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The 21st Century Cures Act (Division A of P.L. 114-255)

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Summary

The 21st Century Cures Act (P.L. 114-255) was signed into law on December 13, 2016, by President Barack Obama. On November 30, 2016, the House passed the House amendment to the Senate amendment to H.R. 34, the 21st Century Cures Act, on a vote of 392 to 26. The bill was then sent to the Senate where it was considered and passed, with only minor technical modification, on December 7, 2016, on a vote of 94 to 5.

The law consists of three divisions:

- Division A—21st Century Cures Act;
- Division B—Helping Families in Mental Health Crisis; and
- Division C—Increasing Choice, Access, and Quality in Health Care for Americans.

CRS has published a series of reports on this law, one on each Division. This is the report for Division A of the law.

This report provides a brief summary of each provision of the 21st Century Cures Act (Division A of P.L. 114-255), by title, subtitle, and section. The Division includes five titles, as follows: (1) Innovation projects and state responses to opioid abuse; (2) Discovery; (3) Development; (4) Delivery; and (5) Savings.

Title I provides funding for biomedical research, including the Precision Medicine Initiative (PMI) and the Cancer Moonshot Initiative, for the opioid crisis response, and for the Food and Drug Administration (FDA) to support certain new activities authorized by the law.

Title II, consisting of seven subtitles, requires or authorizes a number of activities to support biomedical research, including the reauthorization of the National Institutes of Health (NIH) and the reform of that agency through numerous administrative, reporting, and data access provisions. The Title includes provisions that support young investigators funded by NIH; pediatric research; collaborative research such as research on neurological disease; and precision medicine efforts, and specifically the PMI.

Title III, consisting of ten subtitles, focuses on modifying the drug and device approval pathways at the FDA to support innovation, and specifically includes provisions that support patient-focused drug development and streamlined and clarified pathways to approval for drugs, combination products, antimicrobials, Orphan drugs, drugs for rare disease, and regenerative therapies. This Title also contains provisions making modifications to the medical device approval pathway and reforms to the FDA's hiring process. Finally, it addresses FDA's regulation of medical countermeasure and vaccine development.

Title IV focuses on health care delivery, and includes provisions that together address the federal policies to promote the adoption and use of electronic health record (EHR) technology, as well as a handful of Medicare delivery provisions addressing telehealth services in Medicare, site-of-service price transparency for certain Medicare services, Local Coverage Determinations (LCDs) under Medicare, and a technology and pharmaceutical ombudsman for Medicare.

Title V provides savings for the Division, and includes Medicare and Medicaid savings; Patient Protection and Affordable Care Act (ACA, P.L. 111-148, as amended) savings, including Prevention and Public Health Fund (PPHF) and territory funding; and savings from the Strategic Petroleum Reserve (SPR) drawdown.

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Introduction

The 21st Century Cures Act (P.L. 114-255) was signed into law on December 13, 2016, by President Barack Obama. On November 30, 2016, the House passed the House amendment to the Senate amendment to H.R. 34, the 21st Century Cures Act, on a vote of 392 to 26. The bill was then sent to the Senate where it was considered and passed, with only minor technical modification, on December 7, 2016, on a vote of 94 to 5.[1]

The law consists of three divisions:

- Division A—21st Century Cures Act;
- Division B—Helping Families in Mental Health Crisis; and
- Division C—Increasing Choice, Access, and Quality in Health Care for Americans.

CRS has published a series of reports on this law, one on each Division. This is the report for Division A of the law.[2]

Division A of the law provides funding for biomedical research—including the Precision Medicine Initiative (PMI) and the Cancer Moonshot Initiative—and for the opioid crisis response; modifies Food and Drug Administration (FDA) pathways for the approval of regulated medical products; and makes a number of reforms to the National Institutes of Health (NIH). Division A of the law also includes and builds on provisions from both the previously passed House bill, H.R. 6 (The 21st Century Cures Act, passed in July 2015), and a package of Senate medical innovation bills that were considered in the early part of 2016.

As noted, both the House and the Senate considered previous legislation to support medical innovation, primarily through reforms to the NIH and changes to the drug, biologic and device approval pathways at the FDA. On February 3, 2015, Senators Lamar Alexander and Patty Murray, chairman and ranking Member of the Committee on Health, Education, Labor and Pensions, announced the start of a bipartisan initiative to "examine the process for getting safe treatments, devices and cures to patients and the roles of the [FDA] and the [NIH] in that process."[1] This initiative culminated in a package of 19 bipartisan bills that were reported out of the Senate Health, Labor, Education, and Pensions (HELP) Committee in a series of three executive sessions held on February 9, 2016; March 9, 2016; and April 6, 2016. One of these 19 bills, The Adding Zika Virus to the FDA Priority Review Voucher Program Act (S. 2512), subsequently was passed by both chambers and signed into law on April 19, 2016 (P.L. 114-146).

The Senate's medical innovation package was that chamber's companion effort to the House's 21st Century Cures initiative, which resulted in the House passage of H.R. 6, the initial version of the 21st Century Cures Act, on July 10, 2015, on a vote of 344 to 77. H.R. 6 was the result of a series of hearings and roundtable meetings hosted by the House Energy and Commerce Committee dating back to spring 2014. The hearings and roundtables focused on a broad range of topics, including modernizing clinical trials, incorporating patient perspectives into medical research and

[1] The Congressional Budget Office's (CBO) score of H.R. 34 (Rules Committee Print 114-67, as amended by Amendment Number 5) is available at https://www.cbo.gov/sites/default/files/114th-congress-2015-2016/costestimate/H.R.34amendment5.pdf.

[2] For information on Division B, see CRS Report R44718, *The Helping Families in Mental Health Crisis Reform Act of 2016 (Division B of P.L. 114-255)*, coordinated by Erin Bagalman.

regulatory processes, precision/personalized medicine, digital health care, and more. While it consisted of many different provisions, H.R. 6 was primarily focused on efforts to increase strategic investments in medical research at NIH and change some aspects of how the FDA executes its regulatory oversight mission with regard to the review and approval of new drugs, biologics, and medical devices.

This report provides a brief summary of each provision of the 21st Century Cures Act (Division A of P.L. 114-255), by title, subtitle, and section.[3] The Division includes five titles, as follows: (1) Innovation projects and state responses to opioid abuse; (2) Discovery; (3) Development; (4) Delivery; and (5) Savings. Most provision summaries include a brief background of current law in addition to a description of the new provision of law. Throughout the report, clarity takes priority over consistency. For example, some summaries are more detailed than others where such detail is necessary to highlight important changes. A list of acronyms used throughout this report can be found in **Appendix** of this report.

Table 1. CRS Experts List

Erin Bagalman	Opioid epidemic funding
Cliff Binder	Medicaid, Fraud and abuse
Kirsten J. Colello	Medicaid supplemental needs trusts
Agata Dabrowska	FDA drug regulation
Susannah Gopalan	Telehealth in Medicare
Frank Gottron	Medical countermeasures innovation
Jim Hahn	Medicare Part B, Site-of-service price transparency
Elayne J. Heisler	Health care workforce and education
Judith Johnson	National Institutes of Health, FDA medical device and biologics regulation
Sarah A. Lister	Antimicrobial and vaccine development, Collaborative research, PPHF
Annie Mach	ACA territory funding
Paulette Morgan	Durable Medical Equipment
Robert Pirog	Strategic Petroleum Reserve drawdown
C. Stephen Redhead	Data privacy, Health Information Technology, Regulation of medical software
Amanda Sarata	Precision medicine, Clinical laboratory regulation

Title I-Innovation Projects and State Responses to Opioid Abuse

Section 1001. NIH Innovation Projects

The National Institutes of Health (NIH) is the lead federal agency charged with performing and supporting biomedical and behavioral research. It also has major roles in training biomedical

[3] Three sections are excluded from this report due to their technical, non-substantive nature: Section 1004 (Budgetary treatment), Section 3101 (Technical corrections), and Section 3102 (Completed studies).

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.[4] 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%.[5] NIH funding in FY2015 was 22% lower than the FY2003 level, the peak of the doubling period in constant 2012 dollars.[6]

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."[7] The United States continues to be the top supporter of both public and industry medical research.[8] 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 about the U.S. global position in biomedical research investment have made frequent calls for increased support for research at 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."[9] 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.[10] 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.[11]

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[4] For further information, see CRS Report R43341, *NIH Funding: FY1994-FY2017*, by Judith A. Johnson.

[5] 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.

[6] For further information, see CRS Report R43341, *NIH Funding: FY1994-FY2017*, by Judith A. Johnson.

[7] 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.

[8] 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).

[9] 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.

[10] Ibid., p. 5775.

[11] 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.

The Advisory Committee to the Director of NIH, authorized under PHSA Section 222, provides "advice on matters pertinent to NIH mission responsibilities in the conduct and support of biomedical research."[12]

Provision

Section 1001 establishes the "NIH Innovation Account" in the Treasury, to which specified amounts are transferred for each of FY2017 through FY2026. Such amounts from the account are authorized to be appropriated to the NIH Director for the purpose of carrying out the NIH Innovation Projects. Amounts appropriated from this account are available until expended. Specifically, the provision authorizes appropriations to support

- the Precision Medicine Initiative, specified amounts for FY2017 through FY2026, total not to exceed $1.455 billion;
- the Brain Research through Advancing Innovative Neurotechnologies Initiative (BRAIN Initiative), specified amounts for FY2017 through FY2026, total not to exceed $1.511 billion;
- cancer research, specified amounts for FY2017 through FY2023, total not to exceed $1.8 billion; and
- regenerative medicine using adult stem cells, specified amounts for FY2017 through FY2020 that total $30 million; no funds are to be appropriated for this activity after FY2020. This research is to be undertaken in coordination with the FDA.

Within six months of enactment, the NIH Director must submit to the specified congressional committees a work plan including the proposed allocation of funds authorized to be appropriated for each year, FY2017 through FY2026, for the NIH Innovation Projects. Prior to submitting the work plan, the NIH Director must seek recommendations on the allocations of funds and the contents of the proposed work plan from the Advisory Committee to the Director of NIH. The work plan must include recommendations from this Advisory Committee, the amount of money to be obligated or expended in each fiscal year for each NIH Innovation Project, a description and justification of each such project, and a description of how each such project supports the strategic research priorities identified in the NIH Strategic Plan.

Not later than October 1 of each year, FY2018 through FY2027, the Director of NIH must submit to the specified congressional committees a report including the amount of money obligated or expended in the prior fiscal year for each NIH Innovation Project, a description of any such project using funds provided by this section, and whether such projects are advancing the strategic research priorities identified in the NIH Strategic Plan. The specified House and Senate committees may request an update on the allocation of funding under this section or the description of the NIH Innovation Projects, which the NIH Director must provide in the form of additional reports or testimony.

Section 1001 specifies that these funds may be used only for NIH Innovation fund projects (notwithstanding any transfer authority in any appropriations act).

The section also specifies that amounts in the account are not available until appropriated in subsequent appropriations acts. Notably, the amounts subsequently appropriated (i.e., the budget authority and the resulting outlays) for FY2017 through FY2026, up to the amounts transferred, are to be subtracted from any cost estimates provided for purposes of budget controls. Effectively,

[12] NIH, Advisory Committee to the Director, Charter, at http://acd.od.nih.gov/charter.htm.

the appropriations from the account will not be counted against any spending limits, such as the statutory discretionary spending limits; that is, the amounts appropriated from the account will be considered outside those limits for FY2017 through FY2026.

Section 1001 sunsets on September 30, 2026.

Section 1002. FDA Innovation Projects

The Food and Drug Administration (FDA) regulates the safety of foods (including dietary supplements), cosmetics, and radiation-emitting products; the safety and effectiveness of drugs, biologics (e.g., vaccines), and medical devices; and public health aspects of tobacco products.

FDA's budget (i.e., its total program level) has two funding streams: annual appropriations (i.e., discretionary budget authority, or BA) and industry user fees. In FDA's annual appropriations, Congress sets both the total amount of appropriated funds and the total amount of user fees that the agency is authorized to collect and obligate for that fiscal year. Appropriated funds are largely for the Salaries and Expenses account, with a much smaller amount for the Buildings and Facilities account. The different user fees contribute only to the Salaries and Expenses account.

Between FY2012 and FY2016, FDA's total program level increased from $3.832 billion to $4.745 billion. Although congressionally appropriated funding increased by 9% over that time period, user fee revenue increased more than 50%. In FY2016, user fees accounted for 42% of FDA's total program level.[13]

The Science Board to the FDA is an advisory committee that provides "advice to the Commissioner and other appropriate officials on specific complex scientific and technical issues important to FDA and its mission, including emerging issues within the scientific community." Among other things, the Science Board is also tasked with providing, where requested, "expert review of Agency sponsored intramural and extramural scientific research programs."

Provision

Section 1002 establishes the "FDA Innovation Account," to which a total of $500 million is authorized to be transferred over a nine-year period (FY2017-FY2025).[14] It specifies that amounts in the account are not available until appropriated in subsequent appropriations acts and that once made available, these amounts are available until expended. The amounts from the account are authorized to be appropriated to the FDA Commissioner for the purpose of carrying out the FDA Innovation Projects specified as activities under subtitles A through F of Title III (e.g., Subtitle A—Patient Focused Drug Development, Subtitle B—Advancing New Drug Therapies, Subtitle F—Medical Device Innovations), as well as Section 3073 of this Act establishing FDA Intercenter Institutes; these activities are described later in this report.

The amounts subsequently appropriated (i.e., the budget authority and the resulting outlays) for FY2017 through FY2025, up to the amounts transferred, are to be subtracted from any cost estimates provided for purposes of budget controls. Effectively, the appropriations from the account will not be counted against any spending limits, such as the statutory discretionary

[13] CRS Report R44576, *The Food and Drug Administration (FDA) Budget: Fact Sheet*, by Agata Dabrowska and Susan Thaul.

[14] For each of fiscal years 2017 through 2025, the following amounts are authorized to be transferred to the FDA Innovation Account: $20 million in FY2017; $60 million in FY2018; $70 million in FY2019; $75 million in FY2020; $70 million in FY2021; $50 million in FY2022; $50 million in FY2023; $50 million in FY2024; $55million in FY2025.

spending limits; that is, the amounts appropriated from the account will be considered outside those limits for FY2017 through FY2025.

Within six months of enactment, the FDA Commissioner is required to submit to the specified congressional committees a work plan including the proposed allocation of funds authorized to be appropriated for each fiscal year (FY2017 through FY2025) for the FDA Innovation Projects. Prior to submitting the work plan, the FDA Commissioner must seek recommendations on the allocations of funds and the contents of the proposed work plan from the Science Board. The work plan must include recommendations from the Science Board, the amount of money to be obligated or expended in each fiscal year for each FDA Innovation Project, and a description and justification of each such project.

Section 1002 requires the FDA Commissioner, not later than October 1 of each fiscal year 2018 through 2026, to submit to the specified congressional committees a report including the amount of money to be obligated or expended in each fiscal year for each FDA Innovation Project, a description of any such project using funds provided by this section, and how the activities are advancing public health. The specified House and Senate committees may request an update on the allocation of funding under this section or the description of the FDA Innovation Projects, which the FDA Commissioner must provide in the form of additional reports or testimony.

Section 1002 specifies that these funds may be used only for FDA Innovation fund projects (notwithstanding any transfer authority in any appropriations act).

Section 1002 sunsets on September 30, 2025.

Section 1003. Account for the State Response to the Opioid Abuse Crisis

The Substance Abuse and Mental Health Services Administration (SAMHSA) administers block grants authorized by PHSA Title XIX and numerous other grants authorized by PHSA Title V, as well as other activities. Each state that receives a block grant from SAMHSA is required to submit to the HHS Secretary a report about block grant funds received in the preceding fiscal year—including the purposes for which funds were expended, the state's activities under the block grant, and the recipients of block grant funds.

Provision

Section 1003 establishes the "Account for the State Response to the Opioid Abuse Crisis" in the Treasury, to which $500 million is transferred for each of FY2017 and FY2018. Such amounts from the account are authorized to be appropriated to the HHS Secretary for use as grants to support state responses to opioid abuse. Specifically, the provision authorizes appropriations to support two categories of grants to states: (1) grants "for the purpose of addressing the opioid abuse crisis" and (2) grants for activities that supplement opioid-related activities undertaken by the state agency that administers the substance abuse block grant.

Section 1003 requires that such funds (1) shall not be used for any other purpose (notwithstanding any transfer authority in any appropriations act) and (2) shall be subject to the same requirements as SAMHSA's substance abuse prevention and treatment programs under PHSA Titles V and XIX. It further requires a state receiving such a grant to include specified information about the use of the grant in the report already required in connection with the block grants.

The amounts in the account are not available until appropriated in subsequent appropriations acts. Notably, the amounts subsequently appropriated (i.e., the budget authority and the resulting outlays) for FY2017 and FY2018, up to the amounts transferred, are to be subtracted from any cost estimates provided for purposes of budget controls. Effectively, the appropriations from the

account will not be counted against any spending limits, such as the statutory discretionary spending limits; that is, the amounts appropriated from the account will be considered outside those limits for FY2017 and FY2018.

