentinel Initiative "to develop and implement a proactive system"—the Sentinel System—to actively query existing sources of healthcare data (e.g., electronic health record systems and insurance claims databases) "to evaluate possible medical product safety issues quickly and securely." HHS, FDA, *FDA's Sentinel Initiative*, http://www.fda.gov/Safety/FDAsSentinelInitiative/default.htm.

[93] 45 C.F.R. §164.512(b)(1).

[94] 45 C.F.R. §46.102(d).

The clinical data submission must also include an "integrated summary of the data demonstrating substantial evidence of effectiveness for the claimed indications."

Provision

The provision would add a new Section 505H to the FFDCA that would address the data requirements in a supplemental NDA that a sponsor of an approved drug would submit when seeking to add to the approval a new indication that is "qualified" (defined in this section as treating cancer or other indications as determined by the Secretary). FFDCA Section 505H would require the Secretary to "establish a streamlined data review program" through which a sponsor could submit a "qualified data summary," defined as "a summary of clinical data intended to demonstrate safety and effectiveness with respect to a qualified indication for use of a drug," when "there is an existing database acceptable to the Secretary regarding the safety of the drug developed for one or more indications" of the approved drug. The sponsor would also be required to submit "the full data sets used to develop the qualified data summaries ... unless the Secretary determines that the full data sets are not required."

The provision would state a sense of Congress that the new streamlined data review program "should enable the Food and Drug Administration to make approval decisions for certain supplemental applications based on qualified data summaries (as defined in such section 505H)."

The provision would require that the Commissioner of Food and Drugs issue implementation guidance for the streamlined data review program and would allow the Commissioner to issue regulations for implementation.

Subtitle E—Expediting Patient Access

Section 2081. Sense of Congress

Background

FDA uses several formal mechanisms to expedite the development or review processes for drugs that address unmet needs or serious conditions, that have the potential to offer better outcomes or fewer side effects, or that meet other criteria associated with improved public health. FFDCA Section 506, which Congress added in 2012,[95] introduced the breakthrough therapy designation for a drug that would treat a serious condition and for which preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over available therapies on a clinically significant endpoint (or endpoints). FDA provides breakthrough therapies with intensive guidance during drug development and organizational commitment involving senior managers. The requirements for drug approval, however, do not change.[96] Breakthrough therapy designation, therefore, affects the timing and smoothness of the application process. Such designation does not alter the types of evidence required to demonstrate safety and effectiveness.

Provision

The provision would express the sense of Congress that FDA should approve drugs designated as breakthrough therapies "as early as possible in the clinical development process, regardless of the

[95] Section 902 of the Food and Drug Administration Safety and Innovation Act (FDASIA), P.L. 112-144.

[96] The FFDCA and agency regulations allow alteration of the evidence required for approval in other circumstances; these alterations are not connected to breakthrough designation.

phase of development," provided that the applications meet existing standards of evidence of safety and effectiveness, as determined by the HHS Secretary.

Section 2082. Expanded Access Policy

Background

FDA regulates the U.S. sale of drugs and biological products, basing approval or licensure on evidence of the safety and effectiveness for a product's intended uses. Without that approval or licensure, a manufacturer may not distribute the product except for use in the clinical trials that will provide evidence to determine that product's safety and effectiveness. Under certain circumstances, however, FDA may permit the sponsor to provide an unapproved or unlicensed product to patients outside that standard regulatory framework. One such mechanism is expanded access to investigational drugs, commonly referred to as compassionate use.

If excluded from a clinical trial because of its enrollment limitations, a person, acting through a physician, may request access to an investigational new drug outside of the trial. FDA may grant expanded access to a patient with a serious disease or condition for which there is no comparable or satisfactory alternative therapy, if, among other requirements, probable risk to the patient from the drug is less than the probable risk from the disease; if there is sufficient evidence of safety and effectiveness to support the drug's use for this person; and if providing access "will not interfere with the ... clinical investigations to support marketing approval."[97] The widespread use of expanded access is limited by an important factor: whether the manufacturer agrees to provide the drug, which—because it is not FDA-approved—cannot be obtained otherwise. FDA does not have the authority to compel a manufacturer to participate. Manufacturers consider several factors in deciding whether to provide an investigational drug, such as available supply, perceived liability risk, limited staff and facility resources, and need for data to assess safety and effectiveness. Although FDA reports the number of investigational drug requests it receives, manufacturers do not.

Provision

The provision would add a new Section 561A to the FFDCA to require the manufacturer or distributor of an investigational drug to make publicly available its policy "on evaluating and responding to requests ... for provision of such a drug." Required elements of the policy would include contact information for the manufacturer or distributor of the drug, request procedures, "the general criteria the manufacturer or distributor will consider or use to approve such requests," and anticipated time to acknowledge request receipts. The new section would state that posting of policy would not guarantee patients access to an investigational drug. The provision would also allow the manufacturer or distributor to revise its policy at any time.

Section 2083. Finalizing Draft Guidance on Expanded Access

Background

FFDCA Section 561(b) allows a person, acting through a licensed physician, to request a manufacturer or distributor of an investigational product to provide that product under specified circumstances and conditions. The sponsor or clinical investigator must provide the HHS Secretary with information as required by regulations. Although FDA has approved patient access

[97] FFDCA Section 561(b).

in over 99% of the requests to which the sponsor has agreed, some sponsors have been reluctant to provide investigational drugs outside of the standard investigational new drug (IND) processes because of the uncertainty of how FDA would consider potential adverse events associated with the expanded access use in its assessment of the drug's safety, which could influence whether an NDA is approved.

Provision

This provision would require that the HHS Secretary finalize the guidance "Expanded Access to Investigational Drugs for Treatment Use—Qs & As," issued in draft form in May 2013.[98] The provision would require that the final guidance "clearly define how the Secretary of Health and Human Services interprets and uses adverse drug event data reported by investigators in the case of data reported from use under a request submitted under" FFDCA Section 561(b).

Subtitle F—Facilitating Responsible Manufacturer Communications

Section 2101. Facilitating Dissemination of Health Care Economic Information

Background

FFDCA Section 502 includes "[i]f its labeling is false or misleading in any particular" in the list of circumstances under which a drug is "deemed to be misbranded." It allows a drug's sponsor (usually its manufacturer or distributor) to provide health care economic information to entities such as formulary committees for use in decisions regarding drug selection for managed care. The section defines "health care economic information" to mean "any analysis that identifies, measures, or compares the economic consequences, including the costs of the represented health outcomes, of the use of a drug to the use of another drug, to another health care intervention, or to no intervention." The information must be "based on competent and reliable scientific evidence." Provision of this information is allowed only regarding indications that are included in the drug's approval; the provision of health care economic information regarding unapproved indications could be considered false and misleading.

Provision

The provision would amend the description of the recipient of the information to include a "payor" and to refer to the use of the information in the "selection of drugs for coverage or reimbursement." The requirement that information be "based on competent and reliable scientific evidence" would be expanded to include "where applicable, a conspicuous and prominent statement describing any material differences between the health care economic information and the labeling approved for the drug."

The provision would amend the definition of "health care economic information" to include "the clinical data, inputs, clinical or other assumptions, methods, results, and other components underlying or comprising the analysis." It would specify that the economic consequences "may be based on the separate or aggregated clinical consequences of the represented health outcomes, of

[98] FDA, "DRAFT Guidance for Industry: Expanded Access to Investigational Drugs for Treatment Use—Qs & As," May 2013, http://www.fda.gov/downloads/drugs/guidancecomplianceregulatoryinformation/guidances/ucm351261.pdf

the use of a drug." Lastly, the revised definition would rephrase the reference to comparisons, without an apparent change in authority or policy.

Section 2102. Facilitating Responsible Communication of Scientific and Medical Developments

Background

FFDCA Section 505 governs approval of new drugs; approval is linked to the intended use (indication) of the drug, for which FDA reviews evidence of safety and effectiveness that the sponsor provides. Approval is also linked to the drug labeling provided by the sponsor. Intended uses not covered by the approval are not included in the labeling.

FFDCA Section 502 describes circumstances under which a product is to be deemed *misbranded*. Among those are when labeling is false or misleading. FDA has interpreted the FFDCA, therefore, to prohibit a manufacturer from promoting or advertising a drug for any use not listed in the FDA-approved labeling, which contains those claims for which FDA has reviewed safety and effectiveness evidence.[99] However, FDA's interpretation has been challenged and is in dispute.[100]

FDA has acknowledged that the manufacturer has information that may be useful to clinicians in their treatment of patients. In a 2009 guidance,[101] for example, the agency noted that "[o]nce a drug or medical device has been approved or cleared by FDA, generally, healthcare professionals may lawfully use or prescribe that product for uses or treatment regimens that are not included in the product's approved labeling (or, in the case of a medical device cleared under the 510(k) process, in the product's statement of intended uses)." FDA, therefore, recognized the "public health and policy justification" in allowing certain information on unapproved uses of approved products. FDA has released several draft guidance documents[102] to characterize the circumstances

[99] Materials from FDA's Bad Ad Program describe elements of false or misleading ads. These include promotion of an unapproved use. See FDA, "Truthful Prescription Drug Advertising and Promotion," http://www.fda.gov/Drugs/GuidanceComplianceRegulatoryInformation/Surveillance/DrugMarketingAdvertisingandCommunications/ucm209384.htm#ExamplesofViolations. See also FFDCA §§ 301 and 502(a).

[100] A December 2012 Court of Appeals decision (United States v. Caronia) "reversed the criminal conviction of a pharmaceutical representative who had promoted an off-label use of a drug on First Amendment grounds" (Andrew Nolan, "Is There a Constitutional Right to Promote an Unapproved Use for a Drug?" Legal Sidebar, Congressional Research Service, April 2, 2013, http://www.crs.gov/LegalSidebar/details.aspx?ID=436&Source=search).

[101] FDA, "Guidance for Industry: Good Reprint Practices for the Distribution of Medical Journal Articles and Medical or Scientific Reference Publications on Unapproved New Uses of Approved Drugs and Approved or Cleared Medical Devices," Office of the Commissioner, Office of Policy, January 2009, http://www.fda.gov/RegulatoryInformation/Guidances/ucm125126.htm.

[102] FDA, "'Off-Label' and Investigational Use Of Marketed Drugs, Biologics, and Medical Devices - Information Sheet," Guidance for Institutional Review Boards and Clinical Investigators, http://www.fda.gov/regulatoryinformation/guidances/ucm126486.htm; FDA, "DRAFT Guidance for Industry: Distributing Scientific and Medical Publications on Risk Information for Approved Prescription Drugs and Biological Products—Recommended Practices," Center for Drug Evaluation and Research, Center for Biologics Evaluation and Research, and Center for Veterinary Medicine, June 2014, http://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM400104.pdf; FDA, "Revised DRAFT Guidance for Industry: Distributing Scientific and Medical Publications on Unapproved New Uses—Recommended Practices," Center for Drug Evaluation and Research, Center for Biologics Evaluation and Research, and Center for Devices and Radiological Health, February 2014, http://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM387652.pdf; and FDA, "DRAFT Guidance for Industry: Responding to Unsolicited Requests for Off-Label Information About Prescription Drugs and Medical Devices," Center for Drug Evaluation and Research, Center for Biologics Evaluation Research, Center for Veterinary Medicine, and Center for Devices and Radiological Health, December 2011,

in which it would allow manufacturers to provide information on off-label uses of drugs approved for other uses without triggering the misbranding provision and without using a manufacturer's dissemination of the information as evidence of an intended new indication.

Provision

The provision would require the HHS Secretary to "issue draft guidance on facilitating the responsible dissemination of truthful and non-misleading scientific and medical information not included in the approved labeling of drugs and devices."

Subtitle G—Antibiotic Drug Development

Section 2121. Approval of Certain Drugs for Use in a Limited Population of Patients

Background

According to the CDC, each year in the United States, at least 2 million people become infected with bacteria that are resistant to antibiotics, and at least 23,000 of them die from these infections.[103] Antibiotics are intended for short-term use, making the development of new ones potentially less attractive to drug developers. Addressing barriers to antibiotic drug approval may help counter this problem. One such proposal is the so-called Limited Population Antibacterial Drug (LPAD) approval pathway for new antibacterial drugs.[104] Such a pathway would involve smaller clinical trials in a limited population of patients that have serious or life-threatening infections and unmet medical needs due to the lack of an effective approved antibiotic. This streamlined approach would result in more uncertainty about potential risks posed by the product, and therefore a greater need for post-market scrutiny.[105]

Provision

The stated purpose of this provision, in subsection (a), is

> to help expedite the development and availability of treatments for serious or life-threatening bacterial or fungal infections in patients with unmet needs, while maintaining safety and effectiveness standards for such treatments, taking into account the severity of the infection and the availability or lack of alternative treatments.

It would do so by adding, in subsection (b), a new FFDCA subsection 505(z), an expedited review pathway, effective upon enactment, for certain antibacterial and antifungal drugs (including biologics) intended for use in limited, defined populations of patients that have severe,

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http://www.fda.gov/downloads/drugs/guidancecomplianceregulatoryinformation/guidances/ucm285145.pdf.

[103] Centers for Disease Control and Prevention (CDC), "Antibiotic Resistance Threats in the United States, 2013," http://www.cdc.gov/drugresistance/threat-report-2013/.

[104] See for example testimony of Janet Woodcock, Director, FDA Center for Drug Evaluation and Research, U.S. Congress, House Committee on Energy and Commerce, Subcommittee on Health, *21st Century Cures: Examining Ways to Combat Antibiotic Resistance and Foster New Drug Development*, 113th Cong., 2nd sess., September 19, 2014, http://www.fda.gov/newsevents/testimony/ucm415387.htm.

[105] Ibid. See also Executive Office of the President, President's Council of Advisors on Science and Technology (PCAST), *Report to the President on Combating Antibiotic Resistance*, "Goal 4.2. Drug approval based on clinical trials in limited patient populations," September 2014, pp. 32 ff., https://www.whitehouse.gov/administration/eop/ostp/pcast.

life-threatening infections for which current treatment options may be limited or absent, and for which the benefits of a product could outweigh harms that would not be acceptable in broader population use. This review pathway would include the following elements, among others:

- Upon a sponsor's request, FDA may enter into written agreement with the sponsor to define the process and data needed to review the limited population use application. The process could not proceed without such written agreement.
- The Secretary may consider limited data sets and non-clinical data as substantial evidence of safety and effectiveness, recognizing the smaller populations available for study of an LPAD drug, and the different balance of benefit versus harm in these populations.
- The process must adhere to existing goals and procedures agreed upon by sponsors and FDA in the Prescription Drug User Fee Amendments of 2012 (P.L. 112-144, Title I).
- Products approved using this pathway must carry prominent labeling noting the intended use for a limited and specific population of patients.
- Sponsors must submit promotional materials to FDA for review 30 days prior to dissemination.
- Sponsors may pursue this pathway concurrently with other specified streamlined approval pathways, as applicable.
- This provision would not alter current prescribing or other medical practices (such as off-label prescribing).

Subsection (c) of this provision would require FDA to issue draft implementation guidance within 18 months of enactment. Subsection (d) provides conforming amendments. Subsection (e) would require the Secretary to conduct and publish an assessment of the program within 48 months of enactment, and seek public input. Subsection (f) would allow the Secretary to expand the limited population use pathway if deemed beneficial by the assessment above. Subsection (g) would add a new subsection 317U to the PHSA to establish a monitoring system for the use of antibacterial and antifungal drugs, including products approved under the limited population use pathway, as well as changes in bacterial and fungal resistance to drugs. The Secretary would be required to make summaries of data from this system publicly available.

Section 2122. Susceptibility Test Interpretive Criteria for Microorganisms

Background

Laboratory tests can help clinicians determine whether a drug is likely to work against a specific infection by showing whether the infectious organism is susceptible (vs. resistant) to that drug. The criteria that distinguish susceptibility from resistance are called "breakpoints." Under current law and regulation, breakpoint information must be provided on antimicrobial drug labels, and labels for Antimicrobial Susceptibility Testing (AST) devices must reflect the relevant drug label(s). Generally, sponsors must apply to make changes to information contained in these drug and device labels, and FDA must approve label changes for these drugs and devices. However, the susceptibility of infectious organisms may change over time, rendering label information inaccurate for clinical decision-making purposes. FDA, clinicians, and others have sought to

streamline FDA's process to ensure that antimicrobial drug and AST device labels reflect current information.[106]

Provision

Subsection (a) of this provision would replace the existing language of FFDCA Section 511 (which requires the Secretary to publish guidance for industry regarding the review of antibiotic drugs) with new language. It would require the Secretary to establish, within one year of enactment, a public "Interpretive Criteria Website," and to review and revise the content of such website every six months thereafter. The stated purpose of the website is to identify and publish current, generally accepted standards, including breakpoint information (i.e., interpretive criteria), used to guide testing of test bacteria, fungi, or other microorganisms for susceptibility to antimicrobial drugs, and to use these criteria to inform the use of AST devices.

The Secretary would be required to identify appropriate susceptibility test interpretive criteria ("criteria") for approved or licensed antimicrobial drugs through review of preclinical, clinical, and statistical information and other available evidence. Within one year of enactment, the Secretary would be required to establish, and thereafter maintain, the Interpretive Criteria Website containing two lists: (1) a list of any criteria standards established by a nationally or internationally recognized standard development organization, where such organization meets specified requirements for transparency and management of potential conflicts of interest, among other things; and (2) a list of criteria that, although determined by the Secretary to be appropriate with respect to approved or licensed antimicrobial drugs, lack a recognized standard, for one of several stated reasons. The website would have to include several specific disclaimers regarding the uses and limitations of the information presented. The Secretary would be required to publish in the *Federal Register* a notice of establishment of the website not later than the date on which it is established.

The Secretary would be required to review any new or updated criteria standards from a recognized standard development organization, revise the website accordingly, and make public a notice of any such revisions on the FDA agency website, at least every six months. Any such notices would be required to be compiled and published in the *Federal Register* at least annually, with a request for public comments. The Secretary would be allowed to consider public comments, among other things, in revising website content.

Both criteria standards and non-standard criteria listed on the website would be considered to be recognized standards for the purpose of premarket review and other legal requirements for devices, pursuant to FFDCA Section 514(c)(1). However, sponsors would be allowed to use standards other than those listed by FDA under this section in seeking approval or clearance of a drug or device. The provision would require that antimicrobial drugs sold after the Interpretive Criteria Website is established carry a reference to the website on the label, and that sponsors of antimicrobial drugs sold before the website was established submit, within one year of establishment of the website, supplemental applications to similarly change the label. The provision would clarify that reference to the website in the labeling of an antimicrobial drug

[106] See, for example, testimony of Janet Woodcock, Director, FDA Center for Drug Evaluation and Research, U.S. Congress, House Committee on Energy and Commerce, Subcommittee on Health, *21st Century Cures: Examining Ways to Combat Antibiotic Resistance and Foster New Drug Development*, 113th Cong., 2nd sess., September 19, 2014, http://www.fda.gov/newsevents/testimony/ucm415387.htm; and FDA, "Guidance for Industry, Updating Labeling for Susceptibility Test Information in Systemic Antibacterial Drug Products and Antimicrobial Susceptibility Testing Devices," June 2009.

would not constitute misbranding, and state that FFDCA Section 511 should not be construed to allow the Secretary to disclose protected trade secret or confidential information.

The provision would allow the Secretary to authorize the marketing of an AST device for which the label references information from the website in lieu of information from clinical trials, and directs practitioners to information on the labels of drugs tested using such device.

Subsection (b) of the provision would make conforming amendments to the FFDCA. Subsection (c) would require the Secretary to report to Congress regarding progress in implementing this section. Subsection (d) would exempt FDA from requirements under the Paperwork Reduction Act when updating the list of susceptibility test interpretive criteria standards.[107] Subsection (e) states that provisions of Subtitle G of the bill should not be construed to restrict antibiotic or other drug prescribing or administering practices by health care practitioners.