Title II- Discovery

Subtitle A- National Institutes of Health Reauthorization

Section 2001. National Institutes of Health Reauthorization

NIH derives its statutory authority from the Public Health Service Act of 1944 (PHSA), as amended.[15] PHSA Section 301 grants the HHS Secretary broad permanent authority to conduct and sponsor research.[16] In addition, PHSA Title IV, "National Research Institutes," authorizes in greater detail various activities, functions, and responsibilities of the NIH Director and the institutes and centers.[17] The last major NIH reauthorization was the NIH Reform Act of 2006 (P.L. 109-482). The NIH Reform Act, in PHSA Section 402A, 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, have provided authority for NIH programs to continue from FY2009 to the present.

Provision

Section 2001 amends PHSA Section 402A, to authorize appropriations for NIH in FY2018 ($34,851,000,000), FY2019 ($35,585,871,000), and FY2020 ($36,472,442,775).

Section 2002. Eureka Prize Competitions

Section 105 of the America COMPETES Reauthorization Act of 2010 (P.L. 111-358) provides federal agencies with broad authority to carry out programs designed to stimulate innovation through prize competitions.[18] 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) publishes annual reports on the implementation of Section 105 as required by P.L. 111-358.[19] 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 is available on the Challenge.gov website.[20] A search of the website on December 8, 2016, resulted in 15 competitions conducted by NIH or one of the NIH ICs. Examples of research topics covered in the various challenges include breast cancer genetics, antimicrobial resistance, and drug abuse and addiction research.

[15] 42 U.S.C. §§201-300mm-61.

[16] 42 U.S.C. §241.

[17] 42 U.S.C. §§281-290b.

[18] For more information, see CRS Report R43880, *The America COMPETES Acts: An Overview*, by Heather B. Gonzalez.

[19] Office of Science and Technology Policy, "Implementation of Federal Prize Authority: Fiscal Year 2015 Progress Report," August 2016, at https://www.whitehouse.gov/sites/default/files/fy2015_competes_prizes_report.pdf.

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

Provision

Section 2002 requires the NIH Director, under authorities in 15 U.S.C. §3719, to support prize competitions for one or both of the following goals: (1) identifying and funding areas of biomedical science that could realize significant advancements through a prize competition and (2) improving health outcomes, particularly with respect to human diseases and conditions such as those that are serious and represent a significant disease burden in the United States. With regard to the second goal, the prize competition may also target human diseases and conditions where public and private investment in research is disproportionately small relative to federal government expenditures for prevention and treatment activities and those diseases and conditions with potential for a significant return on investment. The section requires the NIH Director to collect information on the effect of prize competition innovations on advancing biomedical science or improving health outcomes and the effect of the innovations on federal expenditures. This information must be included in the NIH triennial report, required in PHSA Section 403.

Subtitle B- Advancing Precision Medicine

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. On February 25, 2016, the White House hosted a Precision Medicine Initiative (PMI) Summit to mark the one-year anniversary of the initiative's launch, first announced in the 2015 State of the Union address. In the first year, the PMI's three key entities—National Institutes of Health (NIH), Food and Drug Administration (FDA), and the Office of the National Coordinator for Health Information Technology (ONC)—began work in this area. The FY2017 President's budget requests a total of $309 million for the PMI: $4 million to FDA, $5 million to ONC, and the remaining $300 million to NIH.

Precision medicine research efforts rely on the collection of large amounts of health and other data; therefore, access to this data may be a concern in the context of this type of research. The sharing of genetic and genomic data among private individuals, researchers, and the federal government has, at times, prompted concerns that the information, if collected or retained by a federal executive branch agency, could be subject to public release pursuant to the Freedom of Information Act (FOIA). FOIA, however, specifies nine categories of information that may be exempted from the rule of disclosure, allowing agencies to withhold applicable records. Exemption 3 allows agencies to withhold applicable records if the data are specifically exempted from disclosure by a statute other than FOIA, if that statute meets criteria laid out in FOIA. These types of Exemption 3 statutes are often referred to as b(3) exemptions because they are authorized in 5 U.S.C. §552(b)(3).

As a mechanism for addressing compelled disclosure of research data, NIH currently issues Certificates of Confidentiality pursuant to PHSA Section 301(d) (42 U.S.C. §241(d)) at the request of an investigator. A Certificate of Confidentiality protects investigators from being compelled to disclose information that would identify research subjects in any civil, criminal, administrative, legislative, or other proceeding. In this way, having a Certificate of Confidentiality can help promote participation in research by adding an additional layer of privacy protection.

At the other end of the spectrum, the sharing of research data—specifically, genomic data generated by NIH-funded research—has also received attention in the context of precision medicine. NIH has established a comprehensive policy for the sharing of genomic data that "applies to all NIH-funded research that generates large-scale human or non-human genomic data

as well as the use of these data for subsequent research." This policy requires investigators to outline their data-sharing plans as part of their funding applications; if investigators fail to submit the required data, NIH may withhold funding.

Sections 2011-2014. Precision Medicine Establishment and Data Protections

Provisions

Title II, Subtitle B, has four sections (Sections 2011-2014) that together aim to support precision medicine by (1) codifying the PMI; (2) requiring issuance of Certificates of Confidentiality to investigators of federally funded research; (3) protecting identifiable, sensitive information from release under FOIA; and (4) requiring the sharing of NIH-supported research data in certain circumstances.

Section 2011 codifies the President's Precision Medicine Initiative (PMI) in a new PHSA Section 498E, by encouraging the HHS Secretary to establish and carry out the PMI, and by allowing specified components and authorities in the carrying out of the PMI as well as identifying requirements of the initiative, including, for example, complying with existing law and regulation regarding human research subjects protections. It also requires, not later than one year after enactment, the HHS Secretary to submit a report to Congress on relevant data access policies and procedures, and consultation with experts in the development of those policies.

Section 2012 amends PHSA Section 301(d) to require the HHS Secretary to issue a Certificate of Confidentiality to research investigators of research funded wholly or in part by the federal government in which sensitive, identifiable information is collected to protect the privacy of research participants. The section prohibits the individual with the certificate from disclosing sensitive information about the research participants, with certain exceptions, as specified, and would make this type of information immune from the legal process. In addition, the section requires that these protections exist in perpetuity and that the HHS Secretary must minimize the burden to researchers of compliance with this section, and must coordinate across involved HHS entities. The requirements of this section become effective 180 days after the date of enactment of the act.

Section 2013 amends PHSA Section 301 to allow the HHS Secretary to exempt from disclosure under FOIA exemption (b)(3) specified biomedical information that identifies an individual or that has an associated risk that the information may be reidentified. The HHS Secretary is required to make each such exemption available in writing and to the public, upon request. However, this does not limit individual research participants access to their own data.

Section 2014 amends PHSA Section 402(b) to allow the HHS Secretary to require recipients of NIH grants or agreements to share data generated from such NIH grants or agreements in a manner consistent with all applicable federal law regarding human subject protections, propriety interests, confidential commercial information, and intellectual property.

Subtitle C- Supporting Young Emerging Scientists

Section 2021. Investing in the Next Generation of Researchers.

Congress has had a long-standing interest in developing the future biomedical research workforce. Recent concerns have focused on ways to reduce the time between when young investigators complete their training and when they receive their first independent NIH research grant (i.e., achieve research independence). NIH has created a number of initiatives to shorten this

time, in part to better retain young investigators in biomedical research. The Departments of Labor, Health and Human Services, and Education, and Related Agencies Appropriations Act, 2016 (P.L. 114-113, Division H), instructed the NIH Director to enter into a contract with the National Academy of Sciences (NAS) to conduct a comprehensive study of the policies affecting the next generation of researchers in the United States.

Provision

Section 2021 amends Part A of Title IV of the PHSA by adding a new Section 404M, which establishes the Next Generation of Researchers Initiative (the Initiative) within the office of the NIH Director. The Initiative requires the NIH Director to coordinate all NIH policies and programs focused on promoting and providing opportunities for new researchers and for promoting earlier research independence. Among other things, the NIH Director would have to coordinate with relevant agencies, professional associations, and academic institutions to improve and update information on the biomedical workforce to inform training, recruitment, and retention programs of biomedical researchers. In establishing the Initiative, the NIH Director is required to consider recommendations made by NAS in its study on the next generation of researchers. Not later than two years after completion of the NAS study, the NIH Director would be required to submit a report to specified congressional committees regarding any actions taken by NIH with respect to the NAS recommendations.

Section 2022. Improvement of Loan Repayment Program.

NIH funds seven loan repayment programs for researchers. Three of these are intramural programs that provide educational loan repayment benefits 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). Four of these programs help extramural researchers repay their educational loans. 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 benefits under each of these programs. Under current law, appropriations for loan repayments remain available until the end of the second fiscal year after they are appropriated.

Provision

Section 2022 renames PHSA Section 487A “Intramural Loan Repayment Program” and consolidates existing NIH intramural loan repayment programs. Specifically, it (1) transfers the authority to administer these program from the HHS Secretary to the NIH Director; (2) increases annual loan repayment amounts from a maximum of $35,000 to a maximum of $50,000; and (3) provides loan repayment benefits for individuals who conduct research in areas of emerging scientific or workforce needs, in addition to individuals who conduct research on AIDS, and clinical researchers from disadvantaged backgrounds. In addition, Section 2022 authorizes the NIH Director to amend the categories eligible for intramural loan repayment as scientific and workforce priorities change. Finally, the section prohibits the NIH Director from entering into a loan repayment contract with individuals unless they have substantial amounts of educational loans relative to income as determined by the NIH Director and permits amounts appropriated for new loan repayment contracts to remain available until the end of the second fiscal year after they are appropriated.

Section 2022 similarly amends the NIH's extramural loan repayment program. Specifically, it (1) retitles PHSA 487B "Extramural Loan Repayment Program," (2) transfers authority for the program from the HHS Secretary to the NIH Director, (3) increases loan repayment amounts to a maximum of $50,000 per year, (4) prohibits the NIH Director from entering into a loan repayment contract with individuals unless they have substantial amounts of educational loans relative to income as determined by the NIH Director, and (5) permits amounts appropriated for new loan repayment contracts to remain available until the end of the second fiscal year after they are appropriated. In addition, Section 2022 retains authorization for current topics eligible for extramural loan repayment (contraception and infertility, pediatric research, minority health disparities, clinical research, and clinical research conducted by individuals from disadvantaged backgrounds). The section makes extramural researchers who are conducting research in an area of emerging scientific or workforce need eligible for loan repayment benefits, and it authorizes the NIH Director to amend the categories eligible for extramural loan repayment as scientific and workforce priorities change.

Finally, Section 2022 repeals existing authorizations for NIH loan repayment programs in PHSA Sections 464z, 487C, 487E, and 487F. It requires a GAO report, not later than 18 months after enactment, that (1) reports on NIH efforts to attract, retain, and develop emerging scientists, including underrepresented individuals in the sciences; (2) reports on the research areas where individuals are receiving increased loan repayment amounts; and (3) analyzes the impact of changes included in this act on addressing workforce shortages.

Subtitle D- National Institutes of Health Planning and Administration

Section 2031. National Institutes of Health Strategic Plan

PHSA Section 402(b)(5) 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." Current law does not direct NIH Institutes and Centers (ICs) to coordinate or collaborate in the development of IC strategic plans. NIH provides access to many of its strategic plans on the agency's website.[21] The NIH Reform Act of 2006 (P.L. 109-482) enhanced the authority of the NIH Director's Office to perform strategic planning and provided for trans-NIH initiatives by enacting the Common Fund into law and requiring strategic planning for the fund. The Common Fund is part of the Office of the Director and is intended to support research in emerging areas of scientific opportunity, public health challenges, and knowledge gaps that might benefit from collaboration between two or more ICs.

Provision

Section 2031 amends PHSA Section 402 by adding a new subsection (m), which describes a strategic plan for NIH. Within two years of enactment, and once every six years thereafter, the NIH Director, in consultation with the IC Directors, must develop and submit to the appropriate committees of Congress, and post on the NIH website, a six-year NIH Strategic Plan. The NIH Strategic Plan is expected to provide direction to the biomedical research investments made by NIH, facilitate IC collaboration, leverage scientific opportunity, and advance biomedicine.

The NIH Strategic Plan must identify research priorities, such as advancement of treatment, cure and prevention of health conditions, emerging scientific opportunities, and rising public health

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

challenges. The research strategy must address the disease burden in the United States, including rare diseases, and the many factors that contribute to health disparities. Other elements to be included in the NIH Strategic Plan are (1) coordination of research among the ICs; (2) priorities for funding research through the Common Fund; (3) training the biomedical workforce; and (4) collaboration with other agencies and departments. The individual IC strategic plans are required to be prepared regularly, to be informed by the NIH Strategic Plan, and to have a common template. The NIH Director must consult with the IC directors, researchers, patient advocacy groups, and industry leaders when developing the strategic plan.

Section 2032. Triennial Reports

PHSA Title IV establishes numerous reporting requirements for the NIH Director related to the activities of the agency. Specifically, PHSA Section 403(a) requires the NIH Director to submit to Congress biennially a report on NIH activities. Among other things, the report must include an assessment of the state of biomedical and behavioral research, and details of all the research activities conducted or supported by the ICs of NIH.

Provision

Section 2032 amends PHSA Section 403(a) by replacing the biennial reporting requirement of the NIH Director with a triennial requirement. The section adds new, and clarifies existing, reporting requirements, including a description of intra-NIH activities and funding made available for conducting and supporting research that involves collaboration between an IC and one or more other ICs.

Section 2033. Increasing Accountability at the National Institutes of Health

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

Research supported by NIH is first evaluated by a peer review system.[22] Scientists who seek to compete for NIH research funding must submit detailed applications describing the research they plan to undertake. NIH considers the applications under a two-tiered system of peer review. First, the applications are reviewed for scientific and technical merit by committees composed of nongovernment scientists who are experts in the relevant fields of research. Each application is thoroughly discussed and given a score representing the average of the scores assigned by the reviewers. That score becomes the main determinant in whether an applicant will receive funding from an IC for the research proposal. The funding decisions are fine-tuned by a second level of peer review in the ICs, when the applications are considered for program relevance by the IC's National Advisory Councils or Boards, which are composed of scientific and lay representatives.

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."[23]

[22] Peer review requirements described in PHSA Section 492.

[23] P.L. 102-394 and 5 U.S.C. §5376.

Provision

Section 2033 amends PHSA Section 405 with regard to the appointment and terms of the Director of the National Cancer Institute and the directors of other NIH ICs. It requires that directors of ICs be appointed by the HHS Secretary acting through the NIH Director. The Director of the National Cancer Institute continues to be appointed by the President. It specifies five-year terms for the IC Directors who are appointed by the HHS Secretary acting through the NIH Director, and authorizes the NIH Director to remove an IC Director prior to the end of a five-year term if necessary. It permits 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 requires 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 is deemed to be appointed for a five-year term as of the date of enactment.

Section 2033 specifies that the compensation limitations in Section 202 of the Labor/HHS/ED Appropriations Act, 1993, related to time-limited appointments of consultants and individual scientists, do not apply to directors appointed under this new authority.[24]

The section adds a new requirement that before a new research grant is made by an IC, 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, "programs or projects funded by other agencies on similar research topics and advice by staff and the advisory council or board of such national research institute or national center."

Section 2033 also requires the HHS Secretary to submit a report to Congress, not later than two years following enactment, "on efforts to prevent and eliminate duplicative biomedical research that is not necessary for scientific purposes." Among other things, the report must "describe how the HHS Secretary operationally distinguishes necessary and appropriate scientific replication from unnecessary duplication, and provide examples of instances where the HHS Secretary has identified unnecessarily duplicative research and the steps taken to eliminate the unnecessary duplication."

Section 2034. Reducing Administrative Burden for Researchers

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."[25] 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 43% 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)

[24] Ibid.

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

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."[26]

Provision

Section 2034 includes a series of requirements that aim to address the administrative burden on researchers funded by NIH and other federal agencies. First, it directs the HHS Secretary, within two years of enactment, to lead a review by research funding agencies of all financial conflict-of-interest regulations and policies and to make revisions to harmonize the policies and reduce the administrative burden on researchers, as appropriate. It also requires the HHS Secretary to update this policy and, in doing so, take into account certain specified considerations regarding financial interest disclosures. Second, it requires the NIH Director to implement measures that aim to reduce the administrative burdens experienced by primary NIH grant awardees related to monitoring grant sub-recipients. Third, the HHS Secretary, in consultation with the NIH Director, is required to evaluate financial expenditure reporting procedures and requirements for NIH funding recipients and take appropriate action to avoid duplication of effort and minimize burden to funding recipients.

Fourth, within two years of enactment, the HHS Secretary, in consultation with the NIH Director, the Secretary of Agriculture, and the FDA Commissioner, must complete a review of regulations and policies for the care and use of laboratory animals and make appropriate revisions to reduce administrative burden on investigators. Fifth, the HHS Secretary is required to clarify the applicability of OMB Uniform Guidance requirements regarding documentation of personnel expenses for entities receiving HHS grants.

Finally, within one year of enactment, the OMB Director is required to establish a Research Policy Board, consisting of up to 10 federal and 9 to 12 nonfederal members, as specified, to provide the NIH Director and other members of the federal government with information on the effects of regulations related to federal research requirements. The board makes recommendations on harmonizing regulations and policies to minimize administrative burden across federal research agencies. Within two years of enactment, and once thereafter, the board must submit a report to specified offices in OMB, the heads of relevant federal departments and agencies, and specified House and Senate committees. The report must provide recommendations on scientific research policy, including regulatory benefits and burdens. The board will sunset on September 30, 2021. The section also requires that GAO, within four years of enactment, conduct an evaluation of board activities regarding its purpose and responsibilities and submit a report to Congress.

Section 2035. Exemption of the National Institutes of Health from the Paperwork Reduction Act Requirements.

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

[26] 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."[27] The PRA requires that federal agencies receive clearance from OIRA before requesting most types of information from the public.[28] PRA clearance is required when standardized information is collected from 10 or more respondents within a 12-month period.[29] 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.[30]

Provision

Section 2035 amends PHSA Section 301 by adding a subsection stating that the PRA does not apply to the collection of information during the conduct of NIH research.

Section 2036. High-Risk, High-Reward Research

Other transaction (OT) authority is a special vehicle used by certain federal agencies for obtaining or advancing research and development (R&D).[31] 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. 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 and certain procurement statutes that govern contracts.

Provision

Section 2036 adds a new PHSA Section 402(n) to allow the NIH Director to approve requests by IC Directors, or program officers within the Office of the Director, to engage in transactions other than a contract, grant, or agreement with respect to projects that carry out (1) the Precision Medicine Initiative, or (2) "research that represents important areas of emerging scientific opportunities, rising public health challenges, or knowledge gaps that deserve special emphasis and would benefit from conducting or supporting additional research that involves collaboration between 2 or more [ICs], or would otherwise benefit from strategic coordination and planning."[32] This provision also requires internal NIH reporting on the use of this authority and requires the HHS Secretary, through the NIH Director, to submit a report to Congress evaluating the activities under this new subsection by September 30, 2020.

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[27] 44 U.S.C. §3501.

[28] For further information about the PRA, see CRS Report RL30590, *Paperwork Reduction Act Reauthorization and Government Information Management Issues* (out of print; available to congressional clients from the author on request), and CRS Report RL32397, *Federal Rulemaking: The Role of the Office of Information and Regulatory Affairs*, coordinated by Maeve P. Carey.

[29] 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.

[30] 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.

[31] For further information, see U.S. Government Accountability Office, *DOD Research: Acquiring Research by Nontraditional Means*, GAO/NSIAD-96-11, March 29, 1996, https://www.gpo.gov/fdsys/pkg/GAOREPORTS-NSIAD-96-11/pdf/GAOREPORTS-NSIAD-96-11.pdf.

[32] PHSA Section 402(b)(7)(A)(i).

Section 2037. National Center for Advancing Translational Sciences.

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 three phases.

> **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. Sometimes Phase II clinical trials are divided into **Phase IIA** (to assess dosing requirements) and **Phase IIB** (to study efficacy). 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.[33]

Under current law in PHSA Section 479, NIH's National Center for Advancing Translational Sciences (NCATS) may develop and provide infrastructure and resources for all phases of clinical trials research; however, it may support clinical trial activities only through the end of Phase IIA, with specific exceptions. NCATS may support clinical trial activities through the end of Phase IIB for treatment of a rare disease or condition if (1) it gives public notice for a period of at least 120 days of NCATS's 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. This section does not authorize the HHS Secretary to disclose trade secret information or other privileged or confidential information.

Provision

Section 2037 amends PHSA Section 479 to extend NCATS's authority to support clinical trial activities through the end of Phase IIB (instead of Phase IIA) and extends the exception for treatment of a rare disease or condition through the end of Phase III (instead of Phase IIB).

It adds material to the NCATS annual/biennial report regarding methods and tools developed since the previous report and whether such methods and tools are being used by the FDA to support medical product reviews. Under the Cures Act, the next NCATS report, following enactment, will include a complete list of all such methods and tools developed by research supported by NCATS.

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

Section 2038. Collaboration and Coordination to Enhance Research

Racial and ethnic minorities traditionally 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."[34] Biological differences (e.g., genetic differences) may affect how people process or respond to medical products. This variation could make a treatment less effective or perhaps more toxic for individuals with specific genotypes. Therefore, it is important to study in clinical trials the safety and effectiveness of medical products in a broadly representative sample of people who will likely use the products following FDA approval.

PHSA Section 492B requires the NIH Director to include women and minorities in NIH-funded clinical research and to conduct or support outreach to recruit minorities and women into clinical research. Section 492B(d) requires the NIH Director, in consultation with the directors of the NIH's Office of Research on Women's Health and the Office of Research on Minority Health, to develop guidelines regarding the requirements under Section 492B.[35]

Provision

Section 2038 amends PHSA Section 402(b), requiring the NIH Director, in assessing research priorities, to assemble accurate data on study populations in clinical research that specifies the inclusion of women, members of minority groups, relevant age categories (including pediatric subgroups), and other demographic variables. The data must be disaggregated by research area, condition, and disease categories and made publically available on the NIH website. The NIH Director is required to foster collaboration between the ICs that conduct research on human subjects, allow for an increase in the number of subjects studied, and utilize a diverse study population with special consideration of the determinants that contribute to health disparities.

Section 2038 amends PHSA Section 492B to make the biennial report a triennial report and requires that the report contain specified data on the number of women and members of minority groups included in clinical research projects conducted during the reporting period.

Section 2038 amends PHSA Section 486 to specify that the coordinating committee for the Office of Research on Women's Health will include NIH IC Directors or their senior staff-level designees.

Section 2038 adds a new PHSA Section 404N, Population Focused Research, which requires the NIH Director to encourage efforts to improve research related to the health of sexual and gender minority populations through the increased participation of such groups in clinical research. The HHS Secretary, in collaboration with the NIH Director and taking into account the recommendations of the National Academy of Medicine, is required to continue to support research for the development of appropriate measures related to reporting health information of sexual and gender minority populations. Within two years of enactment, the HHS Secretary is required to disseminate and make public such measures.

Section 2038 also amends PHSA Section 464z-3, adding that the National Institute on Minority Health and Health Disparities Director may foster partnerships between the ICs and may

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

[35] See "NIH Policy and Guidelines on The Inclusion of Women and Minorities as Subjects in Clinical Research," at http://grants.nih.gov/grants/funding/women_min/guidelines_amended_10_2001.htm.

encourage the funding of collaborative research to achieve the goals of NIH related to minority health and health disparities.

Section 2038 requires the NIH Director, within two years of enactment and taking into consideration the findings of the working group established under Section 2039, to develop policies for basic research to assess relevant biological variables, including sex, and how differences between male and female cells, tissues, or animals may be studied and permits the NIH Director to amend these policies as appropriate. It also requires the NIH Director to (1) consult with the Office of Research on Women's Health, the Office of Laboratory Animal Welfare, and appropriate members of the scientific and academic communities; and (2) conduct outreach in developing (and updating) policies on the influence of sex as a variable in basic research, among other requirements. With respect to clinical research involving women and minorities, the NIH Director must, within one year of enactment, update the guidelines established under PHSA Section 492B(d) to reflect the science regarding sex differences and improve adherence to the requirements of Section 492B of the PHSA, among other things.

Section 2038 requires the NIH Director, within six months of enactment, to convene a workshop of experts on pediatrics and older populations to provide input on appropriate age groups to be included in research studies. Within six months of the workshop, the NIH Director must determine if it is necessary to update NIH policies "on the inclusion of relevant age groups in clinical studies." The Director is required to make available to the public the findings and conclusions of the workshop and the updates to policies. The Director must ensure that age-related data reported in the triennial report are made publicly available on the NIH website.

Section 2039. Enhancing the Rigor and Reproducibility of Scientific Research

Research supported by NIH is evaluated by a peer review system.[36] Scientists competing for NIH funding submit detailed applications describing their research plan. NIH considers the applications under a two-tiered system of peer review. First, the applications are reviewed for scientific and technical merit by committees composed of nongovernment scientists who are experts in the relevant fields of research. Each application is thoroughly discussed and given a score, which becomes the main determinant in whether an applicant will receive IC funding. A second level of review occurs in the ICs when the applications are considered for program relevance by the IC's National Advisory Councils or Boards, composed of scientific and lay representatives. The peer review system does not necessarily evaluate the applications for reproducibility.

Provision

Section 2039 requires the HHS Secretary, acting through the NIH Director, to convene a working group to make recommendations for a formal policy to enhance the rigor and reproducibility of NIH-funded scientific research. The working group must consider various specified factors, including, for example, preclinical and clinical experiment design and methods of statistical analysis. It also requires the NIH Director, not later than 18 months after enactment, to consider the recommendations and develop or update policies as appropriate. Finally, the NIH Director must issue a report to the HHS Secretary and Congress, within two years of enactment, regarding the recommendations and any subsequent policy changes. This section does not authorize the HHS Secretary to disclose trade secret information or other privileged or confidential information.

[36] Peer review requirements described in PHSA Section 492.

Section 2040. Improving Medical Rehabilitation Research at the National Institutes of Health

PHSA Section 452 established in 1990 the National Center for Medical Rehabilitation Research (the Center) within the Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD) at NIH to conduct and support research, and disseminate information, on the rehabilitation of individuals with physical disabilities. It also required the NIH Director to create a Medical Rehabilitation Coordinating Committee and a National Advisory Board on Medical Rehabilitation Research.

The section also requires NICHD Director—in collaboration with the Director of the Center, the Coordinating Committee, and the Advisory Board—as created by this section—to develop, and periodically revise and update, a comprehensive plan for medical rehabilitation research.

Provision

Section 2040 amends PHSA Section 452 instructing the Director of the Center—in collaboration with the Director of the Institute, the coordinating committee, and the advisory board—to develop and, not less than every five years, revise and update a comprehensive plan for medical rehabilitation research. The research plan must include goals and objectives for such research. Prior to revising and updating the research plan, the Director of the Center must report to the coordinating committee and the advisory board on the progress made toward achieving the research goals and objectives, and provide recommendations for revising and updating the plan. Within 30 days of revising and updating the plan, the Director of the Center is required to transmit the plan to the President, and to specified congressional committees.

In addition, Section 2040 requires the HHS Secretary, along with the other federal agencies, to review their medical rehabilitation research programs and take action to avoid duplication among those programs through actions such as entering into interagency agreements. Finally, Section 2040 defines medical rehabilitation research as "the science of mechanisms and interventions that prevent, improve, restore, or replace lost, underdeveloped, or deteriorating function."

Section 2041.Task Force on Research Specific to Pregnant Women and Lactating Women

Provision

Within 90 days of enactment, Section 2041 requires the HHS Secretary to establish a Task Force on Research Specific to Pregnant and Lactating Women. The section specifies the duties, membership, meeting schedule, and reporting requirements of the task force, which would be terminated two years after its establishment, with an option for a two-year extension. It requires the HHS Secretary, not later than two years after enactment, to update regulations and guidance, as appropriate, regarding the inclusion of pregnant women and lactating women in research. This section does not authorize the HHS Secretary to disclose trade secret information or other privileged or confidential information.

Section 2042. Streamlining National Institutes of Health Reporting Requirements

PHSA Title IV establishes numerous reporting requirements for the NIH Director related to the activities of the agency. Specifically, PHSA Section 403(a) requires the NIH Director to submit to

Congress biennially a report on NIH activities. Among other things, the report must include an assessment of the state of biomedical and behavioral research, and details of all the research activities conducted or supported by the ICs of NIH.

Provision

Section 2042 modifies or eliminates a number of different NIH reporting requirements. Within two years of enactment, the heads of each IC must submit to the NIH Director a report on the amount of funding made available for conducting or supporting research that involves collaboration between a given IC and at least one other IC. This information will be included in the triennial report required by Section 403(a), as amended by Section 2032.

It also (1) eliminates an annual reporting requirement regarding the number of experts and consultants whose services are used by NIH; (2) makes a minor modification to the doctoral degree reporting requirement; (3) makes a technical correction to a vaccine reporting requirement; (4) changes the NCATS annual report to a biennial report; (5) eliminates the report on Centers of Excellence; (6) eliminates the periodic reports on rapid HIV testing; and (7) eliminates the National Institute on Nursing Research biennial report.

Section 2043. Reimbursement for Research Substances and Living Organisms

PHSA Section 301(a) establishes the general research authorities of the Public Health Service through the HHS Secretary. Specifically, it requires the HHS Secretary to "conduct in the Service, and encourage, cooperate with, and render assistance to other appropriate public authorities, scientific institutions, and scientists in the conduct of, and promote the coordination of, research, investigations, experiments, demonstrations, and studies relating to the causes, diagnosis, treatment, control, and prevention of physical and mental diseases and impairments of man." As part of these authorities, the HHS Secretary is authorized to make available substances and living organisms for biomedical and behavioral research.

Provision

Section 2043 amends PHSA Section 301(a) allowing the HHS Secretary, where research substances and living organisms are made available to researchers through contractors, to direct the contractors to collect payments for the costs incurred while making these substances and organisms available. These amounts would be credited to the appropriations accounts that incurred such costs and would be available until expended.

Section 2044. Sense of the Congress on Increased Inclusion of Underrepresented Populations in Clinical Trials

PHSA Section 492B requires that the NIH Director ensure that clinical research conducted or supported by NIH include members of minority groups as subjects. Each IC advisory council must prepare biennial reports describing the manner in which the IC has complied with this requirement. The report is submitted to the IC Director and is included in the biennial report under PHSA Section 403.

Provision

Section 2044 states that it is the sense of Congress that the National Institute on Minority Health and Health Disparities should include within its strategic plan ways to increase representation of underrepresented populations in clinical trials.

Subtitle E- Advancement of the National Institutes of Health Research and Data Access

Sections 2051. Technical Updates to Clinical Trials Database

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 PHSA Section 402 subsections(i)-(j). Subparagraph 402(j)(2)(B) requires the NIH Director to ensure that the public may, in addition to keyword 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.

Under PHSA Section 402(j), 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 section also required the HHS Secretary, by rulemaking, to expand the requirements for submission of summary results information, and authorized the HHS Secretary 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. The comment period was extended until March 23, 2015; about 900 comments were received. The final rule was published on September 21, 2016, and is expected take effect on January 18, 2017.[37]

Provision

Section 2051 amends PHSA Section 402(j)(2)(D), regarding posting of data, by adding new language requiring the NIH Director to inform responsible parties of the option to request that information for a medical device clinical trial be publically posted prior to the date of clearance or approval. Section 2051 adds language that defines "combination product" for purposes of this database.

Section 2052. Compliance Activities Reports

PHSA Section 402(i)-(j) delineates the requirements for the clinical trials database but currently does not require the submission of a report to Congress.

Provision

Section 2052 requires the HHS Secretary, acting through the NIH Director and in collaboration with the FDA Commissioner, to submit to Congress, not later than two years after enactment, a report that "describes education and outreach, guidance, enforcement, and other activities undertaken to encourage compliance with Section 402(j) of the PHSA" (i.e., with submission to the clinical trials database).

[37] NIH provides information on selected events, policies, and laws related to the development and expansion of Clinical Trials.gov at https://clinicaltrials.gov/ct2/about-site/history.

This section also requires the HHS Secretary, acting through the NIH Director and in collaboration with the FDA Commissioner, to submit to Congress a report on registered clinical trials, as specified, including activities undertaken by the HHS Secretary to educate responsible persons about compliance with the requirements in Section 402(j). The HHS Secretary must submit an initial report not later than two years after the compliance date of the final rule implementing Section 402(j) of the PHSA. Two follow-up reports are required, which include information on actions taken to enforce compliance with the ClinicalTrials.gov reporting requirements.

Section 2053. Updates to Policies to Improve Data

PHSA Section 492B requires the NIH Director to include women and minorities in NIH-funded clinical research and to conduct or support outreach to recruit minorities and women into clinical research. Section 492B(c) requires the NIH Director to "ensure that the trial is designed and carried out in a manner sufficient to provide for a valid analysis of whether the variables being studied in the trial affect women or members of minority groups, as the case may be, differently than other subjects in the trial."

Provision

Section 2053 amends PHSA Section 492B(c), adding that the NIH Director must consider whether grant award recipients conducting research related to the inclusion of women and minority populations in clinical research have complied with the reporting requirements of ClinicalTrials.gov. The NIH Director must also take such compliance into consideration when awarding any future grants to such an entity. The Director of NIH must encourage the reporting of results to ClinicalTrial.gov "through any additional means determined appropriate by the Director."

Section 2054. Consultation

PHSA Section 402(i)-(j) requires that the HHS Secretary consult with FDA, NIH, and the Centers for Disease Control and Prevention (CDC) prior to establishing the "data bank of information on clinical trials for drugs for serious or life-threatening diseases and conditions." In addition, it requires that the HHS Secretary consult with experts in risk communication to ensure that posted information regarding the database is not misleading to patients or the lay public. The HHS Secretary must also consult with other federal agencies to ensure that clinical trial information is submitted to the database.

Provision

Section 2054 requires, within 90 days of enactment, the HHS Secretary to consult with relevant federal agencies, including FDA, the Office of the National Coordinator for Health Information Technology, and NIH, as well as other stakeholders (including patients, researchers, physicians, industry representatives, and developers of health information technology), to receive recommendations to improve ClinicalTrials.gov, including improvements in usability, functionality, and search capability.

Subtitle F- Facilitating Collaborative Research

Section 2061. National Neurological Conditions Surveillance System

The PHSA does not explicitly authorize or require surveillance of neurological diseases in general, although the HHS 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 and autism spectrum disorder).[38]

Provision

Section 2061 adds a new PHSA Section 399S-1, which requires the HHS Secretary, through the CDC Director and in consultation with specified parties, to establish a National Neurological Conditions Surveillance System by enhancing and expanding relevant surveillance infrastructure and activities. The system may include a registry. In establishing the system, the HHS Secretary is required to collect and manage information in order to facilitate research and, as is practicable, to include information on incidence and prevalence, demographics, risk factors, and diagnostic and progression markers.[39] Additional data elements may include the natural history, prevention, detection, management, and treatment approaches for the diseases, and the development of outcome measures. The HHS Secretary initially may address a limited number of neurological diseases.