Section 2123. Encouraging the Development and Use of DISARM Drugs

Background

Under Medicare's Hospital Inpatient Prospective Payment System (IPPS), reimbursement is often predetermined for each discharge based on a patient's condition and related treatment strategy, and other factors. To account for patients' needs, Medicare assigns discharges to Medicare-severity diagnosis related groups (MS-DRGs). The capitated MS-DRG payment can discourage the use of new technologies if they are more expensive than standard care. To address this, Sections 1886(d)(5)(K) and (L) of the Social Security Act (SSA) authorize additional payments for new medical services and technologies under the IPPS in addition to the MS-DRG reimbursement.[108]

In 2012, Congress passed the Generating Antibiotic Incentives Now (GAIN) Act (Title VIII of the Food and Drug Administration Safety and Innovation Act, P.L. 112-144) to address barriers to antibiotic drug development. Under GAIN, a qualified infectious disease product (QIDP)—defined as a human antibacterial or antifungal drug intended to treat serious or life-threatening infections—would, among other things, be eligible for expedited FDA review and extended postmarket patent exclusivity under FFDCA Section 505E.[109]

Provision

Subsection (a) of this provision would add a new subparagraph 1886(d)(5)(M) to the SSA to provide add-on payments under the IPPS for the use of "DISARM drugs" for discharges from eligible hospitals, beginning with FY2018. It defines a "DISARM drug" as one that is FDA-approved on or after December 1, 2014, and that FDA determines is an antimicrobial product (1) that is intended to treat an infection for which there is an unmet medical need and that is associated with high mortality or morbidity, as determined by FDA in consultation with the CDC Director and the infectious disease professional community; and (2) that is either intended to treat a qualifying pathogen, or is a QIDP (as defined in FFDCA Sections 505E(f) and 505E(g), respectively). Once made, such determination would be revocable only if the request for it

[107] See CRS Report R40636, *Paperwork Reduction Act (PRA): OMB and Agency Responsibilities and Burden Estimates*.

[108] Centers for Medicare & Medicaid Services (CMS), "New Medical Services and New Technologies," http://www.cms.gov/Medicare/Medicare-Fee-for-Service-Payment/AcuteInpatientPPS/newtech.html.

[109] See "Title VIII—Generating Antibiotic Incentives Now," in CRS Report R42680, *The Food and Drug Administration Safety and Innovation Act (FDASIA, P.L. 112-144)*.

contained a false statement. The Secretary would be required to list new antimicrobial drugs that are eligible for add-on payments in annual IPPS rulemaking. Once listed, add-on payments made for new antimicrobial drugs would be made for the first five consecutive fiscal years (and would include prior fiscal years if an antimicrobial drug qualified as a new technology add-on payment). The provision defines "eligible hospital" to mean an acute care hospital in the 50 states and Washington, DC, that is reimbursed by Medicare under the IPPS and that reports its antimicrobial drug use data to the CDC's National Healthcare Safety Network or similar surveillance system.

Additional payment for an eligible DISARM drug would be in the amount provided for such drug under SSA Section 1847A, which establishes Medicare's reimbursement methodology for drugs covered under Medicare Part B. The total amount of add-on payments for DISARM drugs during a fiscal year would be capped at 0.02% of the estimated total Medicare program payments made under SSA Section 1886(d) during such fiscal year. The Secretary would be allowed to reduce the reimbursement rates pro rata for DISARM drugs to ensure add-on payments do not exceed the 0.02% cap.

Conforming amendments would ensure no duplication under the new medical services and technology add-on payment policy for an eligible drug. In addition, this provision would require manufacturers participating in the Medicaid drug rebate program (under SSA Section 1927) to report to the Secretary within 30 days of the end of each rebate period the manufacturer's wholesale acquisition cost and information on sales at a nominal price. These data, like other drug price data required under the Medicaid rebate program for Medicare drugs (under SSA Section 1847A), would have to be reported by National Drug Code and package size.

Subsection (b) of this provision would require the Comptroller General to study and report within one year of enactment on barriers to the development of DISARM drugs, and make recommendations to overcome such barriers.

Subsection (c) of this provision would require the CDC Director to study and report within three years of enactment on the impact of additional Medicare payment for DISARM drugs on usage practices and the development of resistance by individuals to such drugs.[110]

Subtitle H—Vaccine Access, Certainty, and Innovation

Background

Authority for the National Vaccine Program (NVP), in Title XXI of the PHSA, establishes responsibilities for the Secretary of HHS to coordinate a variety of vaccine activities across the federal government. The Advisory Committee on Immunization Practices (ACIP) advises the Director of the Centers for Disease Control and Prevention regarding the use of vaccines that are licensed by the Food and Drug Administration for use in the United States.[111] ACIP is not explicitly authorized in the PHSA or elsewhere in federal law. Rather, its authority is based in general authority of the HHS Secretary to establish advisory committees.[112] However, ACIP has been given explicit statutory roles under the PHSA and the Social Security Act (SSA), and

[110] Amendment 5 (Slaughter) to H.R. 6 added subsection (c) regarding the CDC report; it was approved by voice vote.

[111] The FDA evaluates the safety and effectiveness of individual vaccine products in making its licensure decisions. The ACIP recommends how licensed vaccines should be used for specific public health purposes, for example, the routine immunization of infants, or use among selected groups for outbreak control.

[112] PHSA Section 222; 42 U.S.C. §217a.

ACIP's actions pursuant to these roles affect the market for vaccine products. These roles are as follows:

- PHSA Section 2713 requires most private health insurance plans, unless grandfathered, to cover, without any cost-sharing, immunizations recommended by ACIP.[113] Pursuant to regulations, this requirement is effective for a vaccine if and when an ACIP recommendation for use of that vaccine has been adopted by CDC and published on CDC's Immunization Schedules.[114]
- SSA Section 1928 establishes the Vaccines for Children (VFC) program, which provides federally purchased vaccines free of charge to eligible children.[115] VFC vaccines are those for which ACIP has issued a recommendation for use in children.

ACIP is subject to requirements of the Federal Advisory Committee Act, which is intended to improve the transparency of executive branch advisory committees.[116] It typically meets three times per year to discuss and vote on recommendations. Vaccine-specific work groups within ACIP carry out their work during and between full committee meetings.[117]

Section 2141. Timely Review of Vaccines by the Advisory Committee on Immunization Practices

Provision

The provision would add a 10th item to the statutory list of responsibilities of the Director of the National Vaccine Program, pursuant to PHSA Section 2102(a). It would require the NVP Director to direct ACIP to consider the use of any vaccine newly licensed or licensed for a new indication by FDA at the committee's next regularly scheduled meeting. It would require ACIP to make recommendations on an expedited basis in two circumstances: (1) if ACIP does not issue recommendations regarding a licensed vaccine at its next regularly scheduled meeting, and is then asked by the vaccine's sponsor to make such recommendations on an expedited basis; or (2) when FDA licenses a vaccine that is designated as a breakthrough therapy to treat a serious or life-threatening disease or condition (pursuant to Section 506 of the Federal Food, Drug, and Cosmetic Act [FFDCA]).

Section 2142. Review of Processes and Consistency of ACIP Recommendations

Provision

The provision would require the CDC Director to review ACIP processes, evaluation criteria, and consistency in issuing recommendations, and to publish a report on such review not later than 18 months after enactment, including recommendations to improve the consistency of ACIP's processes.

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[113] 42 U.S.C. § 300gg-13(a)(2). Regulations are at 45 C.F.R. §147.130.

[114] An immunization schedule is the series of immunizations recommended for an individual over time, depending on age and other characteristics. CDC, "Immunization Schedules," http://www.cdc.gov/vaccines/schedules/index.html.

[115] SSA subsections 1928(c) (2)(B)(i) and 1928(e); 42 U.S.C. §§1396s(c)(2)(B)(i) and 1396s(e). For more information, see CDC VFC home page, http://www.cdc.gov/vaccines/programs/vfc/index.html.

[116] For more information, see CRS Report 97-71, *Access to Government Information In the United States: A Primer*.

[117] For more information about the ACIP, see CDC ACIP home page, http://www.cdc.gov/vaccines/acip/index.html.

Section 2143. Meetings Between CDC and Vaccine Developers

Provision

The provision would require CDC personnel to meet with vaccine developers regarding their vaccine products that are either licensed by FDA, or for which a developer intends to seek licensure. The stated purpose is for CDC to share with developers information about epidemiology and related matters that could inform the sponsor's vaccine research and development plan. The section specifies types of information that may be shared, deadlines, representation at meetings, and other administrative matters.

Subtitle I—Orphan Product Extensions Now; Incentives for Certain Products for Limited Populations

Section 2151. Extension of Exclusivity Periods for a Drug Approved for a New Indication for a Rare Disease or Condition

Background

Through various statutory authorities and regulatory actions, FDA provides incentives to those who would develop certain categories of drugs. The main set of incentives is the granting of market exclusivity. The FFDCA has provisions to grant market exclusivity for statutorily defined time periods (in months or years) to the holder of the NDA for a product that is, for example, the first generic version of a drug to come to market, a drug used in the treatment of a rare disease or condition, certain pediatric uses of approved drugs, and new qualified infectious disease products. During the period of exclusivity, FDA does not grant marketing approval to another manufacturer's product.

Provision

This provision would add a new Section 505I to the FFDCA that would add six months to the exclusivity period of an approved drug already on the market when FDA approves a supplemental application for that drug for a new indication to prevent, diagnose, or treat a rare disease or condition. The sponsor of a drug that receives the extended exclusivity under this provision would be required to notify the Secretary "of any discontinuance of the production of the drug for solely commercial reasons at least one year before such discontinuance." The six-month extension would not be available for a drug that had already received a six-month extension under this provision.

Section 2152. Reauthorization of Rare Pediatric Disease Priority Review Voucher Incentive Program

Background

The Food and Drug Administration Safety and Innovation Act (P.L. 112-144) added FFDCA Section 529 to create a new program, funded by user fees, to provide a transferable voucher, under specified conditions, to a sponsor of an approved new drug or biological product for a rare pediatric disease to be used for the priority review of another application. In addition, FDASIA terminated the authority to award such vouchers one year after the Secretary awards the third

priority voucher and required the GAO, beginning on the date of the third voucher award, to study and then report on the effectiveness of the voucher program in the development of products that prevent or treat rare pediatric diseases. FDA awarded the third voucher in March 2015, triggering the March 2016 sunset of this authority.

Provision

This provision would extend the authority to award such priority review vouchers until December 31, 2018. A new drug application or a biologics license application that was submitted to FDA after the enactment of H.R. 6 and before December 31, 2018, would remain eligible to receive a priority review voucher even if approval comes after December 31, 2018. It also would amend the definition of "rare pediatric disease" by adding the words shown here in italics: "The disease *is a serious or life-threatening disease in which the serious or life-threatening manifestations* primarily affect individuals aged from birth to 18 years, including age groups often called neonates, infants, children, and adolescents." The provision would add to the list of characteristics of a rare disease product application that the product not have received a tropical disease priority review voucher.[118]

This provision would require that GAO study the voucher program and report to Congress on its effectiveness as an incentive for developing drugs that treat or prevent rare pediatric diseases and that would not otherwise have been developed.

Subtitle J—Domestic Manufacturing and Export Efficiencies

Section 2161. Grants for Studying the Process of Continuous Drug Manufacturing

Background

In March 2015 congressional testimony, the then Commissioner of Food and Drugs spoke of new manufacturing technologies that could eventually "lower costs, limit drug shortages, and reduce supply chain vulnerabilities."[119] Continuous manufacturing, for example, could produce a drug in a "continuous stream" rather than in a "series of sequential and discrete" operations. She noted the need for "academic research in this area and expanding opportunities for collaboration, possibly through public-private partnerships or consortia."

Provision

The provision would authorize the Commissioner of Food and Drugs to "award grants to institutions of higher education and nonprofit organizations for the purpose of studying and recommending improvements to the process of continuous manufacturing of drugs and biological products and similar innovative monitoring and control techniques." To do so, it would authorize $5 million to be appropriated for each of fiscal years FY2016 through FY2020.

[118] FFDCA Section 524. Priority review to encourage treatments for tropical diseases.

[119] Statement of Margaret A. Hamburg, M.D., Commissioner of Food and Drugs, Food and Drug Administration, Department of Health and Human Services, before the Committee on Health, Education, Labor and Pensions, United States Senate, March 10, 2015, http://www.fda.gov/newsevents/testimony/ucm437481.htm.

Section 2162. Re-Exportation Among Members of the European Economic Area

Background

Section 1003(f) of the Controlled Substances Import and Export Act allows the Attorney General to authorize "any controlled substance that is in schedule I or II, or is a narcotic drug in schedule III or IV, to be exported from the United States to a country for subsequent export from that country to another country" under certain conditions. One condition is that the controlled substance is not exported from the second country.

Provision

The provision would amend Section 1003(f) of the Controlled Substances Import and Export Act to modify the prohibition on exporting a controlled substance from a second country to allow exporting from the second country to a member of the European Economic Area. It would allow the continued re-exportation of the controlled substance among members of the European Economic Area, provided certain conditions are met. This provision would require the person who exported the controlled substance from the United States to provide documentation to the Attorney General after each re-exportation. A new subsection (g) would prohibit the Attorney General from promulgating or enforcing any regulation, guidance, or policy that impedes re-exportation of controlled substances among European Economic Area countries.

Subtitle K—Enhancing Combination Products Review

Section 2181. Enhancing Combination Products Review

Background

FDA regulatory authority over medical product safety and effectiveness covers drugs, biological products, and medical devices. The agency generally divides responsibilities for the review of marketing applications in its product-centered offices. The Center for Drug Evaluation and Research reviews new drug applications for approval, the Center for Biologics Evaluation and Research reviews biologics license applications for licensure, and the Center for Devices and Radiological Health reviews premarket approval applications for approval and 510(k) notifications for clearance. In 2002, Congress directed FDA to establish an Office of Combination Products to facilitate the timely review and regulation of drug-device, drug-biologic, and device-biologic combination products.[120]

Provision

The provision would amend FFDCA Section 503(g)(4)(C) to require that the Secretary "issue final guidance that describes the responsibilities of each agency center regarding its review of combination products."

[120] Section 2014 of the Medical Device User Fee and Modernization Act of 2002 (MDUFMA, P.L. 107-250).

Subtitle L—Priority Review for Breakthrough Devices

Background

FDA requires all medical device product manufacturers to register their facilities, list their devices with the agency, and follow general controls requirements. FDA classifies devices according to the risk they pose to the patient. Medical devices that present only minimal risk, such as plastic bandages, can be legally marketed upon registration alone. These low-risk devices are deemed exempt from premarket review, and manufacturers need not submit an application to FDA prior to marketing. About two-thirds of medical devices listed with FDA are exempt from premarket review; therefore, these devices would not have a need for "priority review."

Most moderate- and high-risk devices must go through premarket review to obtain the agency's permission prior to marketing. FDA grants this permission when a manufacturer meets regulatory premarket requirements and agrees to any necessary postmarket requirements, which vary according to the risk that a device presents. In general, for moderate-risk and high-risk medical devices, manufacturers can use two pathways to bring such devices to market with FDA's permission: (1) the PMA pathway and (2) the 510(k) pathway. There is a fundamental difference between the PMA and 510(k) pathways. In a PMA review, FDA determines whether the device is reasonably safe and effective for its intended use. In a 510(k) review, FDA determines whether the device is substantially equivalent to another device whose safety and effectiveness may never have been assessed.

The PMA pathway consists of conducting clinical studies, then submitting a premarket approval (PMA) application with evidence providing reasonable assurance that the device is safe and effective. The PMA process is generally used for novel and high-risk devices. It results in a type of FDA permission called *approval*. The other pathway involves submitting a premarket notification—also known as a 510(k), after the section in the FFDCA that authorized this type of notification. Under the 510(k), the manufacturer demonstrates that the device is substantially equivalent to a device already on the market (a predicate device) that does not require a PMA. The 510(k) process is unique to medical devices and if successful results in FDA *clearance*. Substantial equivalence is determined by comparing the performance characteristics of a new device with those of a predicate device; clinical data demonstrating safety and effectiveness are usually not required. Each year FDA typically evaluates about 40 PMA applications and more than 4,000 510(k) notifications. In general, FDA has 90 days to review a 510(k) notification and 180 days to review a PMA application.

The time it takes to review a medical device—total review time—is composed of the time FDA handles the application—FDA time—plus the amount of time the device sponsor or submitter takes to respond to requests by FDA for additional information about the device. FDA reviewers frequently need to ask for additional information—called an AI Letter—from the 510(k) device sponsors due to the incomplete nature or poor quality of the original submission.[121] For example, during 2014, FDA reviewers needed to request additional information from the sponsors of 70% of 510(k) submissions made to the agency.[122] During 2014, FDA data indicate that 59% of PMA applications had a major deficiency requiring additional information from the device sponsor.[123]

[121] FDA/CDRH, Analysis of Premarket Review Times Under the 510(k) Program, July 2011, at http://www.fda.gov/downloads/AboutFDA/CentersOffices/OfficeofMedicalProductsandTobacco/CDRH/CDRHReports/UCM263386.pdf.

[122] See page 169 of 334 at http://www.fda.gov/downloads/MedicalDevices/DeviceRegulationandGuidance/Overview/MedicalDeviceUserFeeandModernizationActMDUFMA/UCM446492.pdf.

[123] See page 11 of 334 at http://www.fda.gov/downloads/MedicalDevices/DeviceRegulationandGuidance/Overview/

Average time to review a 510(k) notification in 2014 was 115 total days—71 FDA days plus 45 submitter days.[124] Average time to review a PMA application in 2014 was 242 total days—190 FDA days plus 51 submitter days.[125]

Section 2201. Priority Review for Breakthrough Devices

Background

Under FFDCA Section 515(d)(5), in order to provide for more effective treatment or diagnosis of life-threatening or irreversibly debilitating human diseases or conditions, the Secretary shall provide review priority for devices representing breakthrough technologies, for which no approved alternatives exist, which offer significant advantages over existing approved alternatives, or the availability of which is in the best interest of the patients.

On April 23, 2014, FDA issued the following draft guidance: *Expedited Access for Premarket Approval Medical Devices Intended for Unmet Medical Need for Life Threatening or Irreversibly Debilitating Diseases or Conditions - Draft Guidance for Industry and Food and Drug Administration Staff*. As indicated in the title, the FDA draft guidance covered only PMA medical devices. FDA issued final guidance on April 13, 2015.[126]

The focus of the guidance is on balancing risks versus benefits for the patient; drafting a Data Development Plan by the medical device sponsor; and collecting postmarket data on a medical device that has received a priority review designation. The expedited review process described in the FDA guidance relies on the use of surrogate endpoints[127] and the collection of postmarket data on the medical device that has received a priority review designation in exchange for lower requirements in the premarket review process, such as less information in the PMA application. According to FDA, the "Expedited Access PMA," or "EAP," program features "earlier and more interactive engagement with FDA staff—including the involvement of senior management and a collaboratively developed plan for collecting the scientific and clinical data to support approval—features that, taken together, should provide these patients with earlier access to safe and effective medical devices."[128]

FDA intends to withdraw approval for a device if the sponsor fails to adhere to the postmarket requirements, such as data collection, or if the postmarket data proves the device is not safe and effective:

MedicalDeviceUserFeeandModernizationActMDUFMA/UCM446492.pdf.

[124] See page 171 of 334 at http://www.fda.gov/downloads/MedicalDevices/DeviceRegulationandGuidance/Overview/MedicalDeviceUserFeeandModernizationActMDUFMA/UCM446492.pdf.

[125] See page 12 of 334 at http://www.fda.gov/downloads/MedicalDevices/DeviceRegulationandGuidance/Overview/MedicalDeviceUserFeeandModernizationActMDUFMA/UCM446492.pdf.

[126] See http://www.fda.gov/downloads/MedicalDevices/DeviceRegulationandGuidance/GuidanceDocuments/UCM393978.pdf. Note that the final FDA Guidance added de novo 510(k) devices. A de novo 510(k), though requiring more data than a traditional 510(k), often requires less information than a PMA application. According to the final guidance, de novo devices "are not eligible for the full scope of the EAP program."

[127] The FDA guidance on pages 23-24 describes a surrogate endpoint as follows: "a surrogate endpoint is not itself a measure of clinical benefit, but is used in trials as a substitute which is reasonably likely to predict clinical benefit, based on epidemiologic, therapeutic, pathophysiologic or other scientific evidence. The types of measurements which may be used as a surrogate endpoint are in vitro laboratory or medical imaging measurements, or physical signs (e.g., blood pressure measurements in trials of antihypertensive therapeutics, as a surrogate for clinical endpoints such as stroke, myocardial infarction, or mortality)."

[128] See http://www.fda.gov/NewsEvents/Newsroom/PressAnnouncements/ucm394294.htm.