The section also authorizes the HHS Secretary to award grants, contracts, or cooperative agreements with public or private nonprofit entities to implement this provision. The HHS Secretary must make information and analysis obtained from the system available to other federal health agencies and state and local agencies, and, as appropriate and subject to federal privacy laws, to researchers and the public. Within one year of the establishment of a system under this section and biennially thereafter, the HHS Secretary must provide to Congress and the public an interim report on such system. A report on implementation of this section is due to Congress four years after enactment. The section authorizes to be appropriated $5 million for each of fiscal years 2018 through 2022 to carry out activities under this section.

Section 2062. Tick-Borne Diseases

The HHS Secretary is given broad authority to conduct research related to disease under Title III of the PHSA. Specifically, the HHS Secretary is required to conduct research, investigations, experiments, demonstrations, and studies relating to the causes, diagnosis, treatment, control, and prevention of disease.[40] The act does not explicitly address tick-borne diseases, but HHS agencies do carry out research and public health activities on tick-borne diseases under the Secretary's general authority.

[38] PHSA Section 399S; 42 U.S.C. §280g-7 and PHSA Section 399AA; 42 U.S.C. §280i.

[39] 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.

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

Provision

Section 2062 requires the HHS Secretary to continue to conduct or support epidemiological, basic, translational, and clinical research related to vector-borne diseases, including tick-borne diseases. It also requires the HHS Secretary to ensure that the triennial report of the NIH Director to Congress[41] includes information on NIH activities with respect to tick-borne diseases.

The section also requires the HHS Secretary to establish a working group to review the status of research on tick-borne diseases and relevant federal activities, and to report on such activities and any recommended changes every two years. The section provides requirements related to the working group's membership, responsibilities, meeting frequency, and reporting. The working group is subject to the Federal Advisory Committee Act (FACA), and will terminate six years after enactment.

Section 2063. Accessing, Sharing, and Using Health Data for Research Purposes

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.[42] In general, covered entities may use or disclose PHI for the purposes of treatment, payment, and other routine health care operations with few restrictions.[43]

The disclosure of PHI to researchers generally requires an individual's authorization unless an Institutional Review Board (or equivalent Privacy Board) waives the authorization.[44] 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.[45]

Provision

Section 2063 instructs the HHS Secretary, within one year of enactment, to issue guidance clarifying some of the privacy rule's restrictions on researchers' access to PHI. First, the HHS Secretary is required to clarify that the rule's provision prohibiting researchers from removing PHI during preparation of a research protocol permits 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. Second, the Secretary is required to clarify the circumstances under which a HIPAA authorization to use or disclose PHI for future research contains sufficient information; for example, 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

[41] This report is required under PHSA Section 403, 42 U.S.C. §283, as amended by this act.

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

[43] 45 C.F.R. §164.506.

[44] 45 C.F.R. §164.512(i)(1)(i).

[45] 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.

research; (2) states that the authorization will either expire at a specified time or will remain valid unless revoked by the individual; and (3) provides revocation instructions to the individual.

Finally, Section 2063 requires the HHS Secretary, within one year of enactment, to convene a working group to study the uses and disclosures of PHI for research purposes. The working group must include various specified federal and nonfederal members, and must report to the HHS Secretary within one year of its establishment with recommendations on whether the uses and disclosures for research purposes should be modified, as specified. The HHS Secretary must submit the report to Congress and make it publicly available, at which time the working group shall terminate.

Subtitle G- Promoting Pediatric Research

Section 2071. National Pediatric Research Network

In 2013, PHSA Section 409D(d) established the NIH Pediatric Research Network "in order to more effectively support pediatric research and optimize the use of Federal resources."[46]

Provision

Section 2071 amends PHSA Section 409(D)(d) to (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) retains language telling the NIH Director to collaborate with the ICs that carry out pediatric research, (3) amends language to require the NIH Director (it had previously been permitted) to award funding to support the pediatric research consortia, and (4) require that support for the pediatric research consortia not exceed five years.

Section 2072. Global Pediatric Clinical Study Network

Provision

Section 2072 expresses the sense of Congress that (1) NIH should encourage a global pediatric clinical study network through funding to support new and early stage investigators; (2) the HHS Secretary should engage with clinical investigators and international authorities, including those in the European Union, during the formation of the network to encourage their participation; and (3) the HHS Secretary should continue to encourage and facilitate the network after it is established.

[46] 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.

Title III-Development[47]

Subtitle A-Patient Focused Drug Development

The Food and Drug Administration Safety and Innovation Act (FDASIA; P.L. 112-144) expanded FDA's authorities and strengthened the agency's ability to safeguard and advance public health.[48] FDASIA added a new FFDCA Section 569C "Patient Participation in Medical Product Discussion," facilitating increased involvement of patients earlier in the regulatory process for medical product review. Section 569C directs the HHS Secretary to

> develop and implement strategies to solicit the views of patients during the medical product development process and consider the perspectives of patients during regulatory discussions by (1) fostering participation of a patient representative who may serve as a special government employee in appropriate agency meetings with medical product sponsors and investigators; and (2) exploring means to provide for identification of patient representatives who do not have any, or have minimal, financial interests in the medical products industry.

Sections 3001-3004. Patient Experience Data, Patient-Focused Drug Development Guidance, Streamlining Patient Input, Report on Patient Experience Drug Development

Provisions

Section 3001 amends FFDCA Section 569C by adding a new subsection (b), "Statement of Patient Experience," requiring the HHS Secretary, upon approval of a new drug application (NDA), to make public any patient experience data and related information submitted and reviewed as part of the application. "Data and information" refers to patient experience data, information on patient-focused drug development tools, and other relevant information, as determined by the HHS Secretary. "Patient experience data" is defined as

> (1) data that are collected by any persons (including patients, family members and caregivers of patients, patient advocacy organizations, disease research foundations, researchers, and drug manufacturers); and (2) are intended to provide information about patients' experiences with a disease or condition, including—(A) the impact of such disease or condition, or a related therapy, on patients' lives; and (B) patient preferences with respect to treatment of such disease or condition.

Section 3002 requires the HHS Secretary, acting through the FDA Commissioner, to develop a plan to issue draft and final guidance, over a period of five years, regarding the collection of patient experience data and the use of such data in drug development. This section specifies the contents of the guidance documents (e.g., methods that could be used to collect and submit patient experience data, and methodologies, standards, and technologies that could be used to collect and analyze clinical data for regulatory decisionmaking).

Section 3003 exempts FDA from the Paperwork Reduction Act clearance process when requesting patient experience data under sections 3001 and 3002 of the 21st Century Cures Act.

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[47] Subtitle J, "Technical Corrections," is not summarized in this report.

[48] FDA, The Food and Drug Administration Safety and Innovation Act (FDASIA) Section 1137: Patient Participation in Medical Product Discussions Report on Stakeholder Views, February 19, 2016; see http://www.fda.gov/downloads/ForPatients/About/UCM486859.pdf.

Section 3004 requires the HHS Secretary, acting through the FDA Commissioner, to publish on the FDA website, no later than June 1 of 2021, 2026, and 2031, a report "assessing the use of patient experience data in regulatory decision-making, in particular with respect to the review of patient experience data and information on patient-focused drug development tools...."

Subtitle B-Advancing New Drug Therapies

Section 3011. Qualification of Drug Development Tools

Lengthy clinical trials have been found to contribute to the high cost of drug development. In clinical settings where the disease course is long and an extended period of time would be required to measure the intended clinical benefit of a drug, surrogate endpoints—based on the measurement of biomarkers—may be used to determine the clinical benefit of a product, rather than clinical endpoints. Surrogate endpoints "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. The main disadvantage of these endpoints is that favorable effects on surrogates do not automatically translate into benefits to health."[49] A number of drugs have been approved on the basis of surrogate endpoint data and, after adoption into medical practice, have been shown to be harmful through clinical trials or other subsequent analysis.[50] The FDA uses surrogate endpoints in about half of new drug approvals.[51]

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."[52] 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.[53]

FDASIA (P.L. 112-144) amended FFDCA Section 506 (on fast track products) by adding the following: "The HHS 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 HHS 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."[54]

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[49] 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.

[50] 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.

[51] 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.

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

[53] Ibid.

[54] FFDCA §506(d)(2).

Provision

Section 3011 adds a new FFDCA Section 507, "Qualification of Drug Development Tools," which requires the HHS Secretary to establish a process for the qualification of drug development tools. A drug development tool is defined to include (1) a biomarker; (2) a clinical outcome assessment; and (3) any other method, material, or measure that the HHS Secretary determines aids drug development and regulatory review.

Under new FFDCA Section 507, the HHS Secretary is allowed to accept a qualification submission based on factors that include its scientific merit, and the HHS Secretary is 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 HHS Secretary is allowed, through grants or other specified mechanisms, to consult with biomedical research consortia and may consider their recommendations in review of the qualification submission. "Biomedical research consortia" is 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. The HHS Secretary is 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 is allowed to be used to obtain approval or licensure of a drug or biologic or to support a product's investigational use. The HHS Secretary is allowed to rescind or modify the granted qualification if he or she determines the drug development tool is not appropriate for the proposed context; if the HHS Secretary does this, the requestor would be granted a meeting, upon request, with the HHS Secretary to discuss the basis of the decision.

New FFDCA Section 507 requires the HHS Secretary to make public on the FDA website, and update at least biannually, information about the qualification submissions, the HHS Secretary's determinations in response to the submissions, and any subsequent modifications to the HHS Secretary's determinations, among others. It also specifies that nothing in this section is to be construed to allow the HHS 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 is 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.

The section also requires the HHS Secretary, not later than three years 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 is required to, for example, provide "a conceptual framework describing appropriate standards and scientific approaches to support the development of biomarkers." The HHS Secretary is required to issue final guidance not later than six months after the comment period for the draft guidance closes. To inform the guidance, the HHS Secretary is required, in consultation with the biomedical research consortia, to develop a taxonomy for the classification of biomarkers for use in drug development. The HHS Secretary is required to make this publicly available not later than two years after enactment and to finalize the taxonomy not later than one year after the public comment period closes.

The section requires the HHS Secretary, not later than two years after enactment, to convene a public meeting regarding the qualification process under new FFDCA Section 507. The HHS Secretary is also required to publish a report on FDA's website, not later than five years after enactment, to include specified information.

Section 3012. Targeted Drugs for Rare Diseases

Precision medicine is a relatively new term for what has traditionally been called personalized medicine (or targeted 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. Rare diseases often have genetic origins, and advances in medicine have resulted in the development of new treatments that work by targeting the genetic mutations that cause these diseases. It is inherently difficult to develop drugs for rare diseases because of the small patient population available to conduct clinical trials, so targeted therapies are generally first developed for patients with the most frequent disease-causing mutations. However, to provide therapies for the full spectrum of certain genetic rare diseases, additional targeted therapies would need to be developed.

Targeted therapies, 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),[55] a designation that was created by the Orphan Drug Act (P.L. 97-414) to encourage firms to develop pharmaceuticals to treat rare diseases and conditions by providing an extended period of market exclusivity. FFDCA Section 526(a) defines "rare disease or condition" as any disease or condition that affects fewer than 200,000 persons in the United States, or affects more than 200,000 persons in the United States and for which there is no reasonable expectation that the cost of developing and making the drug available in the United States will be recovered from U.S. sales.

Provision

Section 3012 adds a new FFDCA Section 529A "Targeted Drugs for Rare Diseases," with the purpose of facilitating the "development, review, and approval of genetically targeted drugs and variant protein targeted drugs to address an unmet medical need in one or more patient subgroups, including subgroups of patients with different mutations of a gene, with respect to rare diseases or conditions that are serious or life-threatening; and maximize the use of scientific tools or methods, including surrogate endpoints and other biomarkers, for such purposes."

Section 3012 allows the HHS Secretary to permit the sponsor of a new drug application for a genetically targeted drug or a variant protein-targeted drug to rely on data and information that has been previously developed and submitted, either by the same or a different sponsor (with permission), as part of an approved application that incorporates or uses the same or similar genetically targeted technology or for a variant protein-targeted drug.[56] It defines genetically targeted drugs, genetically targeted technology, and variant protein targeted drugs. New FFDCA Section 529A is not to be construed to limit the HHS Secretary's product approval authorities, or to entitle sponsors to obtain information in another sponsor's application without permission of the other sponsor.

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

[56] An example of a variant protein-targeted drug is Gleevec (imatinib), which is used to treat leukemia and other kinds of cancer. It targets at least one variant form of a tyrosine kinase enzyme (an enzyme is a protein) called BCR-Abl tyrosine kinase (chromosol translocation); see http://www.ncbi.nlm.nih.gov/pmc/articles/PMC1907317/.

Section 3013. Reauthorization of Program to Encourage Treatments for Rare Pediatric Diseases

FDASIA (P.L. 112-144) added a new FFDCA Section 529, creating the pediatric priority review voucher program. This voucher program, funded by user fees, provides 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. The term "rare pediatric disease" refers to a disease that affects (1) individuals aged from birth to 18 years, and (2) fewer than 200,000 persons in the United States, or affects more than 200,000 persons in the United States and for which there is no reasonable expectation that the cost of developing and making the drug available in the United States will be recovered from U.S. sales. For example, in 2014, BioMarin was awarded a rare pediatric disease priority review voucher for the drug Vimizim (elosulfase alfa) —a treatment for a rare congenital enzyme disorder.[57] BioMarin sold the voucher to Sanofi and Regeneron for $67.5 million, and it was then used to speed the approval of Praluent (alirocumab) injection, a cholesterol-lowering treatment.[58]

FDASIA terminated the authority to award such vouchers one year after the HHS 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. This authority was extended until September 30, 2016, by the Consolidated Appropriations Act of 2016 (P.L. 114-113).

The Advancing Hope Act of 2016 (P.L. 114-229), reported as part of the package of Senate medical innovation bills, was signed into law on September 30, 2016.[59] The law temporarily extended the program's authority through December 31, 2016. It also amended the definition of "rare pediatric disease" in FFDCA Section 529(a) by adding the following words 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." It also added the requirement that the sponsor of a rare pediatric disease product application that intends to request a voucher for a rare pediatric disease product notify the HHS Secretary of such intent upon submission of the rare pediatric disease product application. In addition, the law required that GAO study the voucher program and report to Congress, by January 31, 2022, on the program's effectiveness as an incentive for developing drugs that treat or prevent rare pediatric diseases and that would not otherwise have been developed.

[57] FDA News Release, "FDA approves Vimizim to treat rare congenital enzyme disorder," February 14, 2014, see http://www.fda.gov/NewsEvents/Newsroom/PressAnnouncements/ucm386008.htm.

[58] RAPS, "First Pediatric Priority Review Voucher Goes up for Sale, Fetching $67M," July 31, 2014, see http://www.raps.org/Regulatory-Focus/News/2014/07/31/19905/First-Pediatric-Priority-Review-Voucher-Goes-up-for-Sale-Fetching-67M/#sthash.RsUDF53u.dpuf. See also "Drug Makers Buy Pricey Vouchers to Speed Products to Market," http://www.wsj.com/articles/drug-firms-buy-pricey-vouchers-to-speed-products-to-market-1445333403.

[59] H.R. 6, Section 2152, Reauthorization of Rare Pediatric Disease Priority Review Voucher Incentive Program, also contained a comparable provision. For additional information, see CRS Report R44502, *Senate Medical Innovation Bills: Overview and Comparison with the 21st Century Cures Act (H.R. 6)*, coordinated by C. Stephen Redhead and Amanda K. Sarata.

Provision

Section 3013 extends the authority to award such priority review vouchers until September 30, 2020. A new drug application or a biologics license application submitted to FDA after the enactment of the 21st Century Cures Act for a product designated as a rare pediatric disease drug before September 30, 2020, remains eligible to receive a priority review voucher, provided it is approved by September 30, 2022. This provision also removes the requirement that GAO study the pediatric priority review program under FFDCA Section 529.

Section 3014. GAO Study of Priority Review Voucher Programs

Under the Prescription Drug User Fee Act (PDUFA) of 1992, FDA agreed to specific goals for improving the drug review time and created a two-tiered system of review times: Standard Review and Priority Review. Compared with the amount of time standard review generally takes (approximately 10 months), a priority review designation means FDA's goal is to take action on an application within 6 months.[60] An application for a drug may receive priority review designation if it is for a drug that treats a serious condition and, if approved, would provide a significant improvement in safety or effectiveness.

An application may also receive priority review if it is the subject of a priority review voucher. Currently, FDA has two authorized priority review voucher programs (the rare pediatric disease priority review program, and the tropical disease priority review program), funded by user fees, which provide a transferable voucher, under specified conditions, to a sponsor of an approved new drug or biological product to be used for the priority review of another application. The purpose of the priority review drug voucher programs is to incentivize development of new treatment for diseases that may otherwise not attract development interest from companies due to either cost or lack of market opportunities. Section 3086 of this bill creates a third priority review voucher program to encourage the development of drugs and vaccines for agents that present a threat to national security.

Provision

Section 3014 requires the Comptroller General to conduct a study addressing the effectiveness and impact of three FDA priority review voucher programs: (1) the neglected tropical disease priority review voucher program, (2) the rare pediatric disease priority review voucher program, and (3) the priority review voucher program for drugs and vaccines to treat agents that present a national security threat. It requires the Comptroller General to submit a report to Congress with specified contents (including drug indications, value of the voucher, resources used for drug review under these programs, and consideration of program improvements) by January 31, 2020. The Comptroller is directed to conduct the study and issue the specified reports in a way that does not compromise national security.

Section 3015. Amendments to the Orphan Drug Grants

The Orphan Drug Act of 1983 (P.L. 97-414) was signed into law to incentivize development of drugs to treat rare diseases, each of which affects fewer than 200,000 individuals in the United States. Since the law's passage, FDA has approved over 400 new orphan drugs and biological products.[61] Incentives for sponsors of orphan drugs include seven years of market exclusivity, tax

[60] FDA, Priority Review, http://www.fda.gov/ForPatients/Approvals/Fast/ucm405405.htm.

[61] FDA, Office of Orphan Products Development, see http://www.fda.gov/AboutFDA/CentersOffices/OfficeofMedicalProductsandTobacco/OfficeofScienceandHealthCoordination/ucm2018190.htm.

credits for clinical trial expenses, user fee waivers, and eligibility for federal grants to cover costs of qualified clinical testing expenses.

The FFDCA contains provisions to grant market exclusivity for statutorily defined time periods (in months or years) to the holder of an approved drug application for a product that is, for example, a drug used in the treatment of a rare disease or condition, the first generic version of a drug to come to market, 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.

Section 5 of the Orphan Drug Act (21 U.S.C. 360ee) allows the HHS Secretary to make grants and enter into contracts with certain entities to assist in "defraying the costs of qualified clinical testing expenses incurred in connection with the development of drugs for rare diseases and conditions." Section 5 defines "qualified testing" as human clinical testing

> (i) which is carried out under an exemption for a drug for a rare disease or condition under section 505(i) of the Federal Food, Drug, and Cosmetic Act (or regulations issued under such section); (ii) which occurs after the date such drug is designated under section 526 of such Act and before the date on which an application with respect to such drug is submitted under section 505(b) or under section 351 of the Public Health Service Act; and (B) preclinical testing involving a drug is designated under section 526 of such Act and before the date on which an application with respect to such drug is submitted under section 505(b) or under section 351 of the Public Health Service Act.

Provision

Section 3015 amends Section 5 of the Orphan Drug Act (21 U.S.C. 360ee) to broaden the use of grants made by the HHS Secretary to assist in "defraying the costs of developing drugs for rare diseases or conditions" to include "prospectively planned and designed observational studies and other analyses conducted to assist in the understanding of the natural history of a rare disease or condition and in the development of a therapy."

Section 3016. Grants for Studying Continuous Drug Manufacturing

In March 2015 congressional testimony, the then FDA Commissioner spoke of new manufacturing technologies that could eventually "lower costs, limit drug shortages, and reduce supply chain vulnerabilities."[62] 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

Section 3016 allows the HHS Secretary 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."

[62] 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.

Subtitle C-Modern Trial Design and Evidence Development

Section 3021. Novel Clinical Trial Designs

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.[63]

Provision

Section 3021 requires the HHS Secretary to conduct a public meeting and issue guidance to assist sponsors in "incorporating complex adaptive and other novel trial designs into proposed clinical protocols and applications for new drugs" under FFDCA Section 505 and biological products under PHSA Section 351. Such guidance must address, for example, the use of complex adaptive and other novel trial designs, including how such trials "help to satisfy the substantial evidence standard" under FFDCA Section 505(d), and the types of quantitative and qualitative information that should be submitted for review. Prior to updating or issuing guidance, the HHS Secretary is required to consult with stakeholders through a public meeting. Not later than 18 months after the date of the public meeting, the HHS Secretary, acting through the FDA Commissioner, must update or issue draft guidance, and final guidance not later than one year after the close of the public comment period on the draft.

Section 3022. Real World Evidence

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

[63] 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.

> 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 rule (21 C.F.R. 314.126) describes 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 should "consider" these characteristics in its determination of effectiveness claims.

Provision

Section 3022 adds a new FFDCA Section 505F, "Utilizing Real World Evidence," requiring the HHS Secretary to "establish a program to evaluate the potential use of real world evidence to help to support the approval of a new indication for a drug approved under Section 505(c) and to help support or satisfy postapproval study requirements." The provision defines "real world evidence" as "data regarding the usage, or the potential benefits or risks, of a drug derived from sources other than randomized clinical trials."

Section 3022 requires the HHS Secretary to establish a draft framework for implementing the program to include specified content (e.g., sources of real world evidence such as ongoing safety surveillance, observational studies, claims, and patient-centered outcomes research activities). It also requires the HHS Secretary, in developing the framework, to consult with interested parties, which could be done via a public-private partnership or a contract, grant, or other appropriate arrangement. The HHS Secretary is required to use the new "program to evaluate the potential use of real world evidence" to inform the development of guidance for industry. This section is not to be construed to alter the standards of evidence for approval of drugs or biologics, including the substantial evidence standard, or to alter "the Secretary's authority to require postapproval studies or clinical trials, or the standards of evidence under which studies or trials are evaluated."

Sections 3023-3024. Protection of Human Research Subjects, Informed Consent Waiver or Alteration for Clinical Investigations

Provisions

The HHS Human Subject Regulations are a core set of federal standards for protecting human subjects in HHS-sponsored research.[64] These regulations are commonly referred to as the "Common Rule" because the same requirements have been adopted by many non-HHS federal departments and agencies, who apply the regulations to the research they fund. Under the Common Rule, research protocols must be approved by an Institutional Review Board (IRB) to

[64] 45 C.F.R. Part 46, Subpart A.

ensure that the rights and welfare of the research subjects are protected.[65] The rule lists several criteria for IRB approval, including the requirement that researchers obtain the informed consent of their research subjects.[66] 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.[67] 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.[68]

HHS has promulgated additional protections for certain vulnerable populations involved in research. Those groups include pregnant women, human fetuses, and neonates; prisoners; and children.[69]

FDA has issued its own set of Human Subject Regulations, which are similar, but not identical, to the Common Rule.[70] 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 currently approved by FDA for diagnosing or treating diseases or conditions that affect fewer than 4,000[71] individuals in the United States each year, ***may*** be used in a facility only after a local IRB has approved their use in that facility, except in certain emergency situations.[72]

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."[73] 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. Last fall, HHS and 15 other federal departments and agencies jointly released a proposed rule to amend the Common Rule.[74] A final rule has not yet been published.

Provisions

Section 3023 requires the HHS Secretary, to the extent possible, to harmonize differences between the HHS Human Subject Regulations and the FDA Human Subject Regulations. The HHS Secretary is 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,

[65] 45 C.F.R. §46.109.

[66] 45 C.F.R. §46.111(a)(4).

[67] 45 C.F.R. §46.116(a).

[68] 45 C.F.R. §46.116(d).

[69] 45 C.F.R. Part 46, Subparts B (pregnant women, fetuses, neonates), C (prisoners), and D (children).

[70] 21 C.F.R. Parts 50, 56, 312, and 812.

[71] Section 3052 of this act changed "fewer than 4,000" to "not more than 8,000."

[72] FFDCA §520(m)(4).

[73] 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.

[74] 80 *Federal Register* 53931, September 8, 2015.

and mechanisms to protect vulnerable populations; and to 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 three years of enactment, the HHS Secretary, in consultation with specified stakeholders, is required to issue regulations or guidance as necessary to implement the harmonization required under this section. The HHS Secretary is further required to submit, within two years of enactment, a report to Congress on the progress made toward completing such harmonization.

Section 3024 amends FFDCA Section 520(g) ("Exemption for Devices for Investigational Use") to allow the HHS Secretary, subject to such conditions as he may prescribe, 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.

Section 3024 also amends FFDCA Section 505(i) (regarding the investigational use of drugs) to modify the existing requirement for informed consent as a condition of the HHS Secretary granting an exemption to allow manufacturers, or sponsors of investigations, to not require certification of informed consent for individuals participating in the clinical trial of a drug if it is not feasible, if it is contrary to the best interest of human beings, or if *the trial poses no more than minimal risk to the participants and includes appropriate safeguards to protect their rights, safety, and welfare* (new language in italics).

Subtitle D-Patient Access to Therapies and Information

Section 3031. Summary Level Review

FFDCA Section 505 and accompanying regulations provide the framework for FDA's approval of a sponsor's new drug application (NDA). For a drug whose active ingredient has never been FDA-approved, the law requires the sponsor to submit an NDA 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))

The clinical data submission must also include an "integrated summary of the data demonstrating substantial evidence of effectiveness for the claimed indications."[75]

[75] 21 C.F.R. §314.50(d)(5)(v).

Provision

Section 3031 amends FFDCA Section 505(c) and PHSA Section 351(a)(2) to permit the HHS Secretary to rely upon "qualified data summaries" to support the approval of a supplemental NDA submitted by the sponsor of an approved drug seeking to add a new "qualified" indication to the approval. A "qualified indication" is one "for a drug that the HHS Secretary determines to be appropriate for summary level review." This provision adds that such supplemental application is eligible only if data demonstrating the safety of the drug are available and acceptable to the HHS Secretary, and all data used to develop the qualified data summaries are submitted as part of the supplemental NDA. It requires the HHS Secretary to post on the FDA website, and update annually, the number of applications reviewed solely based on a qualified data summary, and the average time for completion of reviews using and not using the review flexibility, among other specified information. This section defines qualified data summary as a "summary of clinical data that demonstrates the safety and effectiveness of a drug with respect to a qualified indication."

Section 3032. Expanded Access Policy

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."[76]

If excluded from a clinical trial because of 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."[77] 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 may 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

Section 3032 adds a new FFDCA Section 561A, "Expanded Access Policy Required for Investigational Drugs," to require a manufacturer or distributor of an investigational drug to be used for a serious disease or condition to make its policies on evaluating and responding to compassionate use requests publicly available. Required elements of the policy include contact information for the manufacturer or distributor of the drug, request procedures, "the general

[76] CRS Report R44134, *Access to Unapproved Drugs: FDA Policies on Compassionate Use and Emergency Use Authorization*, by Susan Thaul.

[77] FFDCA §561(b).

criteria the manufacturer or distributor will use to evaluate such requests for individual patients, and for responses to such requests," anticipated time to acknowledge request receipts, and a hyperlink or other reference to the clinical trial record containing information about expanded access to the drug. The new section states that posting of policy would not guarantee patients access to an investigational drug. The provision also allows a manufacturer or distributor to revise its policy at any time. Section 3032 becomes effective on the later of the date that is 60 days after the enactment of the 21st Century Cures Act or "the first initiation of a phase 2 or phase 3 study ... with respect to such investigational drug."

Sections 3033-3036. Regenerative Therapies

Regenerative medicine is defined by NIH as "the process of creating living, functional tissues to repair or replace tissue or organ function lost due to age, disease, damage, or congenital defects."[78] The regulation of cells or tissues intended for implantation or infusion into a human patient is the responsibility of the FDA Center for Biologics Evaluation and Research (CBER). FDA refers to such cells as HCT/Ps, which stands for human cells, tissues, and cellular and tissue-based products. Stem cells are one example of HCT/P. CBER held a public workshop on standards development for cellular therapies and regenerative medicine products in March 2014.

Provisions

Section 3033 adds a paragraph (g) to FFDCA Section 506 to require the HHS Secretary, at the request of the sponsor of a drug, to facilitate an "efficient development program for, and expedite review of" a drug that qualifies as a regenerative advanced therapy. To be eligible for such designation, the drug must (1) be a regenerative medicine therapy, (2) be intended to treat, modify, reverse, or cure a serious or life-threatening disease or condition, and (3) have preliminary clinical evidence indicating that the drug has the potential to address unmet medical needs for such a disease or condition. This designation may be requested with or after submission of an investigational new drug (IND) application. An application regarding a regenerative medicine therapy is eligible for priority review and for accelerated approval in addition to "early interactions [with FDA] to discuss any potential surrogate or intermediate endpoint." The term "regenerative medicine therapy" includes cell therapy, therapeutic tissue engineering products, human cell and tissue products, and combination products using any such therapies or products, except for those regulated under PHSA Section 361 and 21 C.F.R. 1271. This section specifies the procedure through which the sponsor of a drug could request such designation, how the HHS Secretary would respond to the request, and postapproval requirements. This section is not to be construed to alter the authority of the HHS Secretary to approve drugs and license biologics pursuant to the FFDCA and PHSA Section 351, respectively, including standards of evidence, or to alter the requirement of postapproval studies.

Section 3034 requires the HHS Secretary, acting through the FDA Commissioner, to issue draft guidance within one year of enactment of the 21st Century Cures Act, and final guidance not later than 12 months after the close of the public comment period on the draft guidance, "clarifying how, in the context of regenerative advanced therapies, the HHS Secretary will evaluate devices used in the recovery, isolation, or delivery of regenerative advanced therapies," as specified.

Section 3035 requires that before March 1 of each calendar year, with respect to the previous calendar year, the HHS Secretary submit a report to Congress on (1) the number and type of

[78] FDA, Public Workshop: Synergizing Efforts in Standards Development for Cellular Therapies and Regenerative Medicine Products, March 31, 2014. Agenda, transcript, presentation slides at http://www.fda.gov/biologicsbloodvaccines/newsevents/workshopsmeetingsconferences/ucm364114.htm.

applications for regenerative advanced therapies filed, approved or licensed, withdrawn, or denied, and (2) the number of such applications or therapies that were granted accelerated approval or priority review.

Section 3036 amends the FFDCA by adding a new Section 506G, "Standards for Regenerative Medicine and Regenerative Advanced Therapies." This section requires the HHS Secretary, in consultation with the National Institute of Standards and Technology (NIST) and specified stakeholders, to facilitate an effort toward the development of standards for regenerative medicine and advanced therapies through a transparent public process to support, such as "through regulatory predictability, the development, evaluation, and review of" such therapies. It also requires the HHS Secretary to review and update relevant regulations and guidance, as appropriate.

This section further requires the Office of Combination Products (OCP) to help coordinate timely review of combination products across relevant agency centers and to ensure that persons are designated in each primary agency center as points of contact for the sponsors of combination products. It specifies additional duties for OCP related to communication, and facilitating meetings between the agency and the sponsors. It requires the HHS Secretary, not later than four years after enactment and after a public comment period, to issue final guidance on the combination product review process, as specified, and adds reporting requirements to the annual report to Congress on the activities of OCP, as specified.

It amends FFDCA Section 520(h)(4) to prohibit the use of information contained in an application for premarket approval of a class III device from being used in an application for premarket approval of a combination product that contains an approved drug constituent, unless the applicant provides a patent certification and notifies the holder of the approved application and patent owner that the patent is invalid or will not be infringed upon.

It also requires the HHS Secretary to identify, not later than 18 months after enactment, types of combination products and manufacturing processes that the HHS Secretary proposes may adopt different good manufacturing processes or streamlined mechanisms. This list is to be published in the *Federal Register*, finalized after public comment, and updated as needed.

Section 3037. Health Care Economic Information

Under FFDCA Section 502, a drug or device is deemed to be misbranded if, among other things, its labeling is false or misleading. Section 502(a) specifies that health care economic information provided in the course of selecting drugs for managed care or other similar organizations, by a formulary committee or similar entity, is not to be considered false or misleading if the information "directly relates" to a use of the drug as approved under FFDCA Section 505 or licensed under PHSA Section 351(a); the information must also be based on competent and reliable scientific evidence. Information that helps substantiate the health care economic information presented in accordance with this section must be made available to the HHS Secretary upon request. Health care economic information is defined 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."

Health care providers generally may prescribe a drug for an unapproved use when they judge that it is medically appropriate for their patient (often called "off-label" use).[79] Drug companies,

[79] FDA, "Understanding Unapproved Use of Approved Drugs "Off Label," http://www.fda.gov/ForPatients/Other/OffLabel/default.htm.

however, are not allowed to promote or distribute information about unapproved uses that have not been determined by FDA to be safe and effective. Drug and device companies have argued that current regulations prevent them from distributing important information to physicians about off-label uses of their products.[80] In November 2016, FDA held a two-day public meeting to obtain input from various groups regarding off-label uses of approved or cleared medical products.[81]

Provision

Section 3037 amends FFDCA Section 502(a) by allowing drug and device companies to promote health care economic information to payors (e.g., insurance companies), in addition to formulary committees and other similar entities "with knowledge and expertise in the area of health care economic analysis." This section allows for health care economic information to be "related" to an FDA-approved indication rather than "directly-related" as required by current law. This section maintains the "competent and reliable scientific evidence" standard and adds that, where applicable, the health care economic information must be accompanied by "a conspicuous and prominent statement describing any material differences" between the health care economic information and a product's approved labeling.

It also amends the definition of health care economic information by (1) expanding the scope of the term "any analysis" to include "clinical data, inputs, clinical or other assumptions, methods, results, and other components underlying or comprising the analysis"; (2) specifying that analyses comprising health care economic information could be based on the economic consequences of the use of a drug, which may in turn be based on the separate or aggregated clinical consequences of the represented health outcomes; and (3) adding that health care economic information does not include analyses that relate only to a non-FDA-approved indication.

Section 3038. Combination Product Innovation

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 (CDER) reviews new drug applications for approval, the Center for Biologics Evaluation and Research (CBER) reviews biologics license applications for licensure, and the Center for Devices and Radiological Health (CDRH) reviews premarket approval applications for approval and 510(k) notifications for clearance.