> As part of the EAP program, FDA intends to impose postmarket requirements, including requiring post-approval studies as a condition of approval for devices subject to a PMA when applicable.[129] The extent to which FDA will accept certain data to be collected for an EAP Device in the postmarket setting, rather than premarket, is affected by the Agency's current authority to mandate completion of post-approval studies and to withdraw PMA approval for marketed devices for which FDA later determines that there is a lack of a showing of reasonable assurance that the device is safe or effective under the conditions of use prescribed, as well as by the current capabilities of FDA's medical device surveillance system.[130]

Comments on the April 2014 FDA draft guidance questioned FDA's ability to enforce postmarket study requirements and urged the agency and Congress "to evaluate whether FDA has sufficient authorities to promptly withdraw product approval if the necessary data are not promptly collected or suggest that the product benefits do not outweigh risks."[131] One media source stated that, regarding the EAP program, FDA "estimates that, at least in the early stages, on average, about six devices a year may qualify for the program, and the [agency] believes it has the resources available to handle that volume."[132] The estimated six devices would represent about 15% of FDA's total PMA applications in one year. Other comments on the FDA draft guidance questioned whether FDA has sufficient resources to dedicate to the EAP program.[133]

Provision

The provision would add a new Section 515B, "Priority Review for Breakthrough Devices," to Chapter V of the FFDCA. It would "establish a program to provide priority review for devices" representing breakthrough technologies for which no approved alternatives exist, offer significant advantages over existing approved or cleared alternatives, or the availability of which is in the best interest of patients. The provision would allow requests from device sponsors for priority review of not only PMA medical devices, but also 510(k) devices and one other type of regulatory decision involving a medical device.[134] Under the provision, the Secretary would have 60 days to determine whether the request for priority review would be granted. Such requests would be evaluated by a team of experienced FDA staff and managers, chaired by a senior manager. All determinations—either approval or denial of priority review—would require a "substantive summary of the scientific and regulatory rationale" as a "significant decision" under

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[129] 21 CFR 814.82 states: "FDA may impose post-approval requirements in a PMA approval order or by regulation at the time of approval of the PMA or by regulation subsequent to approval." In addition, under Section 522 of the FD&C Act and FDA's implementing regulations at 21 CFR Part 822, FDA may order postmarket surveillance for certain Class III devices.

[130] FDA, Expedited Access for Premarket Approval and De Novo Medical Devices Intended for Unmet Medical Need for Life Threatening or Irreversibly Debilitating Diseases or Conditions, Guidance for Industry and Food and Drug Administration Staff, April 13, 2015, pp. 8-9, http://www.fda.gov/downloads/MedicalDevices/DeviceRegulationandGuidance/GuidanceDocuments/UCM393978.pdf.

Regarding de novo 510(k) devices, the final FDA Guidance on page 9 also stated the following: "FDA would not offer a greater ability to collect postmarket benefit-risk data otherwise typically collected premarket for a de novo request (as we may for a PMA device) because once a de novo request is granted, the product can serve as a predicate for a device that need only demonstrate substantial equivalence for a 510(k) clearance. This would be problematic if we granted a de novo for a device that subsequently was shown not to be safe or effective based on required postmarket data collection."

[131] See http://www.pewtrusts.org/en/about/news-room/news/2014/07/22/pew-comments-to-fda.

[132] David Filmore, "Leap ahead with EAP? FDA proposes new expedited PMA pathway," *The Gray Sheet*, vol. 40, no. 17 (April 28, 2014), pp. 1, 5-6.

[133] See http://center4research.org/public-policy/testimony-briefings-statements/comments-on-expedited-access-for-premarket-approval-medical-devices/.

[134] A petition for classification under Section 513(f)(2) of the FFDCA.

FFDCA Section 517A. The provision would allow a denial of priority review to be reconsidered if a request for reconsideration is made within 30 days of the denial and other specified criteria are met. If a priority review designation for a device is approved by the Secretary, such designation would not be able to be withdrawn by the Secretary because of the subsequent clearance or approval of another "breakthrough" device and therefore specified criteria (i.e., no approved alternatives exist, offer significant advantages over existing approved or cleared alternatives, or the availability of which is in the best interest of patients) are no longer met.

Under the provision, each priority review device would be assigned a team of staff "including a team leader with appropriate subject matter expertise and experience." Oversight of each team would be provided by senior FDA personnel to facilitate the efficient development of the device and the efficient device review. Among other things, FDA would "provide for interactive communication with the device sponsor during the review process," expedite "review of manufacturing and quality systems compliance," and "disclose to the sponsor in advance the topics of any consultation concerning the sponsor's device that [FDA] intends to undertake with external experts or an advisory committee and provide the sponsor an opportunity to recommend such external experts."

FDA would, as appropriate, "coordinate with the sponsor regarding early agreement on a data development plan"; ensure that clinical trial design is as efficient as practicable "through the adoption of shorter or smaller clinical trials, application of surrogate endpoints, and use of adaptive trial designs"; and facilitate "expedited and efficient development and review of the device through utilization of timely postmarket data collection" with regard to PMA applications. FDA would be required to issue guidance on the implementation of the new Section 515B of the FFDCA.

Subtitle M—Medical Device Regulatory Process Improvements

Section 2221. Third-Party Quality System Assessment

Background

Medical device manufacturers must produce their devices in accordance with Good Manufacturing Practice (GMP) requirements, which are described in the Quality System Regulation (QSR).[135] The QSRs require that manufacturers have a quality system for the design, manufacture, packaging, labeling, storage, installation, and servicing of non-exempt finished medical devices intended for commercial distribution in the United States. The regulation requires that various specifications and controls be established for devices; that devices be designed and manufactured under a quality system to meet these specifications; that finished devices meet these specifications; that devices be correctly installed, checked, and serviced; that quality data is analyzed to identify and correct quality problems; and that complaints are processed. Though FDA has identified in QSR the essential elements that a quality system should have, manufacturers have a great deal of leeway to design quality systems that best cover nuances of their devices and the means of producing them.

FDA monitors device problem data and inspects the operations and records of device developers and manufacturers to determine compliance with the GMP/QSR requirements. Establishment

[135] FFDCA §520(f); 21 CFR 820. See also FDA, Medical Devices, Quality System (QS) Regulation/Medical Device Good Manufacturing Practices, http://www.fda.gov/medicaldevices/deviceregulationandguidance/postmarketrequirements/qualitysystemsregulations/.

inspections may be conducted by accredited persons (third parties). For currently marketed devices, a manufacturer is required to notify or submit information to FDA when changes to the design or technical characteristics of a device may affect the performance characteristics of the device. Submission of information to FDA would also be required if a manufacturer seeks to use a device for a new indication, such as to treat a new disease or condition, or a new population, such as pediatric use. Examples of such submissions include a 30-day notice, used by a manufacturer to request modifications in manufacturing procedures or methods of manufacture affecting the safety and effectiveness of a device, and a PMA Supplement, required when a manufacturer requests approval of a change in aspects of an approved device, such as its design, specifications, or labeling. FDA collects a user fee from a manufacturer that files such a submission requesting agency review of a change in the design or performance of a device.

Provision

The provision would add to the FFDCA a new Section 524B, "Third-Party Quality System Assessment." The Secretary would be required to establish a third-party quality system assessment program to accredit persons to assess a manufacturer's quality system and ensure the safety and effectiveness of certain specified changes made to an in-scope device. In-scope device is defined as "a device within the scope of devices agreed to by the requestor [manufacturer] and the accredited person for purposes of a request for certification." Under the provision, the manufacturer has the option to select an accredited person who would be required to assess and, if applicable, certify that a device maker's quality system meets criteria to be issued by the Secretary in a guidance document. The Secretary would be required to rely on such certifications to determine the safety and effectiveness, or substantial equivalence, of device-related changes made to in-scope devices. Certification would be in lieu of compliance with certain specified FDA submission requirements: a 30-day notice;[136] a Special PMA supplement;[137] and certain device-related changes that require a 510(k) premarket notification but do not alter the intended use of the changed device or the fundamental technology of such device.

Under the provision, the process and qualifications for accreditation of persons and accreditation renewal currently in place would be required to apply to the new FFDCA Section 524A.[138] Initiation of assessment services would not begin until after the Secretary issues final guidance. Compensation for accredited persons would be determined by agreement between the accredited person and the requestor and paid by the requestor. The requestor would be required to select an accredited person from a list published by the Secretary.[139]

The provision would require the Secretary to issue draft guidance on the criteria for certification of a quality system not later than 12 months after enactment, and final guidance not later than 12 months after draft guidance is issued. The guidance would be required to contain evaluative criteria to be used by an accredited person to assess and, as applicable, certify a manufacturer's quality system with respect to in-scope devices, as well as criteria for accredited persons to waive or exempt the certification criteria. An assessment summary would be required to be submitted to the Secretary within 30 days of the assessment and would include, as applicable, certification that the requestor has satisfied the Secretary's guidance criteria for quality system certification for the in-scope device and any waivers or exemptions applied by the accredited person.

[136] A 30-day notice is defined in FFDCA Section 515(d)(6).

[137] A Special PMA supplement is described in 21 CFR §814.39(d).

[138] FFDCA Section 704(g).

[139] FFDCA Section 704(g)(4).

Under the provision, the Secretary would have 30 days to review the assessment summary. The summary would be required to be deemed accepted by the Secretary on the 30th day unless the Secretary, by written notice to the accredited person, deems the certification to be provisional beyond the 30-day period, suspended pending further review by the Secretary, or otherwise qualified or cancelled. If after, for example, further information is submitted to support the certification and the Secretary determines that the certification is acceptable, the accredited person would be notified in writing. Certification would remain in effect for two years and may be renewed. Certification would be revoked by written notification to the manufacturer if the Secretary determines the quality system no longer meets the certification criteria with respect to in-scope devices as specified by the Secretary in final guidance. In this case, the manufacturer would be required to comply with any necessary submission requirements (e.g., 30-day notice, Special PMA supplement, 510(k) premarket notification) for any device-related changes. The manufacturer would be eligible to seek recertification of its quality system.

Manufacturers who have received certification under this new Section 524B—and have made device-related changes to in-scope devices, without prior submission of a 510(k) notification—would be required to ensure that an annual summary report is submitted to the Secretary by the accredited person. The report would describe the changes made to the in-scope device and the effective dates of such changes. Manufacturers who have received certification under this new Section 524B—and have made device-related changes to in-scope devices, without prior submission of a 30-day notice or Special PMA Supplement—would be required to notify the Secretary of such changes in the manufacturer's next periodic report.[140] The report would describe the changes made to the in-scope device and the effective dates of such changes. All these reports would be required to be used by the Secretary for an evaluation of the third-party quality system assessment program to be completed by January 31, 2021. Within one year of completing this evaluation, the Secretary would be required to issue on FDA's website a report of the evaluation's findings including recommendations on continuation or expansion of the program. Section 524B would sunset on October 1, 2022.

Section 2222. Valid Scientific Evidence

Background

Evidence submitted to FDA, in a PMA or a 510(k) notification, regarding the use of a medical device in the treatment or diagnosis of disease is obtained from clinical trials, which are research studies involving patients. The design of research studies affects the strength of their evidence, and ranges from what is often referred to as the gold standard—the randomized controlled clinical trial—to case reports or case histories, which are observational studies of one or more patients. "Observational studies are often considered inferior to randomized trials as, in some cases, they have been shown to overestimate treatment effects."[141] In a randomized controlled clinical trial, patients are randomly assigned to two or more groups. One group receives the intervention (such as a new treatment), while the control group receives current therapy or placebo. Randomization ensures that any patient characteristics that might affect the outcome will be roughly equal across each group in the study. Any difference in outcomes between the groups is then due to the intervention.

[140] 21 CFR §814.84(b).

[141] Ravi Thadhani, "Chapter 14: Formal trials versus observational studies," in *Fabry Disease: Perspectives from 5 Years of FOS*, ed. A. Mehta, M. Beck, G. Sunder-Plassmann (Oxford: Oxford PharmaGenesis, 2006).

Under FFDCA Section 513, the effectiveness of a device is to be determined on the basis of well-controlled investigations, including one or more clinical investigations where appropriate. However, an exception is allowed if the Secretary determines that "valid scientific evidence" exists, other than a well-controlled clinical trial that is sufficient to determine the effectiveness of a device.

Provision

The provision would amend FFDCA Section 513(a)(3)(B) and would include as "valid scientific evidence ... evidence described in well-documented case histories, including registry data, that are collected and monitored under an acceptable protocol; studies published in peer-reviewed journals; and, data collected in countries outside the United States." The Secretary would be allowed to request the underlying data for a study published in a peer-reviewed journal if that would be the least burdensome means, to the manufacturer, of evaluating device effectiveness or substantial equivalence. The Secretary would need to furnish a written rationale for requesting the underlying data, and if the data are unavailable, the study would be considered "to be a part of the totality of the evidence with respect to the device, as the Secretary determines appropriate."

Section 2223. Training and Oversight in Least Burdensome Appropriate Means Concept

Background

Section 205 of the Food and Drug Administration Modernization Act of 1997 (FDAMA, P.L. 105-115) amended Section 513 of the FFDCA, adding two provisions commonly referred to as the "Least Burdensome Provisions." The two provisions stipulate that FDA consider the "least burdensome" data or information "necessary" to demonstrate a reasonable assurance of device effectiveness in a PMA application or substantial equivalence to predicate devices with differing technological characteristics in certain 510(k) notifications. Here are the two provisions:

> **Section 513(a)(3)(D)(ii)** Any clinical data, including one or more well-controlled investigations, specified in writing by the Secretary for demonstrating a reasonable assurance of device effectiveness shall be specified as a result of a determination by the Secretary that such data are necessary to establish device effectiveness. The Secretary shall consider, in consultation with the applicant, the least burdensome appropriate means of evaluating device effectiveness that would have a reasonable likelihood of resulting in approval.
>
> **Section 513(i)(1)(D)** Whenever the Secretary requests information to demonstrate that devices with differing technological characteristics are substantially equivalent, the Secretary shall only request information that is necessary to making substantial equivalence determinations. In making such requests, the Secretary shall consider the least burdensome means of demonstrating substantial equivalence and request information accordingly.

FDA published final guidance on the least burdensome provisions on October 4, 2002.[142] The agency has posted on its website activities related to implementation of the least burdensome provision, including training for staff and advisory panels.[143]

[142] FDA, The Least Burdensome Provisions of the FDA Modernization Act of 1997: Concept and Principles; Final Guidance for FDA and Industry, October 4, 2002, http://www.fda.gov/RegulatoryInformation/Guidances/ucm085994.htm.

[143] FDA, Medical Devices, The Least Burdensome Provisions - Activities Related to Implementation,

Provision

The provision would amend FFDCA Section 513 by adding a new subsection (j), "Training and Oversight in Least Burdensome Appropriate Means Concept." Each FDA employee involved in the review of PMA applications or 510(k) notifications, including supervisors, would be required to receive training on the "meaning and implementation of the least burdensome appropriate means concept." The Secretary would be required to issue draft guidance, no later than 12 months after enactment, updating the October 4, 2002, final guidance on the least burdensome provisions. In developing the draft guidance, the Secretary would be required to hold a meeting of stakeholders "to ensure a full record to support the publication of such document." Under the provision, 18 months after the draft guidance is issued, the FDA ombudsman responsible for device premarket review would be required to conduct an audit of the least burdensome training, including "interviews with a representative sample of persons from industry regarding their experience in the device premarket review process."

Regarding PMA applications, the provision would amend FFDCA Section 515(c), adding a new paragraph that would require the Secretary to "consider the least burdensome appropriate means necessary to demonstrate device safety and effectiveness, and request information accordingly." The provision defines the term *necessary* to mean "the minimum required information that would support a determination by the Secretary that an application provides a reasonable assurance of the safety and effectiveness of the device."

Section 2224. Recognition of Standards

Background

Under the Medical Device Amendments of 1976 (MDA, P.L. 94-295), FDA was required to classify all medical devices into one of three classes. Congress provided definitions for the three classes—Class I, Class II, Class III—based on the risk (low-, moderate-, and high-risk, respectively) posed by the device to patients.[144] Device classification determines the type of regulatory requirements that a manufacturer must follow. General controls are the minimum regulations that apply to all FDA-regulated medical devices.[145]

Class II devices are those under current law "which cannot be classified as Class I because the general controls by themselves are insufficient to provide reasonable assurance of safety and effectiveness of the device."[146] Although Class II includes devices that pose a moderate risk to patients, only some have information or special controls available to reduce or mitigate risk. Special controls include special labeling requirements, premarket data requirements, postmarket

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http://www.fda.gov/MedicalDevices/DeviceRegulationandGuidance/Overview/MedicalDeviceProvisionsofFDAModernizationAct/ucm136685.htm#7.

[144] FFDCA §513(a)(1); see also 21 CFR §860.3(c). The agency has developed classifications for over 1,700 distinct types of devices and grouped them into 16 classification panels, such as "cardiovascular devices" or "ear, nose, and throat devices." FDA, Medical Devices, Classify Your Medical Device, December 3, 2012, http://www.fda.gov/MedicalDevices/DeviceRegulationandGuidance/Overview/ClassifyYourDevice/default.htm.

[145] General controls include five elements: (1) establishment registration—such as manufacturers, distributors, repackagers and relabelers, and foreign firms; (2) device listing—listing with FDA of all devices to be marketed; (3) good manufacturing practices (GMP)—manufacturing of devices in accordance with the Quality Systems Regulation (QSR); (4) labeling—labeling of devices or in vitro diagnostic products; and (5) premarket notification—submission to FDA of a premarket notification 510(k).

[146] FFDCA §513(a)(1)(B).

surveillance, patient registries, guidelines, and performance standards.[147] Only about "15% of all device types classified in Class II are subject to special controls."[148] According to a 2011 report by the Institute of Medicine, this is because "FDA has not promulgated performance standards or special controls for the vast majority of types of Class II devices."[149] Although "FDA has procedures for developing, adopting, and implementing guidance and standards," it has been "persistently hindered in fully developing those materials by a lack of or limitations on human, fiscal, and technologic resources and capabilities."[150] According to a 1988 Government Accountability Office (GAO) report, FDA estimated that "40 staff-*years* (not staff-hours) would be required to develop a single performance standard."[151]

In response to agency problems with developing performance standards, the Safe Medical Devices Act of 1990 (P.L. 101-629) simplified the process of establishing performance standards for Class II devices and authorized the use of alternative restrictions, called special controls, at the agency's discretion. The Food and Drug Administration Modernization Act of 1997; (P.L. 105-115) allowed FDA to recognize an appropriate performance standard developed by a U.S. or international organization involved in standard development.[152]

Provision

The provision would amend FFDCA Section 514(c) by adding two new subparagraphs and two new paragraphs. Under the provision, any person would be able to submit to FDA a request for the agency to recognize "all or part of an appropriate standard established by a nationally or internationally recognized standard organization." The Secretary would be required to make a determination to recognize all, part, or none of the standard within 60 days, with a written response indicating the rationale for such a determination, "including the scientific, technical, regulatory, or other basis for such determination." The response and rationale for recognition would be required to be made publically available.

Under the provision, the Secretary would be required to provide to all FDA employees who review premarket submissions for devices "periodic training on the concept and use of recognized standards for purposes of meeting a premarket submission requirement or other applicable requirement." The provision would require the Secretary to publish draft guidance, identifying the principles for recognizing standards, not later than 12 months after enactment, and final guidance not later than 12 months after the public comment period has ended on the draft guidance.

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[147] See FDA, General and Special Controls, last updated on June 26, 2014, http://www.fda.gov/MedicalDevices/DeviceRegulationandGuidance/Overview/GeneralandSpecialControls/default.htm#special.

[148] Institute of Medicine, *Medical Devices and the Public's Health: The FDA 510(k) Clearance Process at 35 Years*, Washington, DC, July 2011, p. 40.

[149] Ibid., p. 3.

[150] Ibid., p. 5.

[151] Ibid., p. 184; and GAO, *Medical Devices: FDA's 510(k) operations could be improved. Report to the chairman, Subcommittee on Health and the Environment, Committee on Energy and Commerce, House of Representatives* (PEMD-88-14), p. 4.

[152] FFDCA §514(c)(1)(A).

Section 2225. Easing Regulatory Burden with Respect to Certain Class I and Class II Devices

Background

Under the Medical Device Amendments of 1976 (MDA), the manufacturer of a new product would submit a notice to the FDA 90 days prior to marketing. This type of premarket review is known as a 510(k) notification, after the section of the MDA requiring that FDA be notified of the new product before it is marketed. The Food and Drug Administration Modernization Act of 1997 (P.L. 105-115) eliminated the requirement of a 510(k) submission for most Class I devices and a small proportion of Class II device types. A 2009 GAO study found that 67% of device types were exempt from premarket review—Class I devices made up 95% and Class II devices made up 5% of these exempt devices.[153]

On July 1, 2015, FDA released guidance that exempts 120 types of medical devices from premarket notification requirements; draft guidance was issued on August 1, 2014.[154] The 120 devices are primarily Class II but also include a few Class I devices and some pre-amendment (pre-MDA) unclassified devices. The guidance states that until "publication of a final rule or order exempting these devices from 510(k), FDA does not intend to enforce compliance with 510(k) requirements for these devices. FDA does not expect manufacturers to submit 510(k)s for these devices during this time period."[155]

Provision

The provision would amend FFDCA Section 510(l) and would require the Secretary, within 120 days of enactment, to identify and publish in the *Federal Register* "any type of class I device that the Secretary determines no longer requires a report under subsection (k) to provide reasonable assurance of safety and effectiveness." Upon publication, each type of Class I device so identified would be exempt from the 510(k) requirement and the "classification regulation applicable to each such type of device" would be deemed amended to incorporate the exemption.