In 2002, Congress directed FDA to establish an Office of Combination Products (OCP) to facilitate the timely review and regulation of drug-device, drug-biologic, and device-biologic combination products, pursuant to the requirements in FFDCA Section 503. Both drugs and devices are defined in the FFDCA as products intended to diagnose, prevent, or treat disease, or otherwise affect the structure or any function of the body. Unlike a drug, however, a device "does not achieve its primary intended purposes through chemical action within or on the body ... and is not dependent upon being metabolized for the achievement of its primary intended purposes."[82] OCP is required to determine the primary mode of action of a combination product and regulate it

[80] STAT, "FDA to hold long-awaited meeting to review off-label marketing," August 31, 2016, https://www.statnews.com/pharmalot/2016/05/27/fda-hhs-free-speech-patient-safety/.

[81] FDA, Docket No. FDA-2016-N-1149, https://s3.amazonaws.com/public-inspection.federalregister.gov/2016-21062.pdf.

[82] FDA, "Is the Product a Medical Device?" See http://www.fda.gov/MedicalDevices/DeviceRegulationandGuidance/Overview/ClassifyYourDevice/ucm051512.htm.

based on that determination. Generally, OCP treats a drug-device combination product as a drug unless the manufacturer can prove that it satisfies the device exclusionary clause (i.e., the product does not rely on chemical action to achieve its primary intended purpose).

A manufacturer whose product is assigned to CDER will have a higher standard of evidence, a potentially higher requirement for supporting data, a higher user fee, and probably a longer premarket review time period than a manufacturer whose product is assigned to CDRH.

Provision

Section 3038 amends FFDCA Section 503(g) to require the HHS Secretary to assign a primary center for the regulation of combination products and to conduct premarket review of these products under a single application whenever appropriate, among other things. It requires the HHS Secretary to determine the primary mode of action for a combination product—defined as the single mode of action expected to make the greatest contribution to the overall intended therapeutic effects of the product—in order to determine how best to review the product. The HHS Secretary is not permitted to determine that the primary mode of action is that of a drug or biologic solely because the combination product has any chemical action within or on the body.

If the sponsor of a combination product disagrees with the HHS Secretary's determination and requests an explanation, the HHS Secretary must provide a substantive scientific rationale for the determination. In addition, the sponsor may propose and, subject to an agreement with the HHS Secretary, conduct additional studies to establish the relevance of any chemical action in the product's primary mode of action. The sponsor may also request a meeting on such combination product, as specified, "to establish clarity and certainty." This paragraph also specifies other procedures for communication between the agency and the sponsor.

For premarket review of a combination product that includes an approved constituent component (e.g., a drug or device), the HHS Secretary is allowed to require that a sponsor submit only that information that is necessary to determine the safety of the combination product, including any incremental risks or benefits posed by the product, taking into account any prior findings for the approved constituent parts.

For a combination product submitted through a device pathway (515, 510(k), or 513(f)(2)) that contains an approved drug constituent, the applicant must certify any patents that claim the approved drug or its use for which the applicant has not obtained a right of reference,[83] and give notice to the holder of the approved application and patent owner that the patent is invalid or will not be infringed upon. [84]

A combination product containing an approved drug constituent that is submitted through a device pathway is eligible for certain regulatory exclusivity periods: new chemical entity exclusivity (five years), new clinical investigation exclusivity three years), pediatric exclusivity (six months), qualified infectious disease product exclusivity (five years), and orphan drug exclusivity (seven years). Notwithstanding any other provision of this subsection, an application for a combination product submitted through a device pathway that contains an approved drug constituent would be considered a 505(b)(2) application[85] for specified purposes. It does not

[83] Such patent information is generally published in the Orange Book when the application is approved.

[84] Right of reference means "the authority to rely upon, and otherwise use, an investigation for the purpose of obtaining approval of an application, including the ability to make available the underlying raw data from the investigation for FDA audit, if necessary." 21 C.F.R. 314.3.

[85] A 505(b)(2) application is one for which one or more of the investigations relied upon by the applicant for approval

prohibit a sponsor from submitting separate applications for the constituent parts of a combination product, unless the HHS Secretary determines that a single application is necessary.

This section further requires OCP to help coordinate timely review of combination products across relevant agency centers and to ensure that persons are designated in each primary agency center as points of contact for the sponsors of combination products. It specifies additional duties for OCP related to communication and facilitating meetings between the agency and the sponsors. It requires the HHS Secretary, not later than four years after enactment and after a public comment period, to issue final guidance on the combination product review process, as specified, and adds reporting requirements to the annual report to Congress on the activities of OCP as specified.

It amends FFDCA Section 520(h)(4) to prohibit the use of information contained in an application for premarket approval of a class III device from being used in an application for premarket approval of a combination product that contains an approved drug constituent, unless the applicant provides a patent certification and notifies the holder of the approved application and patent owner that the patent is invalid or will not be infringed upon.

It also requires the HHS Secretary to identify, not later than 18 months after enactment, types of combination products and manufacturing processes that the HHS Secretary proposes may adopt different good manufacturing processes or streamlined mechanisms. This list is to be published in the *Federal Register*, finalized after public comment, and updated as needed.

Subtitle E-Antimicrobial Innovation and Stewardship

Section 3041. Antibacterial Resistance Monitoring.

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. The U.S. National Strategy for Combating Antibiotic-Resistant Bacteria (CARB) identifies five interrelated goals to control antibiotic resistance (AR):

- Antibiotic Stewardship: the judicious use of antibiotics in health care and agricultural settings;
- One Health Surveillance: integration of public health and animal disease, food, and environmental surveillance for resistant bacteria;
- Diagnostic Innovations: new technologies such as "point-of-care" antibiotic susceptibility tests and tests to identify viral infections;
- Treatment, Prevention, and Control Research and Development: efforts to boost basic research, facilitate clinical trials of new antibiotics, attract private investment, and increase the number of antibiotic drug candidates in the drug development pipeline; and
- International Collaboration: engagement in global AR activities through multiple venues.

"were not conducted by or for the applicant and for which the applicant has not obtained a right of reference or use from the person by or for whom the investigations were conducted." FFDCA §505(b)(2)).

Provision

Section 3041, "Antibacterial Resistance Monitoring," defines the term "antimicrobial" to include any antibacterial or antifungal drugs, and may include drugs that eliminate or inhibit the growth of other microorganisms, as appropriate. The section amends PHSA Section 319E to require the HHS Secretary to carry out the following activities, as specified: (1) encourage and assist in reporting of antimicrobial drug use, drug resistance, and antimicrobial stewardship programs in health care facilities of the Indian Health Service (IHS), Department of Veterans Affairs (VA), and Department of Defense (DOD); (2) report annually on antimicrobial drug resistance trends, stewardship programs, and other matters; (3) provide guidance and other informational materials about antimicrobial stewardship for residential and ambulatory health care facilities; (4) assist states with their antimicrobial resistance prevention activities; and (5) establish a mechanism for facilities to report antimicrobial stewardship activities and evaluate drug resistance data, including for drugs approved under the Limited Population Pathway established in Section 3042 of this Act. The HHS Secretary is required, consistent with laws prohibiting disclosure of trade secret and confidential information, to make data collected pursuant to this section publicly available.

Section 3042. Limited Population Pathway.

Antimicrobial drugs are intended for short-term use, making the development of new ones potentially less attractive to drug developers. Addressing barriers to antimicrobial drug approval may help counter this problem. The so-called limited population drug approval pathway for new antimicrobial drugs is one such approach, involving 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 antimicrobial drug.

Provision

Section 3042 creates a new product review pathway in FFDCA Section 506(h), "Limited Population Pathway for Antibacterial and Antifungal Drugs." This pathway allows the HHS Secretary to approve an antibacterial or antifungal drug as a limited population drug if (1) the drug is intended to treat a serious or life-threatening infection in a limited population of patients with unmet needs; (2) the standards for new drug approval or biologics licensure are met; and (3) the HHS Secretary receives a written request from the sponsor to approve the drug as a limited population drug. This review pathway includes the following elements, among others:

- The HHS Secretary's determination of the safety and efficacy of a limited population drug reflects the benefit-risk profile of the drug in the intended limited population, considering the availability or lack of alternative treatments for this population.
- Products approved using this pathway must carry prominent labeling noting, among other things, 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 expedited development or approval pathways, as applicable. Section 3042 also requires the HHS Secretary to issue, within 18 months of enactment, draft guidance describing criteria, processes, and other considerations for demonstrating the safety and effectiveness of limited population drugs, and final guidance within

18 months of the close of the public comment period on the draft guidance. It requires the HHS Secretary to provide prompt advice to the sponsor regarding the approval of a limited population drug. If such drug obtains approval for a broader indication, this legislation allows the HHS Secretary to remove any post-marketing conditions, such as labeling and promotional review requirements, regarding limited population use. The HHS Secretary must report to Congress at least every two years on the number of requests for approval and the number of approvals of limited population drugs. The Comptroller General must report by December 2021 on activities under this section, the extent to which the limited population drug pathway facilitated approval of treatments for limited populations, and the effects of such pathway, if any, on antimicrobial and antifungal drug resistance. This section shall not be construed to alter the HHS Secretary's authority to approve drugs pursuant to the FFDCA or PHSA.

Section 3043. Prescribing Authority.

In general, the FFDCA regulates the actions of product sponsors in marketing, labeling, and promotion of medical products, but does not regulate the actions of health care providers engaged in the practice of medicine. Consequently, providers are generally allowed to prescribe drugs for uses or at doses other than those uses or doses approved by FDA. This is referred to as "off-label" use.

Provision

Section 3043 states that provisions in Subtitle E and any amendments to them shall not be construed to restrict the prescribing authority of health care professionals (such as off-label prescribing) or limit the practice of health care.

Section 3044. Susceptibility Test Interpretive Criteria for Microorganisms; Antimicrobial Susceptibility Testing Devices.

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 FDA to make changes to information contained in these drug and device labels, and FDA must pre-approve label changes for these drugs and devices. However, the susceptibility of infectious organisms may change over time, sometimes rapidly, 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.

Provision

Section 3044 establishes a new FFDCA Section 511A, "Susceptibility Test Interpretive Criteria for Microorganisms." The stated purpose is to clarify the HHS Secretary's authority to efficiently update susceptibility test interpretive criteria to address the development of drug resistance; to provide for public notice of the availability of recognized interpretive criteria and interpretive criteria standards; and to clear (under FFDCA Section 510(k)), classify (under FFDCA Section 513(f)(2)), or approve (under FFDCA Section 515) AST devices using updated, recognized susceptibility test interpretive criteria.

The section requires the HHS Secretary to identify appropriate susceptibility test interpretive criteria, as specified, and to, within one year of enactment, establish and maintain a public "Interpretive Criteria Website," listing (1) 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) criteria that, although determined by the HHS Secretary to be appropriate with respect to approved or licensed antimicrobial drugs, lack a recognized standard, for one of several stated reasons. The website must include several specific disclaimers regarding the uses and limitations of the information presented. The HHS Secretary is 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 provision clarifies that reference to the website in the labeling of an antimicrobial drug does not constitute misbranding, and states that FFDCA Section 511A shall not be construed to allow the HHS Secretary to disclose protected trade secret or confidential information.

The HHS Secretary is required to review any new or updated criteria standards from a recognized standard development organization, and other changes to interpretive criteria, revise the website accordingly, and publish a notice of any such revisions on the FDA agency website, at least every six months. The website must also be revised upon the approval of any antimicrobial drug when such approval is based on criteria other than those already listed. All such notices must be compiled and published in the Federal Register at least annually, with a request for public comments. The HHS Secretary shall consider public comments, among other things, in revising website content.

Both criteria standards and non-standard criteria listed on the website are 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 may use standards other than those listed by FDA under this section in seeking approval or clearance of a drug or device. Antimicrobial drugs approved after the Interpretive Criteria Website is established must carry a reference to the website on the label. Sponsors of antimicrobial drugs approved before the website is established must submit, within one year of establishment of the website, supplemental applications to similarly change the label.

Section 3044 allows the HHS Secretary, so long as other requirements for clearance are met, 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 antimicrobial drugs for which susceptibility is measured using such device.

Finally, the section explicitly recognizes interpretive criteria standards posted on the Interpretive Criteria Website as device standards. It also (1) requires the HHS Secretary to report to Congress regarding progress in implementing this section; and (2) exempts FDA from requirements under the Paperwork Reduction Act when updating the list of susceptibility test interpretive criteria standards. This section shall not be construed to alter the HHS Secretary's authority to clear devices pursuant to the FFDCA or PHSA.

Subtitle F-Medical Device Innovations

Section 3051. Breakthrough Devices

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 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 premarket approval (PMA) pathway (*approval*) and (2) the 510(k) pathway (*clearance*).[86] 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 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.

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 HHS Secretary is required to provide review priority for devices that represent breakthrough technologies for which no approved alternatives exist, that offer significant advantages over existing approved alternatives, or whose availability is in the best interest of the patients.

To implement this requirement, the FDA, on April 23, 2014, 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 premarket approval (PMA) medical devices; FDA issued final guidance on April 13, 2015, that addressed de novo 510(k) devices, which it concluded are not eligible for the full scope of the priority review program.[87]

The guidance focuses on balancing risks versus benefits for patients, drafting a Data Development Plan by the medical device sponsor, and collecting postmarket data on medical devices that have received a priority review designation. As described in the FDA guidance, for a medical device that has received a priority review designation, the expedited review process involves lower requirements in the premarket review process, such as less information in the PMA application, in exchange for increased collection of postmarket data and reliance on the use of surrogate endpoints.[88] According to FDA, the Expedited Access PMA (EAP) program features

[86] This pathway involves submitting a premarket notification, also known as a 510(k) after the section in the FFDCA that authorized this type of notification.

[87] 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, at 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), a modified type of 510(k) review pathway, 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." For a definition of EAP, see page 9.

[88] 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

"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."[89] 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 prove the device is not safe and effective.

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."[90] 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."[91] 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.[92]

Provision

Section 3051 adds a new Section 515C, "Breakthrough Devices," to Chapter V of the FFDCA. The new section requires the HHS Secretary to establish a program to provide priority review for devices that (1) provide more effective diagnosis or treatment of a life-threatening or irreversibly debilitating condition, and (2) represent breakthrough technologies for which no approved alternatives exist, offer significant advantages over existing alternatives, or are, once available, in the best interest of patients. The section allows requests from device sponsors for designation of priority review of not only PMA medical devices, but also 510(k) devices and one other type of regulatory decision involving a medical device.[93]

The section requires the HHS Secretary in 60 days to determine whether the request for priority review would be granted. Such requests are to be evaluated by a team of experienced FDA staff and senior managers.

If the HHS Secretary approves a priority review designation for a device, the HHS Secretary may not withdraw the designation because another "breakthrough" device was subsequently cleared or approved, thereby resulting in the 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) no longer being met.

Each priority review device is assigned a team of staff, "including a team leader with appropriate subject matter expertise and experience." Senior FDA personnel oversee each team to facilitate the efficient development and review of the device. Among other things, the HHS Secretary is required to "provide for interactive communication with the device sponsor during the review

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)."

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

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

[91] 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.

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

[93] A petition for classification under FFDCA Section 513(f)(2).

process," and expedite "the Secretary's review of manufacturing and quality systems compliance." The HHS Secretary is required to "disclose to the sponsor, not less than 5 business days in advance the topics of any consultation concerning the sponsor's device that the HHS Secretary intends to undertake with external experts or an advisory committee and provide the sponsor an opportunity to recommend such external experts."

The HHS Secretary may, as appropriate, "coordinate with the sponsor regarding early agreement on a data development plan." The HHS Secretary may ensure that clinical trial design is as efficient as practicable and may facilitate "expedited and efficient development and review of the device through utilization of timely postmarket data collection" with regard to PMA applications. Agreements on clinical protocols are binding, but they may be subject to change under certain circumstances. The provision specifies that both the agreement and subsequent changes to the clinical protocol must be agreed to in writing.

The HHS Secretary is required to issue, not later than one year after enactment, guidance on the implementation of the new Section 515C of the FFDCA. In addition, the HHS Secretary is required to issue a report, on January 1, 2019, to the Senate Health, Education, Labor and Pensions Committee and the House Energy and Commerce Committee describing the program added under new FFDCA Section 515C, including recommendations to strengthen the program and better meet patient needs in a timely manner.

Section 3052. Humanitarian Device Exemption

The Humanitarian Device Exemption (HDE) was intended to encourage the development of devices that help treat and diagnose diseases or conditions that affect fewer than 4,000 individuals in the United States per year.[94] 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

Section 3052 amends FFDCA Section 520(m) and allows 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 HHS Secretary, acting through the Commissioner of the FDA, is 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 3053. Recognition of Standards

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.[95] Device classification determines the type of

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

[95] FFDCA §513(a)(1); see also 21 C.F.R. §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.

regulatory requirements that a manufacturer must follow. General controls are the minimum regulations that apply to all FDA-regulated medical devices.[96]

Class II devices are those under current law that "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."[97] Although Class II includes devices that pose a moderate risk to patients, currently only some have information—or special controls—available to reduce or mitigate risk. Special controls include special labeling requirements, premarket data requirements, postmarket surveillance, patient registries, guidelines, and performance standards.[98] According to a 2011 report by the Institute of Medicine, about "15% of all device types classified in Class II are subject to special controls."[99] This is because "FDA has not promulgated performance standards or special controls for the vast majority of types of Class II devices."[100] 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."[101] 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."[102]

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.[103]

Provision

Section 3053 amends FFDCA Section 514(c) by adding two new subparagraphs and two new paragraphs. Under the section, any person may 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 HHS Secretary is 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

[96] 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).