Similarly, the provision would amend FFDCA Section 510(m) and would require the Secretary, within 60 days of enactment, to publish in the *Federal Register* "a list of each type of class II device that the Secretary determines no longer requires a report under subsection (k) to provide reasonable assurance of safety and effectiveness." The Secretary would be required to provide a 60-day public comment period after publication of such a list.

Not later than 180 days after enactment, the Secretary would be required to publish in the *Federal Register* a list representing the final determination on the types of Class II devices that no longer would require a 510(k) notice prior to marketing. Upon publication of the final list, each type of Class II device so listed would be exempt from the 510(k) requirement and the "classification regulation applicable to each such type of device" would be deemed amended to incorporate the exemption.

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[153] U.S. Government Accountability Office, Medical Devices: FDA Should Take Steps to Ensure That High-Risk Device Types Are Approved through the Most Stringent Premarket Review Process, GAO-09-190, January 2009, p. 9, http://www.gao.gov/assets/290/284882.pdf.

[154] FDA Center for Devices and Radiological Health, *Intent to Exempt Certain Unclassified, Class II, and Class I Reserved Medical Devices from Premarket Notification Requirements*, Guidance for Industry and FDA Staff, July 1, 2015, http://www.fda.gov/ucm/groups/fdagov-public/@fdagov-meddev-gen/documents/document/ucm407292.pdf.

[155] Ibid., p. 4-5.

Section 2226. Advisory Committee Process

Background

FDA advisory committees "provide independent expert advice to the agency on a range of complex scientific, technical, and policy issues. An advisory committee meeting also provides a forum for a public hearing on important matters. Although advisory committees provide recommendations to FDA, FDA makes the final decisions."[156]

In April 2015, FDA issued draft guidance entitled "Procedures for Meetings of the Medical Devices Advisory Committee."[157] Once final, the draft guidance will replace two earlier FDA guidance documents.[158] The draft guidance provides information on the processes associated with Medical Devices Advisory Committee panel meetings, such as types of panel meetings, information exchange for panel meetings, and conduct of panel meetings.[159] The Medical Devices Advisory Committee includes 17 different advisory panels, which address topics in various specialty areas.[160] FDA may refer a matter to a particular device panel for advice on a premarket submission if the submission is, for example, of significant public interest or is highly controversial. The agency may also ask a panel to provide advice on regulatory actions, such as device classification, or general scientific matters "that are related to a device type or a general topic that is relevant to medical device safety and effectiveness."[161] Under current law, the advisory panels are composed of

> persons who are qualified by training and experience to evaluate the safety and effectiveness of the devices to be referred to the panel and who, to the extent feasible, possess skill in the use of, or experience in the development, manufacture, or utilization of, such devices. The Secretary shall make appointments to each panel so that each panel shall consist of members with adequately diversified expertise in such fields as clinical and administrative medicine, engineering, biological and physical sciences, and other related professions. In addition, each panel shall include as nonvoting members a representative of consumer interests and a representative of interests of the device manufacturing industry. Scientific, trade, and consumer organizations shall be afforded an opportunity to nominate individuals for appointment to the panels.[162]

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[156] FDA, *Guidance for the Public and FDA Staff on Convening Advisory Committee Meetings*, Draft Guidance, August 2008, http://www.fda.gov/downloads/RegulatoryInformation/Guidances/UCM125651.pdf.

[157] FDA, *Procedures for Meetings of the Medical Devices Advisory Committee*, April 1, 2015, http://www.fda.gov/downloads/MedicalDevices/DeviceRegulationandGuidance/GuidanceDocuments/UCM440348.pdf.

[158] The two documents are *Guidance on Amended Procedures for Advisory Panel Meetings*, http://www.fda.gov/MedicalDevices/DeviceRegulationandGuidance/GuidanceDocuments/ucm073722.htm; and, *Panel Review of Premarket Approval Applications #P91-2 (blue book memo)*, http://www.fda.gov/MedicalDevices/DeviceRegulationandGuidance/GuidanceDocuments/ucm081363.htm.

[159] The draft guidance does not cover meetings of the Medical Device Dispute Resolution Panel.

[160] The advisory panels are (1) Anesthesiology and Respiratory Therapy Devices; (2) Circulatory System Devices; (3) Clinical Chemistry and Clinical Toxicology Devices; (4) Dental Products; (5) Ear, Nose, and Throat Devices; (6) Gastroenterology and Urology Devices; (7) General and Plastic Surgery Devices; (8) General Hospital and Personal Use Devices; (9) Hematology and Pathology Devices; (10) Immunology Devices; (11) Microbiology Devices; (12) Molecular and Clinical Genetics; (13) Neurological Devices; (14) Obstetrics and Gynecology Devices; (15) Ophthalmic Devices; (16) Orthopedic and Rehabilitation Devices; and (17) Radiological Devices.

[161] FDA, *Procedures for Meetings of the Medical Devices Advisory Committee*, April 1, 2015, p. 3, http://www.fda.gov/downloads/MedicalDevices/DeviceRegulationandGuidance/GuidanceDocuments/UCM440348.pdf.

[162] FFDCA Section 513(b)(2).

Provision

The provision would amend FFDCA Section 513(b)(5) by adding two new subparagraphs that would require the Secretary to ensure that there is "adequate expertise" on a device classification panel including by giving "due consideration to the recommendations" of the device manufacturer "on the expertise needed among the voting members of the panel," among other things. The Secretary would be required to ensure this when "a device is specifically the subject of review by a classification panel." The provision would define "adequate expertise" to mean that the classification panel reviewing a premarket submission would include "two or more voting members, with a specialty or other expertise clinically relevant to the device under review, and at least one voting member who is knowledgeable about the technology of the device."

The provision would amend FFDCA Section 513(b)(6) regarding the panel review process and participation in the panel meeting, adding that the device manufacturer, or its representative, would be allowed time during a panel meeting to correct misstatements of fact or provide clarifying information, subject to the discretion of the panel chairperson.

The provision would strike subparagraph (B) in FFDCA Section 513(b)(6) and replace it with a similar subparagraph, delineating that adequate time for presentations would be required to be provided to the device manufacturer and the Secretary, and would add that the panel would be allowed to pose questions to the representative of the manufacturer and consider the responses in the panel's review of the device.

Section 2227. Humanitarian Device Exemption Application

Background

The Humanitarian Device Exemption (HDE) was intended to encourage the development of devices that aid in the treatment and diagnosis of diseases or conditions that affect fewer than 4,000 individuals in the United States per year.[163] An HDE application is similar to a PMA but is exempt from the effectiveness requirements to encourage manufacturers to develop devices for these small markets.

Provision

The provision would amend FFDCA Section 520(m) and would allow an HDE to be granted to treat and diagnose diseases or conditions that affect not more than 8,000 individuals in the United States. Within 18 months of enactment, the Secretary, acting through the Commissioner of the FDA, would be required to publish draft guidance that "defines the criteria for establishing 'probable benefit'" when evaluating whether the health benefit of an HDE device outweighs the risk of injury or illness from using such a device.

Section 2228. CLIA Waiver Study Design Guidance for In Vitro Diagnostics

Background

The Clinical Laboratory Improvement Amendments (CLIA) of 1988 provide CMS with authority to regulate clinical laboratories to ensure the accuracy of test results, given that these results drive clinical decisionmaking.[164] CLIA requires laboratories to receive certification before they are

[163] The Humanitarian Device Exemption was authorized by the Safe Medical Devices Act of 1990 (P.L. 101-629).

[164] PHSA §353; 42 U.S.C. §263a.

allowed to carry out clinical laboratory testing on a human sample. CLIA certification is based on the level of complexity of testing that a laboratory is performing, graded as low, moderate, or high. FDA is responsible for categorizing clinical laboratory tests according to their level of complexity.[165] Laboratories that perform only low-complexity tests (called waived tests) receive a certificate of waiver (COW) from CMS. Conversely, only laboratories certified to do so may perform moderate- and high-complexity tests.

FDA determines whether a test is waived (i.e., low-complexity) or not based on information submitted about the test by the manufacturer, and has issued guidance to support the manufacturer's submission of this information.[166] Under current law, waived tests are those "that have been approved by FDA for home use or that, as determined by the Secretary, are simple laboratory examinations and procedures that have an insignificant risk of an erroneous result."[167] The guidance recommends approaches for demonstrating that a test is both "simple" and has "an insignificant risk of an erroneous result." Demonstrating the latter includes showing that a test's accuracy is comparable to a method whose accuracy has already been established and documented. (Section V of the guidance document addresses approaches to demonstrating accuracy.)

Provision

The provision would require the Secretary, not later than 12 months after enactment, to publish draft guidance that revises Section V of the current guidance, including providing guidance on the appropriate use of comparable performance between a waived user and a moderately complex laboratory user to demonstrate accuracy. Not later than 12 months after the comment period closes for the draft guidance, the Secretary would be required to publish final revised guidance.

Subtitle N—Sensible Oversight for Technology Which Advances Regulatory Efficiency

Background

The Food and Drug Administration Safety and Innovation Act directed FDA, the Federal Communications Commission (FCC), and the Office of the National Coordinator for Health Information Technology (ONC) to recommend a "risk-based regulatory framework pertaining to health information technology, including mobile medical applications, that promotes innovation, protects patient safety, and avoids regulatory duplication."[168] The recommendations, published in an April 2014 report, proposed separating products into three categories: (1) products that perform administrative functions, such as billing and practice-management software; (2) products that perform health management functions, such as medication management and most clinical decision support; and (3) products that perform medical device functions, such as disease

[165] See FDA, "CLIA Categorizations," http://www.fda.gov/medicaldevices/deviceregulationandguidance/ivdregulatoryassistance/ucm393229.htm.

[166] FDA, "Guidance for Industry and FDA Staff: Recommendations for Clinical Laboratory Improvement Amendments of 1988 (CLIA) Waiver Applications for Manufacturers of In Vitro Diagnostic Devices," Center for Devices and Radiological Health, January 30, 2008, http://www.fda.gov/downloads/MedicalDevices/DeviceRegulationandGuidance/GuidanceDocuments/ucm070890.pdf.

[167] PHSA §353(d)(3), "Requirements for Certificate of Waiver"; 42 U.S.C. §263a(d)(3).

[168] P.L. 112-144, §618, July 9, 2012.

detection and diagnosis software, and applications that control the operation of other devices.[169] The report recommended limiting FDA oversight to products in the third category.

In a separate action, FDA in February 2015 released updated guidance on a risk-based approach to regulating mobile medical applications.[170] First, the agency provided examples of mobile applications that do not meet the statutory definition of a medical device and so are not subject to its regulatory authority. They include applications used to automate general office operations, and applications intended for general patient education. Second, FDA gave examples of mobile applications that may meet the definition of a medical device but for which the agency intends to exercise enforcement discretion—meaning that it does not intend to enforce the FFDCA's requirements—because the applications pose a lower risk to the public. This category includes clinical decision support software and applications that use patient characteristics to provide patient-specific screening and counseling recommendations.

Finally, FDA provided examples of mobile applications that are the focus of the agency's regulatory oversight. These applications meet the definition of a medical device, and they pose a risk to patient safety if they do not function as intended. Examples include applications that connect to an existing device to control its operation, function, or energy source, and applications used in active patient monitoring or analyzing patient-specific medical data from a connected device.

Section 2241. Health Software

Provision

The provision would amend Section 201 of the FFDCA ("Definitions") to define "health software" as software that is intended for use (1) in administrative or operational support or the processing and maintenance of financial records; (2) in workflow and related recordkeeping; (3) in the transfer, aggregation, conversion, storage, management, or transmission of data—provided the software is not used in active patient monitoring or in controlling the functions of a device to which it is connected; (4) to organize and present health and wellness information; and (5) to analyze information and provide recommended options, both general and patient-specific, for the prevention, diagnosis, treatment, cure, or mitigation of a particular disease or condition (i.e., clinical decision support).

Health software does not include software that (1) through use of an in vitro diagnostic device, acquires, processes, or analyzes an image or physiological signal; (2) is intended for use with, or to support, supplement, or augment, one or more parent devices; (3) is an integral part of a device necessary to support the use of a device; or (4) is used in the manufacture and transfusion of blood products.

[169] Food and Drug Administration, *FDASIA Health IT Report, Proposed Strategy and Recommendations for a Risk-Based Framework*, April 2014, http://www.fda.gov/downloads/AboutFDA/CentersOffices/OfficeofMedicalProductsandTobacco/CDRH/CDRHReports/UCM391521.pdf.

[170] Food and Drug Administration, *Mobile Medical Applications: Guidance for Industry and Food and Drug Administration Staff*, February 9, 2015, http://www.fda.gov/downloads/MedicalDevices/UCM263366.pdf.

Section 2242. Applicability and Inapplicability of Regulation

Provision

The provision would add a new Section 524B of the FFDCA ("Health Software") to prohibit FDA from regulating health software. The prohibition would not apply to health software that provides patient-specific clinical decision support and that poses a significant risk to patient safety, based on a determination of the Secretary made according to certain specified criteria.

The Secretary would be required to review existing health software regulations and guidance, and would have the authority to implement a new regulatory framework by modifying existing regulations and guidance or by issuing new regulations or guidance. The Secretary would be permitted to take such actions by administrative order published in the *Federal Register* following publication of a proposed order. The Secretary would be required to consult with stakeholders before issuing regulations, an administrative order, or guidance.

Section 2243. Exclusion from Definition of Device

Provision

The provision would amend the definition of "device" in Section 201(h) of the FFDCA ("Definitions") to exclude health software, other than software that provides patient-specific clinical decision support and that poses a significant risk to patient safety.

Subtitle O—Streamlining Clinical Trials

Background

The HHS Human Subject Regulations are a core set of federal standards for protecting human subjects in HHS-sponsored research.[171] These regulations are commonly referred to as the Common Rule because the same requirements have been adopted by many other federal departments and agencies, who are to apply the regulations to the research they fund. Under the Common Rule, research protocols must be approved by an Institutional Review Board (IRB) to ensure that the rights and welfare of the research subjects are protected.[172] The rule lists several criteria for IRB approval, including the requirement that researchers obtain the informed consent of their research subjects.[173] In addition, it sets out the types of information that must be provided to prospective research subjects during the informed consent process, including an explanation of the purpose of the research, a description of the research procedures, and a description of the risks and benefits of the research.[174] An IRB may decide to waive the informed consent requirement if it determines that (1) the research poses no more than minimal risk to the subjects, (2) the waiver will not adversely affect the rights and welfare of the subjects, and (3) the research is not practicable without a waiver.[175]

[171] 45 C.F.R. Part 46, Subpart A.

[172] 45 C.F.R. §46.109.

[173] 45 C.F.R. §46.111(a)(4).

[174] 45 C.F.R. §46.116(a).

[175] 45 C.F.R. §46.116(d).

HHS has promulgated additional protections for certain vulnerable populations involved in research. Those groups include pregnant women, human fetuses, and neonates; prisoners; and children.[176]

FDA has issued its own set of Human Subject Regulations, which are similar, but not identical, to the Common Rule.[177] FDA applies these regulations to all the research it regulates, including clinical trials of new drugs and medical devices, regardless of the source of funding for the research. Humanitarian use devices, which are approved by FDA for diagnosing or treating diseases or conditions that affect fewer than 4,000 individuals in the United States each year, can be used in a facility only after a local IRB has approved their use in that facility, except in certain emergency situations.[178]

In July 2011, HHS published an advance notice of proposed rulemaking (ANPRM) requesting public comment on a broad range of amendments to the Common Rule, with the goal of "enhancing the effectiveness of the research oversight system by improving the protections for human subjects while also reducing burdens, delays, and ambiguity for investigators and human subjects."[179] HHS sought comments on such changes as refining the current risk-based regulatory framework, coordinating IRB review of multisite studies, and harmonizing the regulations and guidance of different agencies. HHS has yet to publish a proposed rule based on the extensive comments it received on the ANPRM.

Section 2261. Protection of Human Subjects in Research; Applicability of Rules

Provision

The provision instructs the Secretary, to the extent possible, to harmonize differences between the HHS Human Subject Regulations and the FDA Human Subject Regulations. The Secretary would be required to modify the HHS and FDA regulations and associated rules for vulnerable populations to reduce regulatory duplications and unnecessary delays; accommodate multisite and cooperative research projects; incorporate local consideration, community values, and mechanisms to protect vulnerable populations; and ensure that human research that is subject to the HHS regulations or to the FDA regulations may use joint or shared IRB review, an independent IRB, or some other IRB arrangement to avoid duplication of effort.

Within 36 months of enactment, the Secretary, in consultation with stakeholders, would be required to issue regulations and guidance as necessary to implement this provision and help facilitate the broader use of single, central, or lead IRBs. Such regulations and guidance must address arrangements to avoid regulatory duplication and unnecessary delays; for example, by delineating the roles of IRBs in multisite or cooperative studies and by standardizing informed consent and other processes and legal documents. Concerns about regulatory and legal liability contributing to research sponsors' decisions to rely on local IRBs must also be addressed. The Secretary would be required to complete the harmonization process not later than 36 months after enactment, and would have to submit a progress report to Congress within 24 months of enactment. In addition, within 12 months of enactment, the Secretary would be required to

[176] 45 C.F.R. Part 46, Subparts B (pregnant women, fetuses, neonates), C (prisoners), and D (children).

[177] 21 C.F.R. Parts 50, 56, 312, and 812.

[178] FFDCA §520(m)(4).

[179] Department of Health and Human Services, Food and Drug Administration, "Human Subject Research Protections: Enhancing Protections for Research Subjects and Reducing Burden, Delay, and Ambiguity for Investigators," 76 *Federal Register* 44512, July 25, 2011.

finalize the Draft NIH Policy on Use of a Single Institutional Review Board for Multi-Site Research.[180]

Section 2262. Use of Non-Local Institutional Review Boards for Review of Investigational Device Exemptions and Human Device Exemptions

Provision

The provision would amend Section 520 of the FFDCA by removing the word "local" in all references to local IRBs, including in the stipulation that an approved humanitarian use device can be used in a facility only after a local IRB has approved such use, except in certain emergency situations. Within 12 months of enactment, the Secretary would be required to revise or issue regulations or guidance, as necessary, to carry out these amendments.

Section 2263. Alteration or Waiver of Informed Consent for Clinical Investigations

Provision

The provision would amend Section 520(g) of the FFDCA ("Exemption for Devices for Investigational Use") to waive the informed consent requirement for individuals participating in the clinical trial of a medical device if the trial poses no more than minimal risk to the participants and includes appropriate safeguards to protect their rights, safety, and welfare.

The provision also would amend Section 505(i) of the FFDCA (regarding the investigational use of drugs) to waive the informed consent requirement for individuals participating in the clinical trial of a drug if the trial poses no more than minimal risk to the participants and includes appropriate safeguards to protect their rights, safety, and welfare.

Subtitle P—Improving Scientific Expertise and Outreach at FDA

Section 2281. Silvio O. Conte Senior Biomedical Research Service

Background

The Commissioned Corps of the U.S. Public Health Service (USPHS) is a uniformed service with the mission "to protect, promote, and advance the health and safety of our Nation."[181] The Ready Reserve Corps of the USPHS was authorized by the ACA to assist full-time Commissioned Corps personnel on short notice as needed. The Silvio O. Conte Senior Biomedical Research Service (the Service), established by PHSA Section 228, may include up to 500 members who are authorized in addition to members of the commissioned Regular Corps, the Reserve Corps, and in the Senior Executive Service otherwise authorized. The authority does not require that the number of members in those three entities be reduced to offset the number of Service members.

[180] Department of Health and Human Services, National Institutes of Health, "Announcement of a Draft NIH Policy on the Use of a Single Institutional Review Board for Multi-Site Research," 80 *Federal Register* 511, January 6, 2015.

[181] Commissioned Corps of the U.S. Public Health Service, *Mission and Core Values*, http://www.usphs.gov/aboutus/mission.aspx.