[97] FFDCA §513(a)(1)(B).

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

[99] 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.

[100] Ibid., p. 3.

[101] Ibid., p. 5.

[102] 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. GAO's legal name was changed from the General Accounting Office to the Government Accountability Office on July 7, 2004, by the GAO Human Capital Reform Act of 2004 (P.L. 108-271).

[103] FFDCA §514(c)(1)(A).

for such determination." The response and rationale for recognition must be made publically available.

Under the section, the HHS Secretary is 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 HHS Secretary must publish guidance identifying the principles for recognizing standards.

Section 3054. Certain Class I and Class II Devices

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.[104]

On July 1, 2015, FDA released guidance that exempts 120 medical devices from premarket notification requirements; draft guidance was issued on August 1, 2014.[105] The 120 devices are primarily Class II but 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."[106]

Provision

Section 3054 amends FFDCA Section 510(l) and requires the HHS Secretary, within 120 days of enactment and at least once every five years thereafter, as the HHS Secretary determines appropriate, 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 is exempt from the 510(k) requirement and the "classification regulation applicable to each such type of device" is deemed amended to incorporate the exemption.

Similarly, the section amends FFDCA Section 510(m) and requires the HHS Secretary, within 90 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 HHS Secretary must provide a 60-day public comment period after publication of such a list.

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[104] 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.

[105] 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.

[106] Ibid., pp. 4-5.

Not later than 210 days after enactment, the HHS Secretary is required to publish in the *Federal Register* a list representing the final determination on the types of Class II devices that no longer require a 510(k) notice prior to marketing. Upon publication of the final list, each type of Class II device so listed is exempt from the 510(k) requirement and the "classification regulation applicable to each such type of device" is deemed amended to incorporate the exemption.

Section 3055. Classification Panels

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."[107]

In April 2015, FDA issued draft guidance entitled "Procedures for Meetings of the Medical Devices Advisory Committee."[108] Once final, the draft guidance will replace two earlier FDA guidance documents.[109] 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.[110] The Medical Devices Advisory Committee includes 17 different advisory panels, which address topics in various specialty areas.[111] 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."[112]

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 HHS 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

[107] 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.

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

[109] The two documents are (1) *Guidance on Amended Procedures for Advisory Panel Meetings*, http://www.fda.gov/MedicalDevices/DeviceRegulationandGuidance/GuidanceDocuments/ucm073722.htm and (2) *Panel Review of Premarket Approval Applications #P91-2 (blue book memo)*, http://www.fda.gov/MedicalDevices/DeviceRegulationandGuidance/GuidanceDocuments/ucm081363.htm.

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

[111] 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.

[112] 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.

industry. Scientific, trade, and consumer organizations shall be afforded an opportunity to nominate individuals for appointment to the panels.[113]

Provision

Section 3055 amends FFDCA Section 513(b)(5) by adding two new subparagraphs that require the HHS Secretary to ensure that there is "adequate expertise" on a device classification panel, including by giving the device manufacturer the opportunity to provide advice "on the expertise needed among the voting members of the panel," among other things. The HHS Secretary is required to ensure this expertise when "a device is specifically the subject of review by a classification panel." The provision defines "adequate expertise" to mean that the classification panel reviewing a premarket submission includes "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."

Each year the HHS Secretary must provide an "opportunity for patients, representatives of patients, and sponsors of medical device submissions to provide recommendations for individuals with appropriate expertise to fill voting member positions on classification panels."

The section amends FFDCA Section 513(b)(6) regarding the panel review process and participation in the panel meeting, adding that the device manufacturer, or its representative, must 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 section strikes subparagraph (B) in FFDCA Section 513(b)(6) and replaces it with a similar subparagraph, delineating that adequate time for presentations is required to be provided to the device manufacturer and the HHS Secretary, and adds that the panel may pose questions to the representative of the manufacturer and consider the responses in the panel's review of the device.

Section 3056. Institutional Review Board Flexibility

The HHS Human Subject Regulations are a core set of federal standards for protecting human subjects in HHS-sponsored research.[114] These regulations are commonly referred to as the Common Rule because the same requirements have been adopted by many other federal departments and agencies, which 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 research subjects are protected.[115]

FDA has issued its own set of Human Subject Regulations, which are similar, but not identical, to the Common Rule.[116] 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. All clinical evaluations of investigational devices (unless exempt) must have an investigational device exemption (IDE) before the clinical study is initiated.[117] An IDE allows an unapproved device (most commonly an invasive or life-sustaining device) to be used in a clinical

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

[114] 45 C.F.R. Part 46, Subpart A.

[115] 45 C.F.R. §46.109.

[116] 21 C.F.R. Parts 50, 56, 312, and 812.

[117] See 21 C.F.R. §812. Devices are exempt from IDE requirements when testing is noninvasive, does not require invasive sampling, does not introduce energy into a subject, and is not stand-alone (i.e., is not used for diagnosis without confirmation by other methods or medically established procedures). See 21 C.F.R. §812.2(c)(3).

study to collect the data required to support a PMA submission.[118] The IDE permits a device to be shipped lawfully for investigation of the device without requiring that the manufacturer comply with other requirements of the FFDCA, such as registration and listing. Devices approved by FDA via the HDE are for diagnosing or treating diseases or conditions that affect fewer than 4,000 individuals in the United States each year. An HDE application is similar to a PMA, but it is exempt from the effectiveness requirements. Such devices may be used in a facility only after a local IRB has approved their use in that facility, except in certain emergency situations.[119]

Provision

Section 3056 amends FFDCA Section 520(g), regarding IDEs, and FFDCA Section 520(m), regarding HDEs, by removing the word "local" in all references to local IRBs, including in the stipulation that an approved humanitarian use device may be used in a facility only after a local IRB has approved such use, except in certain emergency situations.

Section 3057. CLIA Waiver Improvements

The Clinical Laboratory Improvement Amendments (CLIA) of 1988 (CLIA, P.L. 100-578) provide the Centers for Medicare & Medicaid Services (CMS) with authority to regulate clinical laboratories to ensure the accuracy of test results, given that these results affect clinical decisionmaking.[120] CLIA requires laboratories to receive certification before they are 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.[121] 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 by the test's manufacturer, and FDA has issued guidance to support the manufacturer's submission of this information.[122] Under current law, waived tests are those "that have been approved by FDA for home use or that, as determined by the HHS Secretary, are simple laboratory examinations and procedures that have an insignificant risk of an erroneous result."[123] The guidance recommends ways to demonstrate 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.)

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[118] FDA, *Device Advice: Investigational Device Exemption (IDE)*, July 9, 2009, http://www.fda.gov/MedicalDevices/DeviceRegulationandGuidance/HowtoMarketYourDevice/InvestigationalDeviceExemptionIDE/default.htm.

[119] FFDCA Section 520(m)(4).

[120] PHSA §353; 42 U.S.C. §263a.

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

[122] 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.

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

Provision

Section 3057 requires the HHS Secretary, acting through the FDA Commissioner, to, not later than one year after enactment, publish draft guidance that revises Section V of the current guidance, including providing clarification on the appropriate use of comparable performance between a waived and moderately complex laboratory user to demonstrate accuracy. It also requires the HHS Secretary, not later than one year after the comment period for the draft guidance closes, to publish final revised guidance.

Section 3058. Least Burdensome Device Review

Section 205 of the Food and Drug Administration Modernization Act of 1997 (FDAMA, P.L. 105-115) amended FFDCA Section 513, adding two provisions commonly referred to as the "Least Burdensome Provisions." (FFDCA Section 513(a)(3)(D)(ii) and Section 513(i)(1)(D)). 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. The two provisions are as follows:

FDA published final guidance on the least burdensome provisions on October 4, 2002.[124] Under the guidance, FDA may allow the use of non-clinical data—such as laboratory and/or animal testing—in place of clinical data for the approval of PMA devices in certain circumstances, such as "devices or modifications of approved devices for which scientifically valid information is available in the public domain."[125] When clinical data are needed, FDA allows manufacturers to consider study designs to shorten the length of the study. Such study designs include the use of "surrogate endpoints and statistical methods, such as Bayesian analyses," and study designs other than the gold standard—the randomized controlled trial.[126] Although FDA allows for substitution of laboratory data in certain circumstances, the absence of problems in laboratory testing may not always predict what happens to a device over time in the human body, where forces that cannot be replicated in laboratory testing act upon the device.

The 2002 FDA guidance states, "[r]eliance on postmarket controls (e.g., ... postmarket surveillance, and the Medical Device Reporting requirements) should be considered as a mechanism to reduce the premarket burden for 510(k)s and PMAs, while still ensuring the safety and effectiveness of the device."[127] However, the FDA's authority to require postmarket studies

[124] 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.

[125] Ibid.

[126] Ibid. In a randomized controlled trial (RCT), participants are randomly assigned to two or more groups. One group receives the intervention (the new treatment), while the control group receives current therapy or a 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 likely due to the intervention. The RCT is often called the gold standard of evidence for a clinical trial. A surrogate end point may not be a reliable predictor of actual patient benefit. It is a laboratory measurement, such as blood pressure or cholesterol level, used as a substitute for a clinically meaningful end point that measures directly how a patient feels, functions, or survives. The use of Bayesian analyses allows studies to be combined in order to reduce the sample size needed for the experimental and/or control device.

[127] 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.

of medical devices is limited. A September 2015 GAO study found that of the 392 postmarket surveillance studies ordered by FDA between May 1, 2008, and February 24, 2015, 88% were inactive, 10% were ongoing, and 2% were complete.[128] Activities related to implementing the least burdensome provision, including training for staff and advisory panels, are posted on FDA's website.[129]

Provision

Section 3058 amends FFDCA Section 513 by adding a new subsection (j), "Training and Oversight of Least Burdensome Requirements." The HHS Secretary must ensure that each FDA employee involved in the review of premarket submissions, including supervisors, receives training on the "meaning and implementation of the least burdensome requirements" and must periodically assess the implementation of such requirements, including employee training.

The FDA ombudsman responsible for device premarket review is required to conduct an audit of the least burdensome training, including the effectiveness of the training, 18 months after enactment. The audit must include "interviews of persons who are representatives of the industry regarding their experience in the device premarket review process" and a list of the measurement tools used to assess the implementation of the least burdensome requirement. A summary of the audit findings must be submitted to the Senate HELP Committee and the House Energy and Commerce Committee and posted on the FDA website within 30 days of completion of the audit.

Regarding PMA applications, the section amends FFDCA Section 515(c), adding a new paragraph that requires the HHS Secretary to "consider the least burdensome appropriate means necessary to demonstrate device safety and effectiveness." It defines the term *necessary* to mean "the minimum required information that would support a determination by the HHS Secretary that an application provides a reasonable assurance of the safety and effectiveness of the device" and states that the role of postmarket information must be considered in determining the least burdensome means of demonstrating a reasonable assurance of device safety and effectiveness.

In addition, the provision amends FFDCA Section 517A(a), adding that each substantive summary of the scientific and regulatory rationale for any decision made by FDA's Center for Devices and Radiological Health (CDRH) regarding the submission or review of a PMA, a 510(k), or an IDE must include a brief statement on how the least burdensome requirements were considered and applied.

Section 3059. Cleaning Instructions and Validation Data

FFDCA Section 510(k) requires medical device manufacturers to register with the HHS Secretary and, at least 90 days prior to introducing a device intended for human use into interstate commerce, to report to the HHS Secretary (1) the class in which the device is classified and (2) actions taken to comply with applicable device regulatory requirements under FFDCA Sections 514 and 515. This notification requirement is part of the 510(k) premarket approval pathway, a process that is unique to medical devices and, if successful, results in FDA *clearance*. Under the 510(k) pathway, the manufacturer must demonstrate that a new device is substantially equivalent to a device already on the market (a predicate device). Substantial equivalence is determined by

[128] GAO, *Medical Devices: FDA Ordered Postmarket Studies to Better Understand Safety Issues, and Many Studies Are Ongoing*, GAO-15-815, September 2015.

[129] FDA, Medical Devices, The Least Burdensome Provisions - Activities Related to Implementation, http://www.fda.gov/MedicalDevices/DeviceRegulationandGuidance/Overview/MedicalDeviceProvisionsofFDAModernizationAct/ucm136685.htm#7.

comparing the performance characteristics of a new device with those of a predicate device; clinical data demonstrating safety and effectiveness are usually not required.

Reusable medical devices are those devices that may be reprocessed and used on multiple patients. In March of 2015, FDA released final guidance on the reprocessing of reusable medical devices: *Reprocessing Medical Devices in Health Care Settings: Validation Methods and Labeling*. This guidance states that, among other things, "[m]anufacturers seeking to bring to market certain reusable devices, such as duodenoscopes, bronchoscopes and endoscopes, should submit to the FDA for review their data validating the effectiveness of their reprocessing methods and instructions."[130]

Under Section 604 of the Food and Drug Administration Safety and Innovation Act (FDASIA), the HHS Secretary was required to withdraw draft guidance, issued by FDA in July 2011, entitled "*Guidance for Industry and FDA Staff—510(k) Device Modifications: Deciding When to Submit a 510(k) for a Change to an Existing Device*," and leave the prior guidance issued in 1997 in effect. Although patient and consumer groups have generally supported a more rigorous 510(k) notification system, industry had voiced concerns that the 2011 guidance would slow the device regulatory process.[131] Section 604 of FDASIA also required a report to House and Senate committees on when a 510(k) notification should be submitted for a modification or change to a legally marketed device. Any new draft guidance (or proposed regulation) on 510(k) device modification could not be issued before the committees received the report. Final guidance (or regulation) could not be issued until one year after the committees had received the report. This report was completed by FDA in January 2014.[132]

Provision

Section 3059 amends FFDCA Section 510 by adding a new subsection (q), "Reusable Medical Devices," which requires the HHS Secretary, not later than 180 days after enactment, to identify and publish a list of reusable device types for which reports under Section 510(k) must include (1) instructions for use and (2) validation data regarding cleaning, disinfection, and sterilization. Reports issued after the publication of this list are required to include instructions for use and validation data, as specified by the HHS Secretary.

The section also requires the HHS Secretary, acting through the FDA Commissioner and not later than one year after the date on which the comment period closes for the draft guidance, to issue final guidance regarding when a notification under 510(k) would have to be submitted for a modification or change to a legally marketed device.

Section 3060. Clarifying Medical Software Regulation

Increasingly, health care facilities are using computer systems for routine administrative and financial transactions (e.g., patient scheduling, claims processing) and for capturing and exchanging clinical information (e.g., electronic health records). One area that is undergoing especially rapid growth and innovation is mobile health. This term refers to the use of portable

[130] FDA, "*Reprocessing Medical Devices in Health Care Settings: Validation Methods and Labeling*," March 15, 2015, http://www.fda.gov/downloads/MedicalDevices/DeviceRegulationandGuidance/GuidanceDocuments/UCM253010.pdf.

[131] Alexander Gafney, "In a Major Victory for Industry, FDA says Existing 510(k) Guidance to Remain 'Mostly Unchanged,'" *RAPS Regulatory Focus*, February 26, 2014, at http://www.raps.org/regulatoryDetail.aspx?id=9982.

[132] FDA, Report to Congress, *Report on FDA's Policy to be Proposed Regarding Premarket Notification Requirements for Modifications to Legally Marketed Devices*, January 7, 2014, at http://www.fda.gov/downloads/AboutFDA/CentersOffices/OfficeofMedicalProductsandTobacco/CDRH/CDRHReports/UCM387121.pdf.

devices, such as smartphones and tablets, for medical purposes. Users interface with mobile devices through the use of software applications ("apps").

Some apps simply access stored medical information, while others capture and input patient data into an electronic health record (EHR). Many apps now provide clinical decision support (CDS) using algorithms that use clinical information to generate customized (i.e., patient-specific) diagnosis and treatment recommendations.

Regulators are particularly interested in mobile apps that could pose a risk to patients if they malfunction. These include apps used to display and transfer data from a patient monitor; apps that control an existing device; and apps that transform a mobile platform into a medical device (e.g., an app that allows patients to use their smartphone to record electrocardiograms using a lead that connects to the phone).

Under the FFDCA, the FDA has regulatory authority over software that meets the statutory definition of a medical device and is "intended for use in the diagnosis of disease or other conditions, or in the cure, mitigation, treatment, or prevention of disease."[133]

FDA released a nonbinding guidance document on mobile medical apps in September 2013, in which it stated its intention to focus on the functionality of the mobile health product, not the mobile platform itself. Thus, the agency did not intend to regulate smartphone or tablet manufacturers. FDA further stated its intention to adopt a risk-based approach by applying its regulatory oversight to "only those mobile apps that are medical devices and whose functionality could pose a risk to patient safety if the mobile app were to not function as intended."[134]

In February 2015, FDA released updated guidance on its risk-based approach to regulating mobile medical apps.[135] The agency provided examples of mobile apps that do not meet the statutory definition of a medical device and so are not subject to its regulatory authority, including apps used to automate general office operations in health care settings. The agency then gave examples of mobile apps 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 apply regulatory oversight—because the apps pose minimal risk to the public. This category includes mobile apps that help asthmatics track inhaler usage and asthma episodes; apps that give patients a portal into their own EHR; and apps intended for individuals to log, track, or make decisions related to general wellness (e.g., Fitbit products).