The Secretary appoints as Service members doctoral-level individuals who are outstanding in the fields of biomedical research or clinical research evaluation. The appointments are made "without regard to the provisions of title 5 regarding appointment." Rate of pay may not exceed that for Level I of the Executive Schedule unless approved by the President. The Secretary may contribute an amount up to 10% of a Service member's pay to that person's already established retirement system at the institution of higher education at which the member had been employed.

Provision

The provision would rename the Service as the Silvio O. Conte Senior Biomedical Research and Biomedical Product Assessment Service. It would specify that the purpose of the Service "is to recruit and retain competitive and qualified scientific and technical experts outstanding in the field of biomedical research, clinical research evaluation, and biomedical product assessment." The provision would eliminate mention of a specified number of authorized service members (up to 500 members in current law). It would also eliminate the requirement that the Service members be authorized in addition to those in the three entities specified in current law. It would not be construed to require the Secretary to reduce the number of employees serving in other employment systems (instead of, as currently, the three specified entities) to offset the number of employees in the Service.

The provision would authorize the Secretary to appoint a member who holds "a master's level degree in engineering, bioinformatics, or a related or emerging field," expanding the current requirement for doctoral-level members. It would change the pay rate limit to that of the President and would eliminate the Secretary's authority to contribute to a member's retirement system at an institution of higher education.

The provision would require that the Secretary publish a report on the HHS website regarding the changes to the Service and whether they "have improved the ability of the Food and Drug Administration to hire and retain qualified experts to fulfill obligations specified under user fee agreements."

Section 2282. Enabling FDA Scientific Engagement

Background

Following allegations of misspent funds during a 2010 General Services Administration meeting held in Las Vegas, the Office of Management and Budget imposed restrictions on conference travel for federal employees in a May 11, 2012, memorandum.[182] The memorandum directed agencies, beginning in FY2013, to spend at least 30% less than what was spent in FY2010 on travel expenses, and stated that agencies "must maintain this reduced level of spending each year through FY 2016." Senior level agency approval is required for all conferences sponsored by an agency where the conference expenses to the agency are over $100,000. Agencies are prohibited from spending more than $500,000 on a single conference. However, this restriction may be waived if the agency head "determines that exceptional circumstances exist whereby spending in excess of $500,000 on a single conference is the most cost-effective option to achieve a compelling purpose."[183]

[182] Promoting Efficient Spending to Support Agency Operations, http://www.whitehouse.gov/sites/default/files/omb/memoranda/2012/m-12-12.pdf.

[183] Ibid.

Provision

The provision would express the sense of Congress that participation in or sponsorship of scientific conferences and meetings is essential to the mission of FDA.

Section 2283. Reagan-Udall Foundation for the Food and Drug Administration

Background

FFDCA Section 770, as added by the Food and Drug Administration Amendments Act of 2007 (FDAAA, P.L. 110-85), created the Reagan-Udall Foundation for the Food and Drug Administration, a nonprofit organization "to advance the mission" of FDA. Its duties cover activities such as identifying and then prioritizing unmet needs; awarding grants or entering into other agreements with scientists, academic consortia, public-private partnerships, nonprofit organizations, and industry; holding meetings and publishing information and data for use by FDA and others; and taking action to obtain patents and licensing of inventions; among others. It is led by a Board of Directors, four of whom are ex officio members, nine from candidates provided by the National Academy of Sciences, and five from candidates provided by "patient and consumer advocacy groups, professional scientific and medical societies, and trade organizations." The section specifies the number of members to be appointed representing each type of group and requires that the ex officio members ensure specific expertise among the members.

Provision

The provision would change the membership of the Board of Directors to allow the voting members of the board to increase the size of the board and appoint new members by majority vote, without regard to the balance of expertise and affiliation required by current law. It would limit to 30% of the membership "representatives of the general pharmaceutical, device, food, cosmetic, and biotechnology industries." The obligation to ensure specific expertise among the members would be broadened to rest with all members of the board, not only ex officio appointees. That broader group would also decide other administrative matters.

The provision would remove the salary cap of the foundation's Executive Director, which is now set at the compensation of the Commissioner. Also amended would be the language regarding separation of funds. The current requirement is that funds received from the Treasury be held in separate accounts from funds received from other sources, including private entities. The provision would change the requirement, so that funds received from the Treasury would be "managed as individual programmatic funds, according to best accounting practices."

Section 2284. Collection of Certain Voluntary Information Exempted from Paperwork Reduction Act

Background

The Paperwork Reduction Act (PRA, 44 U.S.C. Chapter 35), enacted in 1980 and amended in 1995, established the Office of Information and Regulatory Affairs (OIRA) in the Office of Management and Budget (OMB). Congress required that agencies seek OIRA permission before collecting information from the public. The first of 11 stated purposes was to "minimize the paperwork burden for individuals ... and other persons resulting from the collection of

information by and for the Federal Government."[184] The PRA requires that federal agencies receive clearance from OIRA before requesting most types of information from the public.[185] PRA clearance is required when standardized information is collected from 10 or more respondents within a 12-month period.[186] The PRA does not apply to certain types of scientific research, including collections that are neither sponsored nor conducted by the agency and those that are subject to a clinical exception.[187]

Provision

This provision would add a new Section 708A to the FFDCA, which would exempt from the PRA Secretary-initiated collection, such as through surveys or questionnaires, of voluntary information from patients, industry, academia, and other stakeholders.

Section 2285. Hiring Authority for Scientific, Technical, and Professional Personnel

Background

Title 5 of the *United States Code* provides the broad framework of requirements under which many federal employees are hired; however, some subsets of employees are hired under alternative government-wide or agency-specific authorities. Numerous hiring authorities target scientists and other technical workers, for whom federal agencies such as FDA compete with the private sector and nonfederal public employers.[188] For example, FFDCA Section 714 authorizes the Secretary to appoint employees to positions in FDA to perform, administer, or support activities related to review of medical device applications and human generic drugs "without regard to the provisions of title 5, United States Code, governing appointments in the competitive service."

Provision

The provision would add to the FFDCA a new Section 714A allowing the Secretary to "appoint qualified candidates to scientific, technical, or professional positions" in the competitive service within FDA's Center for Drug Evaluation and Research, Center for Biologics Evaluation and Research, and Center for Devices and Radiological Health "without regard to the provisions of title 5, United States Code, governing appointments in the competitive service." The Secretary would be allowed to determine pay (not to exceed the annual rate of pay of the President) for the purposes of retaining qualified employees, notwithstanding certain General Schedule pay rate requirements. The Secretary would be required to submit a report to Congress (1) examining how this authority enhanced FDA's ability to recruit and retain highly qualified individuals and (2)

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[184] 44 U.S.C. §3501.

[185] For further information about the PRA, see CRS Report RL30590, *Paperwork Reduction Act Reauthorization and Government Information Management Issues*, and CRS Report RL32397, *Federal Rulemaking: The Role of the Office of Information and Regulatory Affairs*.

[186] See NIH, Office of Science Policy, Genetics, Health and Society, *What is the Paperwork Reduction Act?*, at http://osp.od.nih.gov/faq/what-paperwork-reduction-act; and, HHS, *Frequently Asked Questions About PRA / Information Collection*, at http://www.hhs.gov/ocio/policy/collection/infocollectfaq.html.

[187] Cass R. Sunstein, *Facilitating Scientific Research by Streamlining the Paperwork Reduction Act Process*, Executive Office of the President, Office of Management and Budget, December 9, 2010, https://www.whitehouse.gov/sites/default/files/omb/memoranda/2011/m11-07.pdf.

[188] CRS Report R40604, *Hiring and Pay Authorities for Federal Scientific and Technical (S&T) Personnel*.

providing recommendations on whether the authority should be reauthorized (although the authorization would not expire) and other personnel authorities that might help the FDA recruit and retain highly qualified personnel.

Subtitle Q—Exempting From Sequestration Certain User Fees

Section 2301. Exempting From Sequestration Certain User Fees of Food and Drug Administration

Background

Sequestration is a budgetary enforcement process involving automatic, largely across-the-board spending reductions that are triggered under certain conditions.[189] It was first authorized by the Balanced Budget and Emergency Deficit Control Act of 1985 (BBEDCA, Title II of P.L. 99-177). Certain federal programs are exempt from sequestration under BBEDCA Section 255. Others are subject to special rules, such as partial sequestration, under BBEDCA Section 256. Under current law, FDA user fees, paid by industry to support FDA's review or regulation of medical products, have been interpreted as being subject to sequestration.[190]

Provision

The provision would add user fees for the following FDA-regulated medical products to the list in BBEDCA Section 255(g), exempting these fees[191] from future sequestrations:

- brand human drugs and biologics and generic human drugs, under FFDCA Sections 736 and 744B, respectively;
- human devices under FFDCA Section 738;
- biosimilar biological products under FFDCA Section 744H; and
- brand and generic animal drugs under FFDCA Sections 740 and 741, respectively.

The provision also would amend BBEDCA Section 256(h) to clarify that administrative expenses funded by the user fees listed above would be exempt from sequestrations that would otherwise apply to agency administrative expenses.

[189] See CRS Report R42050, *Budget "Sequestration" and Selected Program Exemptions and Special Rules*, coordinated by Karen Spar .

[190] See Stephanie Beasley, "FDA Funding Advocates Push 'Permanent Fix' For User Fee Sequestration," *FDA Week*, January 23, 2014. OMB determined that FDA user fees were fully sequestrable in FY2013. Congressional appropriators subsequently released those sequestered funds and made them available to the FDA in the FY2014 Agriculture appropriations act (Section 747 in Division A of the Consolidated Appropriations Act, 2014 (P.L. 113-76)).

[191] This provision would not exempt from sequestration authorized user fees that support FDA activities in its Foods and Tobacco Products programs or various smaller user fees, such as those relating to mammography quality or color certification.

Subtitle R—Other Provisions[192]

Section 2321. Sense of Congress

Background

FFDCA section 519(f) directed the Secretary of HHS to publish regulations establishing a unique device identification (UDI) system for medical devices.[193] The final rule, published in 2013, requires that device manufacturers include a UDI on the device label and package except when the rule provides an exemption or alternative. A UDI is also required to be on the device itself if the device is intended for more than one use and is reprocessed before each use. FDA has identified a number of benefits to be expected following full implementation of the UDI system including, for example: more accurate reporting, reviewing and analyzing of device adverse event reports; more effective management of device recalls; and a more robust postmarket surveillance system, allowing for the premarket approval or clearance of new devices and new uses of currently marketed devices.[194] However, these benefits will only be realized if UDI is incorporated at the point-of-care in electronic health records and in health claims data.[195]

Provision

Provision states that it is "the sense of Congress that recording unique device identifiers at the point-of-care in electronic health record systems could significantly enhance the availability of medical device data for postmarket surveillance purposes."

Title III—Delivery

Subtitle A—Interoperability

Section 3001. Ensuring Interoperability of Health Information Technology

Background

The Health Information Technology for Economic and Clinical Health (HITECH) Act was enacted in 2009 to support the development of a nationwide health information technology (HIT)

[192] Amendment 6 (Fitzpatrick) to H.R. 6 added Subtitle R and Section 2321; it was approved by voice vote. Amendment 7 (Polis) to H.R. 6 was withdrawn. It would have added an alternative Subtitle R and Section 2321 regarding a report, submitted to Congress by the Secretary of HHS, assessing the feasibility, benefits and risks of establishing an expedited two-tiered FDA approval process for medical devices to be lawfully marketed as of the date the device was shown to be safe, regardless of whether the device was shown to be effective.

[193] Section 226 of FDAAA added section 519(f) to the FFDCA. Section 614 of FDASIA required the Secretary to issue proposed regulations for the UDI system no later than December 31, 2012; these were published July 10, 2012, and the final rule was published on September 24, 2013.

[194] For additional benefits, see FDA, Medical Devices, Benefits of a UDI System, at: http://www.fda.gov/MedicalDevices/DeviceRegulationandGuidance/UniqueDeviceIdentification/BenefitsofaUDIsystem/default.htm

[195] Gregory Daniel, Mark McClellan, and Siromi Gardina, et al., *Unique Device Identifiers (UDIs): A Raodmap for Effective Implementation.*, The Engelberg Center for Health Care Reform at Brookings, December 2014, p. p. 5, http://www.fda.gov/downloads/MedicalDevices/DeviceRegulationandGuidance/UniqueDeviceIdentification/BenefitsofaUDIsystem/UCM445330.pdf.

infrastructure for the exchange of electronic health information.[196] The HITECH Act authorized Medicare and Medicaid incentive payments to promote the use of electronic health record (EHR) technology by hospitals and physicians. To date, the EHR incentive programs have provided more than $30 billion in incentive payments and resulted in a dramatic increase in the adoption of EHR systems among health care providers.[197]

Hospitals and physicians qualify for incentive payments under the HITECH Act if they become meaningful users of certified EHR technology. In order to demonstrate meaningful EHR use, providers must attest to using their EHR systems to perform certain functions; for example, capturing patient data, electronic prescribing, and exchanging summary of care information with other health care providers. These meaningful use objectives (and associated metrics) are developed by CMS based on recommendations from the HIT Policy Committee, which was established by the HITECH Act.

The vendors who develop EHR systems must incorporate various technical standards into the technology so that different systems can share information. Those standards are adopted by the HHS Office of the National Coordinator for Health Information Technology (ONC) based on recommendations it receives from the HIT Standards Committee, established by the HITECH Act. The HIT Standards Committee works closely with standards development organizations (SDOs) to create the recommendations. ONC also issues certification criteria, which are used to test and certify that EHR technology has the required capabilities and meets all the standards to support meaningful use.

While the incentive programs have had an enormous impact on EHR adoption rates, many providers that use EHR technology have difficulty exchanging information. Many of the challenges and barriers to EHR interoperability—the ability of EHR systems to exchange and use electronic health information—are well understood and are being addressed. For example, ONC is working with stakeholders on a Shared Nationwide Interoperability Roadmap to identify short- and long-term goals for the next 10 years, with 2017 set as a deadline to achieve basic EHR interoperability (i.e., the ability of EHR systems to send, receive, find, and use a common set of clinical information).[198] In April 2015, ONC released a report to Congress on health information blocking by HIT vendors, health care providers, and health care systems.[199] The report summarizes the evidence for information blocking (i.e., deliberate and unreasonable conduct that interferes with the ability of authorized persons to access, exchange, or use electronic health information); describes the various actions being taken by ONC and other agencies to address information blocking; and outlines the gaps in current knowledge, programs, and authorities that limit the ability of ONC and others to address information blocking.

The Medicare Access and CHIP Reauthorization Act of 2015 included several provisions to address EHR interoperability and information blocking.[200] It requires the Secretary to establish interoperability metrics by July 1, 2016, and sets a goal of widespread EHR interoperability by December 31, 2018, based on those measures. The Secretary must report to Congress by

[196] P.L. 111-5, Division A, Title XIII, and Division B, Title IV.

[197] CMS maintains an extensive website on the Medicare and Medicaid EHR incentive programs at http://www.cms.gov/ehrincentiveprograms.

[198] Office of the National Coordinator for Health Information Technology, *Connecting Health and Care for the Nation: A Shared Nationwide Interoperability Roadmap*, Draft Version 1.0, February 2015, http://www.healthit.gov/sites/default/files/nationwide-interoperability-roadmap-draft-version-1.0.pdf.

[199] Office of the National Coordinator for Health Information Technology, *Report on Health Information Blocking*, Report to Congress, April 2015, http://www.healthit.gov/sites/default/files/reports/info_blocking_040915.pdf.

[200] P.L. 114-10, §106(b), 129 Stat. 87.

December 31, 2019, with recommendations if the goal is not met. In addition, beginning April 16, 2016, health care providers attesting to EHR meaningful use must indicate that they have not knowingly or willfully taken any action to interfere with the interoperability of their EHR technology.

Provision

The provision would make a series of amendments to Title XXX of the PHSA ("Health Information Technology and Quality") to promote the adoption of new interoperability standards; strengthen the existing certification process to ensure that EHR systems and other HIT meet those standards; and establish new enforcement authority and penalties to discourage HIT vendors, health care providers, and health care systems from blocking the exchange and use of electronic health information.

First, the provision would establish interoperability criteria for HIT. To be considered interoperable, the technology would have to allow for the complete access, exchange, and use, and the secure transfer, of all electronically accessible health information under applicable state or federal law. Moreover, the technology must not be configured or implemented to permit information blocking, which is broadly defined to include business, technical, and organizational practices that impede electronic health information access, exchange, or use. Not later than January 1, 2017, the Secretary, in consultation with ONC, would be required to issue guidance on, and provide examples of, HIT interoperability.

Second, the provision would limit the HIT Policy Committee's role to providing policy and priority recommendations to the Secretary and prohibit it from otherwise affecting the development or modification of standards, implementation specifications, and certification criteria. It would terminate the HIT Standards Committee and, in its place, require the Secretary to enter into one or more contracts with accredited SDOs in order to obtain recommendations for an initial set of interoperability standards and accompanying implementation specifications. The Secretary would be required periodically to evaluate and review existing standards, implementation specifications, and certification criteria to determine if modifications or additions to such standards, specifications, and criteria are needed. If that is the case, the Secretary would be required to enter into subsequent contracts with SDOs to obtain additional recommendations. Each SDO under contract also would be required to submit its recommendations to the National Institute of Standards and Technology (NIST). The provision would authorize the appropriation of $10 million, to remain available until expended, for SDO contracts.

Third, the provision would establish rules and a timetable for the Secretary by regulation to adopt recommended interoperability standards. If no standard, implementation specification, or certification criterion were recommended by an SDO, the Secretary would be permitted by regulation to adopt a standard, implementation specification, or certification criterion recommended by the HHS National Committee on Vital and Health Statistics. The Secretary may not adopt any policies, standards, implementation specifications, or certification criteria that are inconsistent with or duplicative of an interoperability standard adopted under this provision.

Fourth, the provision would require the Secretary, by July 1, 2017, to submit to Congress and make publicly available a report on (1) the initial set of adopted interoperability standards, (2) barriers preventing widespread interoperability, and (3) strategies (including specific steps and milestones) for achieving widespread interoperability.

Fifth, beginning January 1, 2018, EHR technology would have to comply with the new interoperability standards in order to obtain certification. In addition, vendors of such technology would have to attest to a series of requirements as a condition of certification, and maintenance of

certification. They include (1) not taking any action (including business, technical, and organizational practices) that constitutes information blocking; (2) making publicly available detailed pricing information on any additional types of costs or fees (or other contractual limitations) associated with using any function or capability for which the EHR technology is to be certified; (3) publishing, and demonstrating the use of, application programming interfaces (APIs) that allow health information maintained by a vendor's EHR technology to be exchanged, accessed, and used without special effort. Beginning January 1, 2019, EHR technology that did not comply with the new interoperability standards, or whose vendor violated the terms of the attestation, would be decertified.

Sixth, the provision would give the HHS Inspector General new enforcement authority to investigate claims of EHR vendors being in violation of the attestation requirements, or EHR vendors and users engaging in information blocking. ONC would be required to establish a standardized process for the public to submit reports of EHR technology that was not interoperable, EHR technology that resulted in information blocking, or actions taken by vendors or users of EHR technology that impeded interoperability or resulted in information blocking. Any individual or entity engaged in such activity, with respect to items or services provided under the Medicare or Medicaid programs, would be subject to civil monetary penalties (CMPs) that the Secretary by rulemaking determined appropriate. The provision would authorize the appropriation of $10 million for FY2017, to remain available until expended, for enforcement activities. And it would allow the Inspector General to retain and use any CMP amounts it collected for such activities. It also would extend the privilege and confidentiality protections under Title IX, Part C, of the PHSA to claims of information blocking and reports related to the vendor attestation requirements.

Seventh, the provision would require the Secretary, within 12 months of enactment, to implement all the requirements of the provision through rulemaking. The Secretary also would be required to clarify that health care providers would not be penalized for the actions of vendors of EHR technology that failed to meet the certification requirements. Not later than January 1, 2017, ONC would be required to publish guidance on the relationship of the HIPAA privacy and security standards to information blocking.

Eighth, the provision would require ONC, by January 1, 2019, to post online information that allows purchasers of EHR technology to compare the functionality, price, and other features of the different products. Beginning in 2019, and each year thereafter, the Secretary would be required to post online a list of decertified EHR technology. The Secretary also would be required periodically to review and confirm that vendors have published APIs, as required for certification.

Ninth, beginning with the 2020 reporting period, eligible professionals and hospitals demonstrating meaningful use of certified EHR technology under the Medicare EHR incentive program would have to attest that they had not engaged in information blocking. Medicare-eligible professionals and hospitals determined not to be meaningful EHR users because their EHR technology was decertified would be eligible for a hardship exemption from the payment adjustment, subject to annual renewal. Similarly, beginning with the 2020 reporting period, eligible professionals and hospitals would not qualify for the Medicaid EHR incentive payments unless they attested that they had not engaged in information blocking. Not later than January 1, 2018, the Secretary would be required to issue guidance to help providers who voluntarily transition between different certified EHR technology by removing disincentives to such transitions.

Finally, the provision would instruct NIST, beginning January 1, 2018, to test the new interoperability standards in order to ensure their efficient implementation and use. It would authorize the appropriation of $15 million, to remain available until expended, for NIST testing.

The provision also would eliminate the interoperability language in the Medicare Access and CHIP Reauthorization Act of 2015 (i.e., Section 106(b) of P.L. 114-10).

The provision concludes by stating that it is the sense of Congress that, among other things (1) interoperability is best achieved if individuals and their authorized representatives (e.g., family members, caregivers) have equal access to the electronic health information of such individuals; (2) individuals and their authorized representatives have the right to the entire medical record; (3) health care providers should not be able to deny a patient's request for access to the entire record; and (4) health care providers should not need the consent of their patients to share patient information with other HIPAA-covered entities as permitted under the privacy rule.

Subtitle B—Telehealth

Section 3021. Telehealth Services under the Medicare Program

Background

Telehealth is the use of electronic information and telecommunications technologies to support remote clinical health care, patient and professional health-related education, and other health care delivery functions. Telemedicine is the use of advanced telecommunications mainly to support clinical care. Live videoconferencing, telemonitoring, and the use of web-based technologies are examples of telecommunications that support telehealth under some parts of Medicare.

The Medicare program is structured in four parts: Part A (hospital insurance), Part B (physician services), Part C (managed care), and Part D (prescription drugs). CMS makes payments to providers for telehealth services under all parts, except Part A. Medicare Part B pays for medically necessary services and preventive services, such as those that are needed to diagnose or treat a medical condition and that meet accepted standards of medical practice.[201] Telehealth services must be provided through live videoconferencing, and there are two payments: (1) to physicians or other professionals at the distant site, for the telehealth consultation, and (2) to the facility where the patient is located, which is the originating site.[202] Medicare Part C (Medicare Advantage, or MA) private health insurance plans may provide basic telehealth benefits as part of the standard benefit; telemonitoring and web-based and phone technologies may be used to provide telehealth services.[203] Under Medicare Part D, Medicare Advantage Prescription Drug (MAPD) plans or Prescription Drug Plans (PDPs) may choose to include telehealth services as part of their plan benefits, for instance, in providing medication therapy management (MTM).[204]

[201] SSA, Section 1862(a)(1)(A).

[202] SSA, Section 1839(m)(2).

[203] The Medicare Prescription Drug, Improvement, and Modernization Act of 2003 (MMA) (P.L. 108-173), enacted on December 8, 2003, added a new ''Part D'' to Sections 1860D-1 through 42 of the Social Security Act, and made significant changes to the existing Part C program, which it named the Medicare Advantage (MA) Program.

[204] Part D Medication Therapy Management (ACA Section 10328); and Durable Medical Equipment (DME) Face to Face (ACA Section 6407). The regulations governing the Part D program are set forth in 42 C.F.R. Part 423—Voluntary Medicare Prescription Drug Benefit. See also CMS, *Medicare Managed Care Manual, Chapter 4, Benefits and Beneficiary Protections, Section 30.3—Examples of Eligible Supplemental Benefits*, which includes (for example) telemonitoring services, remote access technologies, interactive web- and/or telephone-based coaching (available at http://www.cms.gov/Regulations-and-Guidance/Guidance/Manuals/downloads/mc86c04.pdf).

Barriers to increased use of telehealth services under the Medicare program include, but are not limited to, the lack of specific telehealth payments in circumstances other than those specified under current law (Section 1834(m)), uneven broadband access, and current licensure requirements for health professionals. Having evidence-based models that support telehealth as a safe, effective, and appropriate use of technology in health care delivery would help support the expansion of telehealth services. Telehealth or telemedicine services are not appropriate for all conditions and illnesses,[205] and evidence is needed to establish appropriate quality standards in additional areas of health care delivery.[206]

Provision

The provision would require, within a year of enactment, the CMS Administrator and MedPAC to submit information to Congress on telehealth. The CMS Administrator would be required to provide information on subpopulations of Medicare beneficiaries that might benefit from telehealth services; CMMI activities that examine the use of telehealth services in models, projects, or initiatives; the types of high-volume services under the Medicare program that might be suitable for telehealth reimbursement; and barriers to telehealth expansion. MedPAC would be required to identify telehealth services that are reimbursable under Medicare Parts A and B; telehealth services that are reimbursable by private health insurance plans; and potential ways to incorporate into Medicare Parts A and B those telehealth services for which they do not currently reimburse but for which private health insurance plans do reimburse.

The provision would express the sense of Congress that eligibility for telehealth coverage under the Medicare program should be expanded. Any such expansion should recognize that telemedicine is the delivery of safe, effective, quality health care services by a health care provider, using technology as the mode of care delivery; should meet or exceed conditions for Medicare coverage and payment; and should involve clinically appropriate means for delivering telehealth services.

Subtitle C—Encouraging Continuing Medical Education for Physicians

Section 3041. Exempting From Manufacturer Transparency Reporting Certain Transfers Used for Educational Purposes

Background

In recent years, questions have been raised about certain financial relationships between health care professionals, such as physicians, and the pharmaceutical and other medical industries. As part of these relationships, companies may give gifts or make payments to health care professionals as part of their marketing efforts or for other purposes. In an effort to promote transparency and prevent inappropriate relationships, Section 1128G of the Social Security Act[207]

[205] HHS, *Telemedicine for the Medicare Population: Update, Prepared for Agency for Healthcare Research and Quality* (Contract Number: 290-02-0024), February 2006, Prepared by Oregon Evidence-based Practice Center; and MedPAC, *Report to the Congress: Medicare and the Health Care Delivery System* (June 2012).

[206] Institute of Medicine, *The Role of Telehealth in an Evolving Health Care Environment: Workshop Summary*, Washington, DC, 2012, p. 15, http://www.iom.edu/Reports/2012/The-Role-of-Telehealth-in-an-Evolving-Health-Care-Environment.aspx.

[207] 42 U.S.C. §1320a–7h.

generally requires applicable drug, device, biological, or medical supply manufacturers that make a payment or other transfer of value to a "covered recipient" (i.e., a physician or a teaching hospital) to annually report information on such transactions to the Secretary of HHS. Categories of payments and transfers of value that must be reported include amounts for research, gifts, entertainment, consulting fees, grants, meals, and travel. Certain items are exempt from disclosure, including payments or transfers of $10 or less, unless the aggregate annual payments or transfers to a recipient exceeds $100 (which is indexed for inflation); samples intended for patient use; loans of a covered device for a short-term time period; and "educational materials that directly benefit patients or are intended for patient use."[208] Civil monetary penalties may be imposed for noncompliance with this section.

In addressing the scope of the exclusion for educational materials, CMS has stated that while certain items provided to covered recipients, such as medical text books and journal reprints, are important for physicians, they are not directly beneficial to patients or intended for patient use, and thus are reportable under this section.[209] In addition, pursuant to regulations and CMS guidance, applicable manufacturers that contribute funding to a continuing medical education program may be required to report the payments provided to physician speakers.[210] An exception to this reporting exists for indirect payments or other transfers of value where the applicable manufacturer is unaware of the identity of the recipient during the reporting year or by the end of the second quarter of the following reporting year.[211]

Provision

The provision would broaden the exclusions from the reporting requirement under Section 1128G for certain education-related payments or transfers of value. It would specify that items such as peer-reviewed journals, journal reprints, journal supplements, medical conference reports, and medical textbooks may be considered patient educational materials, and therefore exempt from reporting under this section. In addition, in cases where the recipient is a physician, the provision would exclude indirect payments or transfers of value that (1) come from speaking at, or preparing educational materials for, an educational event for health care professionals, so long as the event does not commercially promote a covered drug, device, biological, or medical supply; or (2) are solely for the purpose of providing medical education to the physician. The amendments made by this section would apply to transfers of value made on or after the date of enactment of the act.

[208] 42 U.S.C. §1320a-7h(e)(10)(B).

[209] Medicare, Medicaid, Children's Health Insurance Programs; Transparency Reports and Reporting of Physician Ownership or Investment Interests, 78 *Fed. Reg.* 9458, 9486 (February 8, 2013).

[210] *See* 42 C.F.R. § 403.904; Centers for Medicare and Medicaid Services, Frequently Asked Questions, *available at* https://questions.cms.gov/faq.php?id=5005&faqId=11600. An "indirect payment" is defined as a payment or other transfer of value made by an applicable manufacturer to a covered recipient through a third party, where the applicable manufacturer requires, instructs, directs, or otherwise causes the third party to provide the payment or transfer of value, in whole or in part, to a covered recipient. 42 C.F.R. § 403.902.

[211] 42 C.F.R. § 403.904(h)(1).

Subtitle D—Disposable Medical Technologies

Section 3061. Treatment of Certain Items and Devices

Background

The Medicare Supplementary Medical Insurance Program (Part B) currently covers a wide variety of durable medical equipment (DME) if they are medically necessary and are prescribed by a physician.

Durable medical equipment is equipment that (1) can withstand repeated use, (2) has an expected life of at least three years (effective for items classified as DME after January 1, 2012), (3) is used to serve a medical purpose, (4) generally is not useful in the absence of an illness or injury, and (5) is appropriate for use in the home. DME also includes the drugs necessary for the proper function of certain DME, and supplies, such as surgical dressings, used in conjunction with the DME.

Historically, Medicare has paid for most DME on the basis of fee schedules. Medicare pays 80% of the lesser of the amount charged or the fee schedule amount, while the beneficiary is responsible for the remaining 20%, plus any unmet deductible. Unless otherwise specified by Congress, fee schedule amounts are updated each year by a measure of price inflation. However, studies by federal agencies have shown that Medicare pays above-market prices for certain items of DME. The Medicare Prescription Drug, Improvement, and Modernization Act (MMA, P.L. 108-173) established a Medicare-competitive acquisition program (i.e., competitive bidding), under which prices for selected DME sold in specified areas are determined not by a fee schedule but by the bids of winning suppliers. Prices for DME established through competitive bidding thus far have been lower than prices established through fee schedules.

The Health Care Common Procedure Coding System (HCPCS) is a standard coding system used to identify medical services and procedures furnished by a physician or other provider (HCPCS Level I Codes) and products, supplies, and services not included in the Level I codes (HCPCS Level II Codes). Some HCPCS Level I codes for physician services would include payment for a medical device, such as when a therapeutic or diagnostic infusion is administered in a physician's office using a pump. Durable medical equipment used in a beneficiary's home is identified using HCPCS Level II Codes.[212]

Medicare provides coverage for home health services to beneficiaries (1) who are homebound; (2) who require part-time or intermittent skilled nursing care and/or skilled rehabilitation, or, after establishing prior eligibility, a continuing need for occupational therapy; and (3) for whom the services have been established in a plan of care that has been ordered, certified, and periodically reviewed by a physician. Medicare home health services include a variety of items and services such as nursing visits, home health aide visits, medical supplies, and DME. While most home health services are paid for under a single reimbursement amount, DME is excluded and reimbursed separately. Home health agencies may provide DME directly or under arrangement with a DME supplier.

Provision

The provision would establish a separate payment for certain disposable devices when those items are furnished during a home health episode and paid directly to the home health agency.

[212] http://www.cms.gov/Medicare/Coding/MedHCPCSGenInfo/HCPCS_Coding_Questions.html.

The devices to be paid for by Medicare under this provision would be those items, as of January 1, 2015, for which (1) there is a Level I HCPCS code for which the description for a professional service includes furnishing the device, and (2) there is a separate Level I code for a professional service that uses DME instead of such device. The payment for the disposable devices could not exceed the payment that would be made for the HCPCS Level I code for professional services, including such a device when paid for in a hospital outpatient department. The disposable devices added by this section of the bill would be included in the Medicare home health benefit. These provisions would be effective for devices furnished on or after January 1, 2017.

Subtitle E—Local Coverage Decision Reforms

Section 3081. Improvements in the Medicare Local Coverage Determination (LCD) Process

Background

CMS administers the Medicare program through contracts with private entities, such as Medicare Administrative Contractors (MACs). MACs assist CMS in administering Medicare's day-to-day operations, such as paying fee-for-service (FFS) claims, enrolling providers, coordinating provider customer service, and other activities. MACs also conduct program integrity activities, including prepayment and postpayment claims review, provider audits, and overpayment recoupment. In addition, MACs develop and implement local coverage determinations (LCD) for their jurisdictions.

Medicare covers a broad range of medical treatments, services, and equipment needed by beneficiaries, but there are limitations to Medicare's coverage. To be covered by Medicare, items or services must be considered reasonable and necessary for the diagnosis or treatment of an illness or injury, or to improve the functioning of a body part.[213] Medicare law defines categories of services and items that Medicare routinely covers, but the law does not specify which services or under what conditions these items and services are covered. Under the reasonable and necessary provision, the Secretary has discretion to determine what specific items and services will be covered and under what conditions.

The Secretary has authority to make Medicare coverage policy decisions both nationally and locally.[214] LCDs are MAC decisions on whether, and under what circumstances, to cover a particular item or service on a contractor-wide basis.[215] National coverage decisions (NCDs) are made by CMS to describe the circumstances under which Medicare will cover an item or service on a nationwide basis. The vast majority of coverage policy is determined on a local level by MACs. MACs initiate LCDs and may develop them in the absence of relevant NCDs or as a supplement to an NCD, as long as the LCD policy does not conflict with national Medicare policy.[216]

CMS's Medicare Program Integrity Manual instructs MACs on how to develop LCDs.[217] The process includes several mechanisms for local stakeholder input, including notice and comment

[213] Social Security Act Section 1862(a)(1).

[214] CMS maintains an online database of all coverage decisions at http://www.cms.gov/medicare-coverage-database/.

[215] Medicare Program Integrity Manual, Chapter 13, Section 13.1.3, Local Coverage Determinations.

[216] Section 522 of the Medicare, Medicaid, and SCHIP Benefits Improvement and Protection Act of 2000 (BIPA, P.L. P.L. 106-554).

[217] Medicare Program Integrity Manual, Chapter 13, Section 13.1.3, Local Coverage Determinations.

periods for new LCDs and state-based physician advisory committees, referred to as Carrier Advisory Committees (CACs), to provide formal LCD input. In developing LCDs, MACs use medical literature, the advice of local medical societies and medical consultants, public comments, and comments from their provider community. MACs are responsible for ensuring that LCDs are consistent with all statutes, rulings, regulations, and national coverage decisions.

Provision

The provision would require the Secretary to require MACs to display on their websites and on the Medicare website at least 45 days prior to the effective date the following information for each LCD developed by a MAC for its jurisdiction:

- the entire LCD;
- where and when the proposed LCD was first made public;
- hyperlinks to the proposed LCD and responses to comments submitted to the MAC on the proposed LCD;
- a summary of evidence considered by the contractor during the LCD development, as well as a list of sources of evidence; and
- an explanation of the rationale in support of the proposed LCD.

The provision would be effective for LCDs proposed or revised 180 days after the enactment date.

Subtitle F—Medicare Pharmaceutical and Technology Ombudsman

Section 3101. Medicare Pharmaceutical and Technology Ombudsman

Background

Under current Medicare law, the Secretary is not required to offer ombudsman services to entities that manufacture pharmaceutical, biotechnology, medical device, or diagnostic products for which these entities are seeking Medicare coverage.[218]

Medicare law requires the Secretary to conduct a satisfaction survey at least every five years of beneficiaries, as well as providers and suppliers who submitted appeals (SSA §1869(e)) and to submit a report to Congress on the results of the survey. In addition, SSA Section 1808(c) requires the Secretary to appoint a Medicare Beneficiary Ombudsman. The Office of Medicare Ombudsman (OMO) was created to identify and address systemic issues that affect Medicare beneficiaries, but OMO does not help pharmaceutical, biotechnology, medical device, or diagnostic product manufacturers resolve complaints, grievances, or requests about Medicare coverage. The Medicare Beneficiary Ombudsman is prohibited from serving "as an advocate for any increases in payments or new coverage of services," but is authorized to "identify issues and problems in payment or coverage policies."[219]

[218] According to CMS's annual beneficiary publication, Medicare & You, an ombudsman is someone who reviews complaints and helps to resolve those complaints.

[219] SSA §1808(c)(2).

Provision

The provision would require the Secretary to provide within 12 months of enactment a pharmacy and technology ombudsman within CMS. The pharmacy and technology ombudsman would receive and respond to complaints, grievances, and requests (regarding coverage, coding, or payment) from pharmaceutical, biotechnology, medical device, or diagnostic product manufacturers whose products are covered by Medicare or for which coverage was sought. The pharmaceutical and technology ombudsman would be subject to the same prohibition on advocacy and authority to identify issues as the Medicare Beneficiary Ombudsman.

Subtitle G—Medicare Site-of-Service Price Transparency

Section 3121. Medicare Site-of-Service Price Transparency

Background

Some Medicare-covered items and services can be provided either in a physician's office, in a hospital outpatient department, or in freestanding or hospital-operated ambulatory surgical centers (ASCs); the payments would be determined by the Medicare physician fee schedule (MPFS), the Medicare hospital outpatient prospective payment system (OPPS) fee schedule, or the Medicare ASC payment system, respectively. The Medicare Payment Advisory Commission (MedPAC) has recommended (for instance, in its March and June 2013 reports to Congress) that Medicare implement "site-neutral" policies, for instance, those that would equalize outpatient payment rates at hospitals to those of free-standing physician offices.

Provision

The provision would establish new requirements "to facilitate price transparency with respect to items and services for which payment may be made either to a hospital outpatient department or to an ambulatory surgery center." Beginning in 2017 and in each year thereafter, the Secretary would make information available to the public via a searchable website on (1) the estimated Medicare payment amount for the items and services provided under both the hospital outpatient prospective payment (OPPS) fee schedule and the ambulatory surgical center payment system, and (2) the estimated amount of the beneficiary's liability (for the item or service). The estimated amount of beneficiary liability would be calculated based on the amount for which an individual who does not have any Medicare supplemental coverage is responsible. The Secretary would include the information described above in the annual explanation of Medicare benefits sent to all beneficiaries. The Secretary could also use existing mechanisms, such as the CMS Physician Compare website, to make this information available to beneficiaries. To implement this subsection, the Secretary would transfer $6 million from the Supplemental Medical Insurance Trust Fund to the CMS Program Management Account for FY2015; these funds would remain available until expended.

Subtitle H—Medicare Part D Patient Safety and Drug Abuse Prevention

Section 3141. Programs to Prevent Prescription Drug Abuse Under Medicare Parts C and D

Background

The Medicare Prescription Drug, Improvement, and Modernization Act of 2003 (MMA, P.L. 108-173) established Medicare Part D as a voluntary, outpatient prescription drug benefit. Part D coverage is provided through private insurers, known as plan sponsors, that offer drug-only plans (PDPs) or Medicare Advantage (MA or Part C) managed care plans that include a Part D benefit (MA-PDs). According to recent government studies, some Part D beneficiaries have obtained overlapping prescriptions from multiple prescribers for frequently abused prescription drugs.[220] CMS has taken several steps to address the issue, including implementing an Overutilization Monitoring System (OMS) to more closely track whether Part D sponsors have adequate systems in place to track potential drug abuse.[221]

Provision

The provision would allow Part D plan sponsors to institute a drug management program limiting the number of prescribers and pharmacies that could provide frequently abused drugs to Part D beneficiaries at risk for drug abuse, require Part D plans to implement drug utilization management tools, and expand the authority of Medicare Drug Integrity Contractors (MEDICs).[222] The changes would take effect for Part D plan years beginning more than one year after the date of enactment.

Drug Management Program—The provision would amend Section 1860D-4(c) of the Social Security Act (42 USC 1395w-10(c)) to allow Part D sponsors to designate certain enrollees as at risk for prescription drug abuse based on clinical guidelines developed by the Secretary in consultation with sponsors and other stakeholders and limit them to specific prescribers and pharmacies. Individuals in hospice and certain long-term care settings would be exempt from the at-risk designation. A frequently abused drug would be defined as a controlled substance that the Secretary determined to be frequently abused or diverted.

Sponsors that chose to implement the drug management program would be required to provide identified beneficiaries with an initial and a second notice explaining the at-risk designation, the right to appeal the designation, information about public health programs to combat drug abuse, and an explanation of the drug management program. The notices would also ask the beneficiary for a list of preferred prescribers or pharmacies. Sponsors would assign at-risk beneficiaries to pharmacies and prescribers based on the beneficiary preferences. However, if preferred

[220] Government Accountability Office, "Medicare Part D: Instances of Questionable Access to Prescription Drugs," September 2011, http://www.gao.gov/assets/590/585424.pdf; and CMS, "Supplemental Guidance Related to Improving Drug Utilization Review Controls in Part D," September 6, 2012, p. 30. Available at http://www.cms.gov/Medicare/Prescription-Drug-Coverage/PrescriptionDrugCovContra/RxUtilization.html.

[221] CMS, Final Call Letter 2013, "Improving Drug Utilization Review Controls in Part D." Available at http://www.cms.gov/Medicare/Health-Plans/MedicareAdvtgSpecRateStats/Announcements-and-Documents.html.

[222] CMS contracts with regional MEDICs to help manage audit, oversight, and anti-fraud and abuse efforts in the Part D program. The MEDICS identify cases of suspected fraud, and take action to prevent improper payment Medicare Trust Fund monies and to help recoup inappropriate payments.

prescribers or pharmacies were found to be contributing to drug abuse or diversion, a sponsor could alter the selections.

A beneficiary's at-risk identification would terminate at the earlier of (1) the date the individual demonstrated that he or she was no longer likely to be at risk, or (2) the end of a maximum period that the Secretary would specify. A sponsor could identify an individual as at risk after termination if new information became available.

By January 1, 2016, the Secretary would be required to hold a meeting of stakeholders, including Medicare beneficiaries, advocacy groups, physicians, pharmacists and other clinicians, plan sponsors, and drug manufacturers, for input on issues such as the impact of drug management programs on cost-sharing and drug accessibility, an expedited appeals process, the types of enrollees to be exempted, and the responsibilities of Part D sponsors that offer drug management programs. The Secretary would be required to provide educational materials to Part D enrollees describing the drug management program.

Utilization Management Tools—The provision would amend Section 1860D-4(c) of the Social Security Act to require Part D plans to have a drug utilization tool to prevent drug abuse. The tool could be either (1) a utilization management tool to prevent abuse of frequently used drugs and diversion of drugs at pharmacies, (2) a retrospective review of drug use to identify individuals that receive frequently abused drugs at a frequency or in amounts that are not clinically appropriate and to identify providers of services or suppliers that may facilitate the abuse or diversion of frequently abused drugs, or (3) a consultation process with MEDICs to verify whether an individual enrolling in a Part D plan has been previously identified by another Part D sponsor as an at-risk beneficiary. Sponsors would be required to provide monthly reports to the Secretary and the MEDICs regarding providers and at-risk beneficiaries.

MEDICs—The provision would amend Section 1893 of the Social Security Act (42 U.S.C. 1395ddd) to provide that the Secretary, as part of contracts, authorize MEDICs to directly accept prescription and necessary medical records from pharmacies, prescription drug plans, and other entities to provide information relevant to determining whether a beneficiary is at risk of prescription drug abuse. MEDICs would be required to acknowledge referrals and inform plan sponsors or organizations within 15 days whether an individual was determined to be at risk. MEDIC would be allowed to respond to requests for information from sponsors and other entities in order to prevent fraud and abuse, in compliance with federal privacy laws.[223] The HHS Office of the Inspector General (HHS OIG) would be required to submit a study to Congress, no later than a year after enactment, on the effectiveness of MEDICS in identifying, combatting, and preventing fraud.

[223] See CRS Report R43991, *HIPAA Privacy, Security, Enforcement, and Breach Notification Standards*.

Title IV—Medicaid, Medicare, and Other Reforms

Subtitle A—Medicaid and Medicare Reforms

Section 4001. Limiting Federal Medicaid Reimbursement to States for Durable Medical Equipment (DME) to Medicare Payment Rates

Background

States generally are free to set payment rates for items and services provided under Medicaid as they see fit, subject to certain exceptions and a general requirement that payment policies are consistent with efficiency, economy, and quality of care and are sufficient to provide access equivalent to the general population's access. However, there are federal upper payment limits on fee-for-service reimbursement of certain Medicaid providers—particularly hospitals and nursing facilities. Federal upper payment limit regulations specify that states cannot pay more in the aggregate for inpatient hospital services or nursing facility services than the amount that would be paid for the services under the Medicare principles of reimbursement. No upper payment limit currently applies to durable medical equipment (DME) under Medicaid.

Historically, Medicare has paid for most DME on the basis of fee schedules. Unless otherwise specified by Congress, fee schedule amounts are updated each year by a measure of price inflation. However, studies by federal agencies have shown that Medicare pays above-market prices for certain items of DME. Such overpayments may be due partly to the fee schedule mechanism of payment, which does not reflect market changes. Examples of such market changes include new and less-expensive technologies, changes in production or supplier costs, and geographic price variations.

The Medicare Prescription Drug, Improvement, and Modernization Act (MMA, P.L. 108-173) established a Medicare competitive acquisition program (i.e., competitive bidding) under which prices for selected DME sold in specified areas are determined not by a fee schedule but by the bids of winning suppliers. Payments based on the bids of winning suppliers went into effect in the first nine metropolitan areas on January 1, 2011. The program has since expanded to 100 metropolitan areas. Starting in 2016, the Secretary is required to either expand competitive bidding into additional areas, or apply information gained from competitive bidding to adjust fee schedule amounts in areas where competitive bidding is not taking place. The authorizing legislation also required the Secretary to establish a Competitive Acquisition Ombudsman (CAO) to respond to complaints and inquiries made by suppliers and individuals, and submit to Congress an annual report on the competitive bidding program.[224] The CAO is organized within the Office of the Medicare Ombudsman at CMS.

Provision

The provision would establish, for the first time, an upper payment limit for durable medical equipment under the Medicaid program. Each state that pays for DME on a fee schedule basis would not be able to pay more in the aggregate for DME than would be paid for such items under Part B of the Medicare program (including what would be paid under the Medicare competitive

[224] See the most recent report CAO report: Tangita Daramola, *Medicare Competitive Acquisition Ombudsman*, Centers for Medicare & Medicaid Services, 2011 Report to Congress, https://www.cms.gov/Medicare/Medicare-Fee-for-Service-Payment/DMEPOSCompetitiveBid/Downloads/CAO-2011-Report-to-Congress.pdf.

bidding program). Though the provision would specify an aggregate upper payment limit for Medicaid DME spending in a state, as with other Medicaid upper payment limit restrictions, it does not specify the actual amount paid by the state for the individual items, or whether those payments would vary geographically within the state. The upper payment limit would be effective for payments for items furnished on or after January 1, 2020. This provision would also require the Medicare Ombudsman to evaluate the impact of the competitive acquisition program on beneficiary health status and health outcomes, including its application to the Medicaid program.

Section 4002. Excluding Authorized Generics from Calculation of Average Manufacturer Price

Background

Prescription drugs are an optional Medicaid benefit, but all states cover outpatient drugs. Pharmaceutical manufacturers that voluntarily participate in Medicaid are required to pay rebates to states on covered outpatient drugs, which help Medicaid receive manufacturers' lowest or best price.[225] States then share the rebate they receive from pharmaceutical manufacturers with the federal government.

Under the federal Medicaid law, Medicaid rebate calculations for brand name drugs have two components, a basic rebate and an additional rebate. The basic rebate is the higher of a drug's best price compared to its quarterly AMP or 23.1% of the average manufacturer price (AMP).[226] The additional rebate is calculated by determining whether a drug's price has increased faster than inflation since it was first introduced to the market. The additional rebate is added to the basic rebate to get a brand drug's total rebate. Medicaid rebates for generic drugs have only a basic rebate without an additional adjustment when prices rise faster than inflation.

Authorized generics are drugs that the original patent holder has licensed to a generic drug manufacturer to sell at a negotiated, reduced price.[227] It is argued that authorized generics raise prices for consumers and reduce incentives for generic manufacturers to challenge brand name drug patents. Including authorized generic sales with brand name drug sales has the effect of lowering a product's average manufacturer price (AMP), thereby decreasing manufacturers' Medicaid rebate obligations for those products (both the basic and the additional rebate might be decreased).[228]

Provision

This proposal would require drug manufacturers to exclude sales of authorized generics from the calculation of average manufacturer price for brand name drug products. By removing authorized

[225] For more information on Medicaid prescription drugs, see CRS Report R43778, *Medicaid Prescription Drug Pricing and Policy*.

[226] Average manufacturer price for a Medicaid-covered outpatient drug for a rebate period (calendar quarter) is the average price paid to the manufacturer for the drug in the United States by (i) wholesalers for drugs distributed to retail community pharmacies (RCPs); and (ii) RCPs that purchase drugs directly from drug manufacturers (SSA §1927(k)(1)).

[227] Code of Federal Regulations (CFR) 42 CFR §447.506, an authorized generic drug means any drug sold, licensed, or marketed under an New Drug Application approved by the FDA under Section 505(c) of the Federal Food Drug and Cosmetics Act; and marketed, sold, or distributed under a different labeler code, product code, trade name, trademark, or packaging (other than repackaging the listed drug for use in institutions) than the brand drug.

[228] The additional rebates also would increase if sales of authorized generics are excluded from the calculation of brand-name drug AMPs.

generic sales from the single source product's AMP calculation, the AMP would be higher thus increasing the amount of rebate owed by manufacturers on brand name drugs provided to Medicaid beneficiaries. This provision would take effect on October 1, 2015.

Section 4003. Medicare Payment Incentive for the Transition from Traditional X-Ray Imaging to Digital Radiography and Other Medicare Imaging Payment Provision

Background

Currently, Medicare payments for services of physicians and certain non-physician practitioners, including many imaging services, are made on the basis of a fee schedule. The Medicare physician fee schedule (MPFS) assigns relative values to each of the approximately 7,500 service codes that reflect physician work (i.e., the time, skill, and intensity it takes to provide the service), practice expenses, and malpractice costs. The relative value for a service compares the relative work involved in performing one service with the work involved in providing other physicians' services. The scale used to compare the value of one service with another is known as a resource-based relative value scale (RBRVS). The relative values are adjusted for geographic variation in input costs. The adjusted relative values are then converted into a dollar payment amount by a conversion factor.

The Centers for Medicare & Medicaid Services (CMS), which is responsible for maintaining and updating the fee schedule, continually modifies and refines the methodology for estimating relative value units (RVUs). The American Medical Association/Specialty Society Relative Value Scale Update Committee (RUC) historically has provided advice and recommendations to CMS to assist in these assessments. CMS is required to review the RVUs no less than every five years; the ACA added the requirement that the Secretary periodically identify physician services as being potentially misvalued, and make appropriate adjustments to the relative values of such services under the Medicare physician fee schedule.

In determining adjustments to RVUs used as the basis for calculating Medicare physician reimbursement under the fee schedule, the Secretary of Health and Human Services has authority to adjust the number of RVUs for any service code to take into account changes in medical practice, coding changes, new data on relative value components, or the addition of new procedures. The Secretary of HHS is required to publish an explanation of the basis for such adjustments. These adjustments are subject to a budget neutrality condition. With the exception of certain expenditures that are exempt by statute, the adjustments may not cause the amount of expenditures made under the MPFS to differ from year to year by more than $20 million from the expenditures that would have been incurred without such an adjustment.

Following recommendations from the GAO and MedPAC, CMS established and implemented multiple procedure payment reduction (MPPR) policies to adjust payment to more appropriately reflect efficiencies gained when certain services are provided together, for example, when multiple similar services are performed on the same patient during the same visit. These payment reductions reflect efficiencies that typically occur in either the practice expense (PE) or professional work component of the MPFS (or both) when services are furnished together.

The MPPR amounts have varied across services and over time. For instance, in 2011, CMS established a MPPR to the PE component of payment of select therapy services paid under the MPFS, with payment for the second and subsequent services to be reduced 25%, similar to the reduction applied to multiple surgical procedures and to diagnostic imaging procedures. The

American Taxpayer Relief Act (ATRA) increased that reduction from 25% to 50% beginning April 1, 2013.

Provision

To incentivize the transition from traditional X-ray imaging to digital radiography in both physicians' offices as well as hospital outpatient departments, this provision would modify Section 1848(b) of the Social Security Act (42 U.S.C. 1395w–4(b)) by adding special rules to (1) limit the payment for film X-ray imaging services, and (2) provide incentives for the transition to digital radiography.

Beginning in 2017 and in subsequent years, for both the technical component of the Medicare physician fee schedule (MPFS) payment as well as the payment under the hospital outpatient prospective payment system (OPPS), the Medicare payment for imaging services that are X-rays taken using film would be reduced by 20%. For imaging services that are X-rays taken using computed radiography technology (cassette-based imaging that uses an imaging plate to create the image involved), the payment for the technical component would be reduced by 7% in the years 2018–2022, and by 10% beginning in 2023 and in subsequent years. The Secretary could use modifiers to implement this requirement under the MPFS. Payment reductions resulting from the application of this provision would be exempt from the budget neutrality condition described above relating to the MPFS.

The provision would eliminate the application of the multiple procedure payment reduction policy to the professional component of imaging services for services provided on or after January 1, 2016, and until the year in which the Secretary conducts and publishes, as part of the MPFS Proposed Rule, the following empirical analysis. The Secretary would conduct and publish an empirical analysis of the Resource-Based Relative Value Scale (RBRVS) Data Manager information to determine what, if any, efficiencies exist within the professional component of imaging services when two or more studies are performed on the same patient on the same day. The empirical analysis would include the following: (1) work sheets and other information detailing which physician work activities performed given the typical vignettes were assigned reduction percentages of 0%, 25%, 50%, 75% and 100%; (2) a discussion of the clinical aspects that informed the assignment of the reduction percentages; (3) an explanation of how the percentage reductions for pre-, intra- and postservice work were determined and calculated; and (4) a demonstration that CMS has consulted with practicing radiologists to gain knowledge of how radiologists interpret studies of multiple body parts on the same individual on the same day.

Section 4004. Treatment of Infusion Drugs Furnished Through Durable Medical Equipment

Background

Although most outpatient prescription drugs and biologics are covered under Medicare Part D, Medicare covers certain drugs and biologics under Part B.[229] Part B drugs and biologics include drugs furnished incident to physician services, immunosuppressive drugs following a Medicare-covered organ transplant, erythropoietin for treatment of anemia for persons with ESRD, oral anti-cancer drugs under certain conditions, and drugs furnished through DME. Generally, Medicare reimburses physicians and other providers, such as hospital outpatient clinics, for Part B drugs and biologics at 106% of the volume weighted average of the average sales price for all

[229] Biologics generally are derived from living organisms rather than inorganic chemical compounds.

drugs billed under the same billing code. Health care providers also receive a separate payment for the administration of Part B drugs and biologics. Some Part B drugs and biologics such as blood products, vaccines, and drugs and biologics furnished through an item of DME are reimbursed differently. Drugs furnished through an item of DME, such as an infusion pump, are reimbursed at 95% of the average wholesale price (AWP) of the drug in effect on October 1, 2003.[230] Several reports have shown that AWP reimbursement methodology exceeds acquisition costs for many drugs administered through DME.[231] However, for infused insulin (furnished through an infusion pump) the acquisition cost exceeded the Medicare payment by 50% during the six quarters included in a recent OIG study.[232]

Provision

This provision would require CMS to reimburse DME suppliers for Medicare Part B infusion drugs and biologicals furnished through DME on the basis of 95% of the current AWP beginning on January 1, 2017 (rather than using the 2003 AWP as in current law).

Section 4005. Extension and Expansion of Prior Authorization for Power Mobility Devices (PMDs) and Accessories and Prior Authorization Audit Limitations

Background

The Medicare Supplementary Medical Insurance Program (Part B) currently covers a wide variety of durable medical equipment (DME), including power wheelchairs and other power mobility devices, if they are medically necessary and are prescribed by a physician. There is a history of fraud and abuse associated with DME and particularly power mobility devices,[233] wherein beneficiaries receive power mobility devices that are not medically necessary or Medicare is charged for equipment that is never delivered. The Secretary has the authority to require prior authorization for certain items of DME, or for DME sold by suppliers who, for example, have a pattern of denied claims. In some cases, a supplier or a beneficiary may request an advanced determination of coverage be made prior to delivery of the item and Medicare being billed for the item.

Using demonstration authority, the Secretary established the Prior Authorization of Power Mobility Devices (PMDs) Demonstration in 2012. The demonstration requires power mobility devices in seven states[234] to receive Medicare prior authorization before beneficiaries receive equipment. The demonstration was extended to an additional 12 states[235] in 2014.

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[230] SSA Sec. 1842(o)(1)(D). The average wholesale price of a drug is a commercially published reference price but not an average price paid by purchasers or charged by wholesalers. AWP is considered a manufacturer's suggested wholesale price to retailers as listed in published drug industry compendia (not defined in statute or regulations).

[231] HHS Office of the Inspector General, *Part B Payments for Drugs Infused through Durable Medical Equipment* (OEI-12-12-00310), February 2013.

[232] HHS Office of the Inspector General, *Implementing OIG Recommendations Could Have Reduced Payments for DME Infusion Drugs by Hundreds of Millions of Dollars* (OEI-12-15-00110), April 2015.

[233] Centers for Medicare & Medicaid Services (CMS), "Medicare Announces New Initiatives on Power Wheelchair Coverage and Payment Policy," press release, April 28, 2004, https://www.cms.gov/Newsroom/MediaReleaseDatabase/Press-releases/2004-Press-releases-items/2004-04-28.html.

[234] The original seven states include California, Illinois, Michigan, New York, North Carolina, Florida, and Texas.

[235] The 12 additional states include Maryland, New Jersey, Pennsylvania, Indiana, Kentucky, Ohio, Georgia,

CMS has indicated that it will end the demonstration on August 31, 2015.[236] It includes all power operated vehicles (scooters), the wheelchair bases (without accessories) for standard power wheelchairs, and certain complex rehabilitative power wheelchairs. However, certain complex rehabilitative power wheelchairs are excluded from the demonstration, specifically, chairs categorized as Group 3, and which support the use of additional accessories requiring a power source to operate.[237]

CMS administers the Medicare program through contracts with private entities that help CMS run the day-to-day activities of Medicare, such as paying reimbursement claims, processing provider and supplier enrollment applications. CMS contracts with several private organizations to assist specifically with protecting the trust funds from making improper payments and to identify, recover, and where appropriate prosecute, waste, fraud, and abuse. Recovery Audit Contractors (RACs) are one such entity; RACs help CMS to identify and collect improper payments made in Medicare's fee-for-service (FFS) program. Other entities that assist CMS with waste, fraud, and abuse oversight include Zone Program Integrity Contractors (ZPICs), Medicare Drug Integrity Contractors (MEDICs), Comprehensive Error Rate Testing (CERT) contractors, and the Supplemental Medical Review Contractor.

Provision

The provision would prohibit a DME claim from being subject to a RAC audit if the claim had received either (a) a provisional affirmation under the Secretary's advanced determination authority, or (b) prior authorization under the Power Mobility Devices Prior Authorization Demonstration. The DME claim would, however, still be subject to audits for potential fraud, inappropriate utilization, changes in billing patterns, or for information that could have been considered during an advanced determination.

Not later than 90 days after the date of enactment, the Secretary would be required to extend the Power Mobility Device Prior Authorization Demonstration at least through August 31, 2018, and begin to expand the demonstration, as appropriate, to include additional power mobility devices and accessories, and expand the demonstration to additional states or geographic areas, as appropriate.

The Secretary would be authorized to use Medicare Trust Funds and other funds available to the Secretary as appropriate to fund the continuation and expansion of the Power Mobility Device and Accessories Prior Authorization Demonstration.

Section 4006. Civil Monetary Penalties for Violations Related to Grants, Contracts, and Other Agreements

Background

Title XI of the Social Security Act (SSA) identifies Medicare- and Medicaid-related anti-fraud provisions, which impose penalties and exclusions from federal health care programs on

Tennessee, Louisiana, Missouri, Washington, and Arizona.

[236] For more information, see https://www.cms.gov/Research-Statistics-Data-and-Systems/Monitoring-Programs/Medicare-FFS-Compliance-Programs/Medical-Review/PADemo.html.

[237] For more information about the specific mobility devices included and excluded from the demonstration, see https://www.cms.gov/Research-Statistics-Data-and-Systems/Monitoring-Programs/Medicare-FFS-Compliance-Programs/CERT/Downloads/Fact-Sheet-new-8112.pdf.

individuals and other entities that engage in certain types of misconduct.[238] Under SSA Section 1128A, the Department of Health and Human Services Office of Inspector General (OIG) is authorized to impose civil penalties and assessments on a person, including an organization, agency, or other entity, who engages in various types of improper conduct with respect to federal health care programs, including the imposition of penalties against a person who knowingly presents or causes to be presented to a federal or state employee or agent certain false or fraudulent claims.[239] For example, penalties apply to services that were not provided as claimed, or claims that were part of a pattern of providing items or services that a person knows or should know are not medically necessary. In addition, certain payments made to physicians to reduce or limit services are also prohibited. SSA Section 1128A provides for monetary penalties of up to $10,000 for each item or service claimed, up to $50,000 under certain additional circumstances, as well as treble damages.

Under SSA Section 1128, exclusion from federal health programs is mandatory under certain circumstances, and permissive in others. Exclusions are mandatory for those convicted of certain offenses, including (1) a criminal offense related to the delivery of an item or service under Medicare, Medicaid, or a state health care program; (2) a criminal offense relating to neglect or abuse of patients in connection with the delivery of a health care item or service; and (3) a felony relating to the unlawful manufacture, distribution, prescription, or dispensing of a controlled substance. OIG has permissive authority to exclude an entity or an individual from a federal health program under a number of circumstances, including conviction of certain misdemeanors relating to fraud, theft, embezzlement, breach of fiduciary duty, or other financial misconduct; a conviction based on an interference with or obstruction of an investigation into a criminal offense; and revocation or suspension of a health care practitioner's license for reasons bearing on the individual's or entity's professional competence, professional performance, or financial integrity.

Provision

This provision would make any person (including an organization, agency, or other entity, but excluding beneficiaries) who knowingly commits the following improper conduct related to grants, contracts, or other agreements funded by the Department of Health and Human Services subject to civil monetary penalties (CMPs):

1. knowingly presents or causes to be presented a specified claim that the individual knows or should know was false would be subject, in addition to other penalties prescribed by law, to CMPs of up to $10,000 for each specified claim (in addition, individuals determined to have presented these specified claims would also be subject to assessments of up to three times the amount of the specified claim in lieu of damages sustained by the United States or a specified state agency);
2. knowingly makes, uses or causes to be made or used a false statement, omission, or misrepresentation of a material fact in an application, proposal, bid, progress report, or other document required to receive or retain funding for HHS funded grants, contracts, or other agreements would be subject, in addition to other penalties prescribed by law, to

[238] "Federal health care program" is defined as (1) any plan or program that provides health benefits, whether directly, through insurance, or otherwise, which is funded directly, in whole or in part, by the United States Government [not including health insurance provided to federal government employees] or (2) any state health care program, as defined in Section 1128(h). Federal health care programs include Medicare and Medicaid.

[239] Civil penalties do not apply to beneficiaries under SSA Section 1128A; a beneficiary is defined as an individual who is eligible to receive items or services for which payment may be made under a federal health care program, but excludes any providers, suppliers, or practitioners.

CMPs of up to $50,000 for each false statement, omission, or misrepresentation of material fact (in addition, individuals determined to have made, used, or caused to be made these false or fraudulent specified claims would also be subject to assessments of up to three times the total amount of the funds or property obligated to the Secretary in lieu of damages sustained by the United States or a specified state agency);

3. knowingly makes, uses, or causes to be made or used a false record or statement material to a false or fraudulent specified claim under an HHS funded grant, contract, or other agreement would be subject, in addition to other penalties prescribed by law, to CMPs of up to $50,000 for each false record or statement (in addition, individuals determined to have made, used, or caused to be made these false or fraudulent specified claims would also be subject to assessments of up to three times the amount of the specified claim in lieu of damages sustained by the United States or a specified state agency);
4. knowingly makes, uses, or causes to be made or used, a false record or statement material to an obligation to pay or transmit funds or property to the HHS Secretary (the Secretary) or knowingly conceals or knowingly and improperly avoids or decreases an obligation to pay or transmit funds or property related to an HHS funded grant, contract, or other agreement would be subject, in addition to other penalties prescribed by law, to CMPs of up to $50,000 for each false record or statement or $10,000 for each day that the individual knowingly conceals or knowingly and improperly avoids or decreases an obligation to pay (in addition, individuals determined to have made, used, or caused to be made these false or fraudulent specified claims would also be subject to assessments of up to three times the total amount of the funds or property obligated to the Secretary in lieu of damages sustained by the United States or a specified state agency); and
5. fails to grant timely access, upon reasonable request (as defined in regulations promulgated by the Secretary), to the OIG for conducting audits, investigations, evaluations, or other statutory functions related to grants, contracts, and other agreements with HHS would be subject, in addition to other penalties prescribed by law, to CMPs of up to $15,000 for each day of the failure to grant timely access.

In addition to CMPs, this provision would authorize the Secretary to exclude individuals found to have knowingly committed the improper conduct related to HHS-funded grants, contracts, and other agreements from participation in federal health care programs and to direct state agencies also to exclude these individuals from state health programs. Under this provision the Secretary would be subject to SSA Section 1128A(c), (d), and (g) provisions.

This provision would also authorize the Secretary to retain the CMPs and assessments recovered as a result of this provision and to use those amounts to reimburse the cost of conducting investigations and audits of individuals with HHS-funded grants, contracts, or other agreements and also to monitor related compliance plans that result when CMPs or assessments under this provision are ordered by courts, voluntarily agreed to by the payer, or otherwise. The Secretary would be required under this provision to deposit funds from CMPs and assessments as credits to the appropriations from which they were originally paid or to appropriations for similar purposes currently available. These appropriations would be available for obligation for one year from the date of deposit.

This provision would identify the following terms applicable to SSA Section 1128A(o), (p), (q) and (r):

1. Department would mean the Department of Health and Human Services.

2. Material would mean having a natural tendency to influence, or be capable of influencing, the payment or receipt of money or property.
3. Other agreement would include a cooperative agreement, scholarship, fellowship, loan, subsidy, payment for a specified use, donation agreement, award, or sub-award (regardless of whether one or more of the persons entering into the agreement was a contractor or subcontractor).
4. Program beneficiary would mean, in the case of grant, contract, or other agreement designed to accomplish the objective of awarding or otherwise furnishing benefits or assistance to individuals and for which the Secretary provides funding, an individual who applies for, or who receives, such benefits or assistance from a grant, contract, or agreement. Program beneficiary would not include, with respect to a grant, contract, or other agreement, an officer, employee, or agent of an individual or entity that receives an HHS-funded grant or enters into a contract or other agreement.
5. Recipient includes a sub-recipient or subcontractor.
6. Specified claim means any application, request, or demand under a grant, contract, or other agreement for money or property, whether or not the United States or a specified state agency has title to the money or property, that is not a claim [under SSA Section 1128A(i)(2), claim is defined as an application for payments for items and services under a federal health care program] and that (A) was presented or caused to be presented to an officer, employee, or agent of HHS or any specified state agency; or (B) was made to a contractor, grantee, or any other recipient if the money or property was to be spent or used on HHS's behalf or to advance an HHS program or interest, and if HHS provides or has provided any portion of the money or property requested or demanded; or will reimburse the contractor, grantee, or other recipient for any portion of the money or property which is requested or demanded.
7. Specified state agency means an agency of state government established or designated to administer or supervise the administration of a grant, contract, or other agreement funded in whole or in part by the Secretary.

In addition, the term obligation as used in this provision would mean an established duty, whether fixed or not fixed, arising from an express or implied contractual, grantor-grantee, or licensure-licensee relationship, for a fee-based or similar relationship, from statute to regulation, or from the retention of any overpayment.

This provision also would contain the following conforming amendments:

1. by (A) adding "specified claims" to "claims" in SSA Section 1128A(d), and (B) by adding "specified claims" to "claims" in SSA Sec. 1128A(e),
2. by inserting in SSA Section 1128A(f), "or specified claims", "or, with respect to a person described in subsection (o), the person", and "that are not received by OIG under subsection (q) as reimbursement." As well as by inserting "(or, in the case of a penalty or assessment under subsection (o), by a specified State agency (as defined in subsection (r)(7))".

Subtitle B—Other Reforms

Section 4041. SPR Drawdown

Background

The Strategic Petroleum Reserve (SPR) was authorized by Congress as part of the Energy Policy and Conservation Act of 1975 (42 U.S.C. 6241) (EPCA). Congress authorized the SPR as a response to rising oil prices and petroleum product shortages related to the oil embargo established against the United States, the Netherlands, and Canada by the Organization of the Arab Petroleum Exporting Countries (OAPEC). The SPR is authorized to hold up to 1 billion barrels of oil, although it currently holds 691 million barrels.

The OAPEC embargo also fostered the creation of the International Energy Agency (IEA). The IEA was established to enable oil-importing nations to develop plans and measures for emergency responses to energy crises. IEA member countries, including the United States, are committed to maintaining oil stocks (inventories) equivalent to 90 days of their prior year's net imports, developing programs for demand restraint in the event of emergencies, and agreeing to participate in the allocation of oil deliveries to a shortage among IEA members.[240]

The President may authorize an SPR drawdown upon determining that a severe oil supply interruption exists nationally, or internationally, or is imminent. Under this criterion, drawdowns took place after Hurricane Katrina in 2005, and after the rebellion in Libya in 2011. The Secretary of Energy also has limited authority to release oil from the SPR for a test drawdown, most recently in 2014.

Provision

The provision calls upon the Secretary of Energy to drawdown and sell 4 million barrels of oil in FY2018, 5 million barrels in FY2019, 8 million barrels in FY2020 and FY2021, 10 million barrels in FY2022, and 15 million barrels in each of FY2023 through FY2025. The SPR sales are to occur unless the drawdowns would result in remaining stocks of less than 90 days of emergency reserves. While this requirement ensures a flow of funds to the U.S. Treasury, the actual amount is not predictable. The revenue earned from a drawdown is the product of the quantity of oil times the price of that oil. Oil in the reserve is of many types and quality levels, as determined by viscosity and sulfur content. It is difficult to specify in advance what grade of oil would actually be drawn down under the provision, or in an emergency. Maintaining different grades of crude oil in the SPR allows for flexibility in meeting emergency requirements. Also, each grade of crude oil has its own price based on the relative demand for each type of crude oil based on market conditions. These prices cannot be accurately forecasted.

Using the IEA criterion of 90 days of net import replacement, today the United States exceeds the requirement. In 2014, the United States had net imports of crude oil and petroleum products of 5 million barrels per day. With the SPR holding 691 million barrels, this implies the equivalent of 138 days of import replacement is currently held. However, if the term net imports is limited to only crude oil, U.S. imports in 2014 were 7 million barrels per day, yielding 98 days of import replacement.[241]

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[240] See CRS Report R42460, *The Strategic Petroleum Reserve: Authorization, Operation, and Drawdown Policy*.

[241] The United States imports crude oil, which is refined into petroleum products and exported, leading to a lower value for net imports of crude oil and petroleum products.

Whether these import replacement levels will be greater, or lesser, from FY2018 to FY2025 is unknown. It is uncertain how high domestic production of crude oil, as well as consumption, will be from FY2018 through FY2025. Domestic crude oil production depends on the longevity of light tight oil fields, which have accounted for all the growth in U.S. production, and the price of oil, which determines the financial viability of oil development and production. Domestic oil consumption depends on consumer income levels, petroleum product prices, and regulations on fuel economy standards, as well as other factors.

Subtitle C—Miscellaneous

Section 4061. Lyme Disease and Other Tick-Borne Diseases

Backround

The Secretary is given broad and general authority to conduct research related to disease under Title III of the PHSA. Specifically, the Secretary is required to conduct research, investigations, experiments, demonstrations, and studies relating to the causes, diagnosis, treatment, control, and prevention of disease.[242]

Provision

The provision would add to Title III of the PHSA a new Part W, Lyme Disease and Other Tick-Borne Diseases, with new Sections 399OO (Research), 399OO-1 (Working Group), and 399OO-2 (Strategic Plan). No additional funds would be authorized to carry out Section 4081; the section would have to be carried out using funds otherwise available for this purpose.

New PHSA Section 399OO would require the Secretary to conduct epidemiological, basic, clinical, and translational research on Lyme and other tick-borne diseases. It would also require the Secretary to include information about this research, and progress in improving the outcomes of Lyme and other tick-borne diseases, in the biennial report of the Director of the National Institutes of Health.[243]

New PHSA Section 399OO-1 would require the Secretary to establish a permanent interagency working group (the Working Group) to review all efforts within HHS that relate to Lyme and other tick-borne diseases in order to coordinate and minimize overlap of the efforts, and look at research priorities. The section describes requirements related to the Working Group's membership, responsibilities, meeting frequency, and reporting. The Working Group would be subject to the Federal Advisory Committee Act (FACA).

New PHSA Section 399OO-2 would require the Secretary, not later than three years after enactment and every five years thereafter, to submit to Congress a strategic plan for Lyme and other tick-borne disease research. This strategic plan would be informed by the Working Group's summary of research in this area and would include, among other things, a plan to improve outcomes, diagnosis, treatment, and prevention of Lyme and other tick-borne diseases.

[242] PHSA Section 301 et seq.; 42 U.S.C. §241 et seq.

[243] The biennial report of the NIH Director is required by PHSA Section 403, "Biennial Reports of Director of NIH" (42 U.S.C. §283).

Section 4062. Outreach to Historically Black Colleges and Universities

Provision

The provision would require the Secretary of HHS to ensure that health professionals from underrepresented populations—such as those at historically black colleges and universities, Hispanic-serving institutions, Native American colleges and rural colleges—are aware of research opportunities under this legislation.[244]

[244] Amendment 8 (Jackson Lee) to H.R. 6 added Sec. 4062; it was approved by voice vote.

Appendix A. Guidance, Reports, and Regulations/Rulemaking That H.R. 6 Would Require

Table A-1. Guidance, Reports, and Regulations/Rulemaking That H.R. 6 Would Require

(H.R. 6, as passed by the House on July 10, 2015.)

Section	Guidance	Report[a]	Rule
Sec. 1002. Prize Competitions		X	
Sec. 1022. Increasing accountability at the National Institutes of Health.		IOM	
Sec. 1023. Reducing administrative burdens of researchers.		X	
Sec. 1042. Report.		X	
Sec. 1121. Clinical trial data system.		GAO	
Sec. 1122. National Neurological Diseases Surveillance System.		X	
Sec. 1124. Accessing, sharing, and using health data for research purposes.			X
Sec. 1141. Council for 21st Century Cures.		X	
Sec. 2001. Development and use of patient experience data to enhance structured risk benefit assessment framework.	X		
Sec. 2021. Qualification of drug development tools.	X	X	
Sec. 2041. Precision medicine guidance and other programs of Food and Drug Administration.	X		
Sec. 2061. Broader application of Bayesian statistics and adaptive trial designs.	X		
Sec. 2062. Utilizing evidence from clinical experience.	X		
Sec. 2063. Streamlined data review program.	X		
Sec. 2083. Finalizing draft guidance on expanded access.	X		
Sec. 2102. Facilitating responsible communication of scientific and medical developments.	X		
Sec. 2121. Approval of certain drugs for use in a limited population of patients.	X		
Sec. 2122. Susceptibility test interpretive criteria for microorganisms.		X	
Sec. 2123. Encouraging the development and use of new antimicrobial drugs.		X, GAO	X
Sec. 2142. Review of processes and consistency of ACIP recommendations.		X	
Sec. 2152. Reauthorization of rare pediatric disease priority review voucher incentive program.		GAO	
Sec. 2181. Enhancing combination products review.	X		
Sec. 2201. Priority review for breakthrough devices.	X		
Sec. 2221. Third-party quality system assessment.	X	X	
Sec. 2223. Training and oversight in least burdensome appropriate means concept.	X		
Sec. 2224. Recognition of standards.	X		

Section	Guidance	Report[a]	Rule
Sec. 2227. Humanitarian device exemption application.	X		
Sec. 2228. CLIA waiver study design guidance for in vitro diagnostics.	X		
Sec. 2242. Applicability and inapplicability of regulation.			X
Sec. 2261. Protection of human subjects in research; applicability of rules.	X	X	X
Sec. 2262. Use of non-local institutional review boards for review of investigational device exemptions and human device exemptions.	X		X
Sec. 2281. Silvio O. Conte Senior Biomedical Research Service.		X	
Sec. 2285. Hiring authority for scientific, technical, and professional personnel.		X	
Sec. 3001. Ensuring interoperability of health information technology.	X	X	X
Sec. 3141. Programs to prevent prescription drug abuse under Medicare parts C and D.		X	X
Sec. 4081. Lyme disease and other tick-borne diseases.		X	

Source: Compiled by CRS from H.R. 6, the 21st Century Cures Act, House Committee on Rules Committee Print 114-22.

a. Unless otherwise noted, the Department of Health and Human Services would be responsible for these reports. In some cases, such as Sec. 1002, multiple reports would be required.

Appendix B. List of Abbreviations

ACA	Patient Protection and Affordable Care Act (P.L. 111-148)
ACIP	Advisory Committee on Immunization Practices
ASC	Ambulatory Surgical Centers
ATRA	American Taxpayer Relief Act of 2012 (P.L. 112-240)
AWP	Average Wholesale Price
BBEDCA	Balanced Budget and Emergency Deficit Control Act of 1985 (P.L. 99-177)
CAC	Carrier Advisory Committees
CAN	Cures Acceleration Networks
CAO	Competitive Acquisition Ombudsman
CDC	Centers for Disease Control and Prevention
CFR	Code of Federal Regulations
CLIA	Clinical Laboratory Improvement Amendments
CMS	Centers for Medicare & Medicaid Services
COW	Certificate of Waiver
DME	Durable Medical Equipment
EPCA	Energy Policy and Conservation Act of 1975 (P.L. 94-163)
FACA	Federal Advisory Committee Act (P.L. 92-463)
FDA	Food and Drug Administration
FDAAA	Food and Drug Administration Amendments Act of 2007 (P.L. 110-85)
FDASIA	The Food and Drug Administration Safety and Innovation Act of 2012 (P.L. 112-144)
FDP	Federal Demonstration Partnership
FFDCA	Federal Food, Drug, and Cosmetic Act (P.L. 75-717)
FFS	Fee for Service
GAO	Government Accountability Office
GSA	General Services Administration
HCPCS	The Health Care Common Procedure Coding System
HDE	Humanitarian Device Exemption
HHS	Department of Health and Human Services
HIPAA	Health Insurance Portability and Accountability Act of 1996 (P.L. 104-191)
HITECH	Health Information Technology for Economic and Clinical Health (P.L. 111-5, Division A, Title XIII and Division B, Title IV)
IACUC	Institutional Animal Care and Use Committee
IC	Institutes and Centers
IEA	International Energy Agency
IND	Investigational New Drug
IRB	Institutional Review Board
LCD	Local Coverage Determinations

LPAD	Limited Population Antibacterial Drug
MA	Medicare Advantage
MAC	Medicare Administrative Contractors
MAPD	Medicare Advantage Prescription Drug
MDIC	Medicare Drug Integrity Contractors
MedPAC	Medicare Payment Advisory Commission
MMA	Medicare Prescription Drug, Improvement, and Modernization Act (P.L. 108-173)
MPFS	Medicare Physician Fee Schedule
MPPR	Multiple Procedure Payment Reduction
MTM	Medication Therapy Management
NCATS	National Center for Advancing Translational Sciences
NCD	National Coverage Decisions
NDA	New Drug Application
NIH	National Institutes of Health
NPRN	National Pediatric Research Network
NVP	National Vaccine Program
OAPEC	Organization of the Arab Petroleum Exporting Countries
OECD	Organisation for Economic Co-operation and Development
OIG	Office of Inspector General
OIRA	Office of Information and Regulatory Affairs
OMB	Office of Management and Budget
OMO	Office of Medicare Ombudsman
OMS	Overutilization Monitoring System
ONC	Office of the National Coordinator for Health Information Technology
OPPS	Outpatient Prospective Payments
OT	Other Transaction
PDP	Prescription Drug Plans
PE	Practice Expense
PHSA	Public Health Service Act (P.L. 78-410)
PRA	Paperwork Reduction Act of 1980 (P.L. 104-13)
RBRVS	Resource-Based Relative Value Scale
RUC	American Medical Association/Specialty Society Relative Value Scale Update Committee
RVU	Relative Value Units
SPR	Strategic Petroleum Reserve
SSA	Social Security Act (P.L. 74-271)
USC	United States Code
VCF	Vaccines for Children Program

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