Finally, FDA provided examples of mobile apps that are the focus of the agency's regulatory oversight. These apps meet the definition of a medical device, and they pose a significant risk to patient safety if they do not function as intended. Examples include apps that connect to an existing device for the purpose of controlling its operation, function, or energy source; apps that are used in active patient monitoring or analyzing patient-specific medical device data from a connected device; and apps that transform a mobile platform into a regulated medical device. The updated guidance did not address regulation of CDS software.

Provision

Section 3060 amends FFDCA Section 520 to exclude certain types of health software from the FFDCA definition of medical device, including products that provide a variety of administrative

[133] FFDCA Section 201(h), 21 U.S.C. §321(h).

[134] Food and Drug Administration, *Mobile Medical Applications: Guidance for Industry and Food and Drug Administration Staff*, September 25, 2013.

[135] 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.

and health management functions; electronic health record technology that creates, stores, transfers, and displays patient information; and software that interprets and analyzes patient data to help make clinical diagnosis or treatment decisions (including CDS tools). In general, this would preclude FDA from regulating these products as medical devices.

However, Section 3060 creates an exception allowing FDA to exercise regulatory authority if the agency determines that the use of the software "would be reasonably likely to have serious adverse health consequences" based on specified criteria. One of the criteria is the likelihood and severity of patient harm if the software were not to perform as intended. The exception would apply to EHR systems (and other software that simply creates, stores, transfers, and displays data), as well as CDS and other analytic tools.

Section 3060 requires the HHS Secretary to report, within two years of enactment and biennially thereafter, on the health risks and benefits associated with software determined to be excluded from the medical device definition, and a summary of the impact of such software on patient safety.

Finally, Section 3060 amends FFDCA Section 513(b) to require the HHS Secretary to classify a health software accessory based on its intended use, "notwithstanding the classification of any other device with which such accessory is intended to be used."

Subtitle G-Improving Scientific Expertise and Outreach at FDA

Section 3071. Silvio O. Conte Senior Biomedical Research and Biomedical Product Assessment Service

The Silvio O. Conte Senior Biomedical Research Service (SBRS), established in PHSA Section 228, is a special hiring mechanism used by the HHS Secretary to attract and retain accomplished scientists to work in Public Health Service (PHS) agencies. It is not subject to civil service requirements under Title 5 of the *U.S. Code*, and it is distinct from other PHS hiring mechanisms, such as the PHS Commissioned Corps. SBRS requirements are as prescribed in law and regulation (42 C.F.R. Part 24). Currently, SBRS is limited to 500 members, who are accomplished doctoral-level scientists in biomedical research or clinical research evaluation. The rate of pay may not exceed that for Level I of the Executive Schedule (currently about $206,000 per year) unless approved by the President. The HHS Secretary may contribute up to 10% of a Servicemember's pay to that person's already established retirement system at the institution of higher education at which the member had been employed.

Provision

Section 3071 renames the SBRS as the Silvio O. Conte Senior Biomedical Research and Biomedical Product Assessment Service (the Service). It increases the number of authorized members to 2,000 and adds "biomedical product assessment" as a desired field of expertise. It clarifies that the HHS Secretary is not required to reduce the number of employees serving in other HHS employment systems to offset the number of new employees in the Service.

The provision requires the HHS Secretary to appoint experts to agencies within HHS, "taking into account the need for the expertise of such expert." It also authorizes the appointment of persons who hold "a master's level degree in engineering, bioinformatics, or a related or emerging field," broadening the current requirement for doctoral-level members. It increases the upper pay rate limit to that of the President (currently $400,000 per year) but eliminates the authority to contribute to a member's preexisting retirement system. Finally, the provision requires GAO,

within four years of enactment, to study and report to Congress on the changes to the Service and their effects on HHS departments and agencies.

Section 3072. Hiring Authority for Scientific, Technical, and Professional Personnel

Title 5 of the *U.S. 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.[136] For example, FFDCA Section 714 authorizes the HHS 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

Section 3072 adds a new FFDCA Section 714A, "Hiring Authority for Scientific, Technical, and Professional Personnel," which authorizes the HHS Secretary to "appoint outstanding and qualified candidates to scientific, technical, or professional positions that support the development, review, and regulation of medical products" within the competitive service "without regard to the provisions of title 5, United States Code, governing appointments in the competitive service." The FDA Commissioner is 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. It specifies that this information will be publicly available and that this new provision does not affect the FDA's streamlined hiring authority in FFDCA 714. The provision also requires the HHS Secretary, not later than 18 months after the enactment of the 21st Century Cures Act, to submit a report to Congress on workforce planning and certain specified elements with regard to the FDA workforce. This provision also requires the Comptroller General to conduct a study of FDA's ability "to hire, train, and retain qualified scientific, technical, and professional staff ... necessary to fulfill the mission of the [FDA] to protect and promote public health," among other specified contents with regard to the FDA workforce.

Section 3073. Establishment of Food and Drug Administration Intercenter Institutes

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. CDER reviews new drug applications for approval, CBER reviews biologics license applications for licensure, and CDRH reviews premarket approval applications for approval and 510(k) notifications for clearance.

As part of the Vice President's Cancer Moonshot Initiative, the Obama Administration has proposed an Oncology Center of Excellence to streamline collaboration across FDA's Human Drugs, Biologics, and Devices and Radiological Health programs. According to the FDA's FY2017 Congressional Justification, "With the continued development of companion diagnostic

[136] CRS Report R40604, *Hiring and Pay Authorities for Federal Scientific and Technical (S&T) Personnel*, by Deborah D. Stine and Clinton T. Brass.

tests and the use of combinations of drugs and biologics to treat cancer using methods developed through the science of precision medicine, to most benefit those affected, FDA needs to take an integrated approach in its evaluation of products for the prevention, screening, diagnosis, and treatment of cancer."[137] Although the Administration's proposed center of excellence is specific to cancer, there has arguably been an increase in the number and complexity of diagnostics and therapeutics for other diseases as well, and some groups have suggested that such pilots could be done in other areas (e.g., cardiology, neurology, and infectious disease).[138]

Provision

Section 3073 adds a new FFDCA Section 1014, "Food and Drug Administration Intercenter Institutes," requiring the HHS Secretary to establish one or more "Intercenter Institutes" for a major disease area(s). Such institutes will be responsible for coordinating activities applicable to specific disease area(s) between CDER, CBER, and CDRH; for example, coordinating staff from the three centers with diverse product expertise relevant to a major disease area, and streamlining the review of medical products related to that major disease area. This provision requires the HHS Secretary to establish at least one institute within one year of enactment, and to provide a public comment period while each institute is being implemented. In addition, this provision allows the HHS Secretary to terminate any such institute if the HHS Secretary determines that it is no longer benefitting the public health.

Section 3074. Scientific Engagement

Following allegations of misspent funds during a 2010 General Services Administration meeting held in Las Vegas, the Office of Management and Budget (OMB) imposed restrictions on conference travel for federal employees in memorandum M-12-12.[139] 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 exceed $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."[140]

Provision

Under Section 3074, if attendance at a scientific meeting is directly related to the professional duties of scientific or medical professionals of HHS, then the meetings would not be considered to be conferences for the purposes of (1) federal reporting requirements in annual appropriations acts, and (2) a restriction in OMB memorandum M-12-12 or any other regulation restricting such travel, but would not exempt these meeting from federal travel regulations. The provision also requires that each HHS operating division, not later than 90 days after the end of the fiscal year,

[137] FY2017 Justification of Estimates for Appropriations Committees, FDA, p. 12.

[138] M. McCaughan and K Rawson, "FDA 'Intercenter Institute' Legislation Headed for Senate Mark-Up," FDA Pink Sheet, vol. 78, no. 13, March 28, 2016.

[139] OMB, Promoting Efficient Spending to Support Agency Operations, May 11, 2012, http://www.whitehouse.gov/sites/default/files/omb/memoranda/2012/m-12-12.pdf.

[140] Ibid.

post on its website an annual report on scientific meeting attendance and related travel spending for each fiscal year, including details as specified.

Section 3075. Drug Surveillance

The Food and Drug Administration Amendments Act of 2007 (FDAAA, P.L. 110-85) required FDA to take several actions regarding how it informs the public, expert committees, and others about agency actions and plans and information the agency has developed or gathered about drug safety and effectiveness. Among other things, the law required biweekly screening of the FDA Adverse Event Reporting System (FAERS) database and quarterly reporting on the FAERS website regarding new safety information or potential signals of a serious risk.[141] The FDAAA also required the development and maintenance of a website with extensive drug safety information, and required the HHS Secretary to "prepare, by 18 months after approval of a drug or after use of the drug by 10,000 individuals, whichever is later, a summary analysis of the adverse drug reaction reports received for the drug, including identification of any new risks not previously identified, potential new risks, or known risks reported in an unusual number."[142]

The FDAAA also named the risk-management process "risk evaluation and mitigation strategies" (REMS) and expanded the risk-management authority of FDA.[143] A REMS may include "an elements to assure safe use" (ETASU), which is a restriction on distribution or use that is intended to (1) allow access to those who could benefit from the drug while minimizing their risk of adverse events and (2) block access to those for whom the potential harm would outweigh potential benefit.[144]

Provision

Section 3075 amends FFDCA Section 505(k)(5) to require the HHS Secretary to conduct regular screenings of the FAERS database instead of the bi-weekly screenings required by current law. This provision requires the HHS Secretary to post guidelines on the FDA website, with input from experts, that detail best practices for drug safety surveillance using FAERS and criteria for public posting of adverse event signals. This provision also amends FFDCA Section 505(r)(2)(D) to remove the requirement that the HHS Secretary prepare a summary analysis of the adverse drug reaction reports received for a drug "by 18 months after approval" and instead requires that the HHS Secretary make publicly available on the FDA website "best practices for drug safety surveillance activities for drugs newly approved under this section or section 351 of the [PHSA]."

This provision also amends FFDCA Section 505-1(f)(5)(A), expanding the authority to evaluate the ETASU for a drug to include "or other advisory committee," compared with current law, which designates this responsibility to the HHS Secretary "through the Drug Safety and Risk Management Advisory Committee (or successor committee)" of the FDA. This provision also amends FFDCA Section 505-1(f)(5)(B) to change the requirement that the committee evaluate the ETASU for one or more drugs from "annually" to "periodically."

[141] FFDCA §505(k)(5).

[142] FFDCA §505(r)(2)(D).

[143] The REMS authority is in FFDCA §505-1 [21 U.S.C. §355-1]. REMS are discussed in CRS Report RL34465, *FDA Amendments Act of 2007 (P.L. 110-85)*, by Susan Thaul.

[144] CRS Report R41983, *How FDA Approves Drugs and Regulates Their Safety and Effectiveness*, by Susan Thaul.

Section 3076. Reagan-Udall Foundation for the Food and Drug Administration

Background

FFDCA Section 770, as added by 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, as well as 14 members who are appointed to the Board by the ex officio members, including 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." Section 770 specifies that of the 14 appointed members, four must be representatives of the "general pharmaceutical, device, food, cosmetic, and biotechnology industries;" three must be representatives of academic research organizations; two must be representatives of patient or consumer advocacy organizations; one must be a representative of health care providers; and four must be "at-large members with expertise or experience relevant to the purpose of the Foundation."

Provision

Section 3077 amends FFDCA Section 770 to 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 limits 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 is broadened to rest with all members of the board, not only ex officio appointees. As with the current law, each board member's term of office would last for four years, and initially appointed board members' terms would expire on a staggered basis, as determined by the ex officio members. This provision adds that for the additional board members appointed pursuant to the 21st Century Cures Act, the terms of office for the initially appointed persons may expire on a staggered basis, as determined by the members of the board.

The provision removes the salary cap of the foundation's Executive Director, which is now set at the compensation of the Commissioner. It also amends 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 changes the requirement, so that funds received from the Treasury are "managed as individual programmatic funds, according to best accounting practices."

Subtitle H-Medical Countermeasures Innovation

Following the terrorist attacks of 2001, the federal government determined that it needed additional medical countermeasures (such as diagnostic tests, drugs, vaccines, and other treatments) to respond to an attack using chemical, biological, radiological, or nuclear (CBRN) agents. The Project BioShield Act (P.L. 108-276), the Pandemic and All-Hazards Preparedness Act (PAHPA, P.L. 109-417), and the Pandemic and All-Hazards Preparedness Reauthorization Act of 2013 (PAHPRA, P.L. 113-5) established new authorities and programs in the Department

of Health and Human Services (HHS) to support the development and procurement of new CBRN medical countermeasures.

The Health and Human Services Assistant Secretary for Preparedness and Response (ASPR) coordinates the government-wide effort to develop and procure medical countermeasures. The ASPR is required to provide Congress an annual coordinated 5-year budget plan that includes countermeasure activities outside the ASPR's office such as basic research at National Institutes of Health and stockpiling in the Strategic National Stockpile at the Centers for Disease Control and Prevention. As part of the ASPR Office, the Biomedical Advanced Research and Development Authority (BARDA) supports advanced research and development of CBRN countermeasures through contracts and public-private partnerships. The BARDA also implements Project BioShield, a special process and funding mechanism that allows for the use of specifically appropriated funds to procure countermeasures that still need up to 10 more years of development.

The Cures Act places additional requirements on and provides additional authorities for ASPR and BARDA. It also modifies the process for Project BioShield procurements.

Sections 3081-3085. Medical Countermeasures Innovation

Provisions

Section 3081 requires the HHS Secretary to provide "timely and accurate recommended utilization" guidelines for medical countermeasures in the Strategic National Stockpile. Additionally, it amends the requirement for the HHS Secretary to report to Congress when the amount available for Project BioShield procurements falls below $1.5 billion. This section specifies the recipients of the report as the Senate Committee on Health, Education, Labor, and Pensions, the Senate Committee on Appropriations, the House Committee on Energy and Commerce, and the House Committee on Appropriations. This report is now required by "March 1 of each year in which" the amount available drops below $1.5 billion rather than the previously required deadline of within 30 days of its occurrence.

Section 3082 moves contracting authority for Project BioShield and BARDA advanced research and development from the ASPR to the BARDA Director. This move was recommended by the Blue Ribbon Panel on Biodefense to "reduce unnecessary bureaucratic delays, improve efficiency and decision making, and enhance BARDA program effectiveness and accountability."

Section 3083 requires ASPR to provide additional information in its annual "coordinated 5-year budget plan" and requires that it be made publicly available in "a manner that does not compromise national security." This section also adds the requirement that the budget plan also consider the development of countermeasures and products for emerging infectious diseases that may present "a threat to the nation."

Section 3084 allows the HHS Secretary to partner with "an independent, non-profit entity" to "foster and accelerate the development of medical countermeasures; ... promote the development of new and promising [countermeasure] technologies; ... [and] address unmet public health needs ... such as novel antimicrobials for multidrug resistant organisms and multiuse platform technologies for diagnostics, prophylaxis, vaccines, and therapeutics." This partner may provide business advice and use venture capital practices to invest in companies developing medical countermeasures. The U.S. intelligence community has successfully used a similar strategic investor model to address its unmet technology needs through In-Q-Tel. This section establishes certain criteria for the partner, including prior experience in technology innovation and successful partnering with the federal government. The HHS Secretary acting through the BARDA Director

is to provide the entity with the government needs and requirements and a description of the work to be done under the agreement. The entity is required to provide regular reports on the spending of funds provided by HHS and on progress meeting the identified needs. The Comptroller General is to evaluate this partnership no later than four years after enactment. This authority sunsets on September 30, 2022.

Section 3085 removes the requirement that the President approve each specific use of Project BioShield appropriations. The Blue Ribbon Study Panel on Biodefense recommended this change to streamline the Project BioShield contracting process. This section also specifies the congressional committees that HHS must notify following a decision to use Project BioShield funds as the Senate Committee on Health, Education, Labor, and Pensions, the House Committee on Energy and Commerce, and the Appropriation Committee in each chamber.

Section 3086. Encouraging Treatment for Agents that Present a National Security Threat

Under the Prescription Drug User Fee Act of 1992 (PDUFA), FDA agreed to specific goals for improving the drug review time and created a two-tiered system of review times: Standard Review and Priority Review. Compared with the amount of time standard review generally takes (approximately 10 months), a Priority Review designation means FDA's goal is to take action on an application within 6 months.[145] An application for a drug may receive priority review designation if it is for a drug that treats a serious condition and, if approved, would provide a significant improvement in safety or effectiveness, or if it is the subject of a priority review voucher. Currently, FDA has two authorized priority review voucher programs (the rare pediatric disease priority review program and the tropical disease priority review program), funded by user fees, which provide a transferable voucher, under specified conditions, to a sponsor of an approved new drug or biological product to be used for the priority review of another application. The purpose of the priority review drug voucher programs is to incentivize development of new treatment for diseases that may otherwise not attract development interest from companies due to either cost or lack of market opportunities.

Provision

Section 3086 adds new FFDCA Section 565A, "Priority Review to Encourage Treatments for Agents that Present National Security Threats," establishing a new priority review voucher program, funded by user fees, to provide a transferable voucher, under specified conditions, to a sponsor of an approved new human drug product application for a material threat medical countermeasure to be used for the priority review of another application. This section defines a "material threat medical countermeasure application" as, among other things, a human drug application "to prevent, or treat harm from a biological, chemical, radiological, or nuclear agent identified as a material threat" under the Public Health Service Act,[146] or "to mitigate, prevent, or treat harm from a condition that may result in adverse health consequences or death and may be caused by administering a drug, or biological product against such agent." The HHS Secretary's authority to award such voucher is set to sunset on October 1, 2023.

[145] FDA, Priority Review, http://www.fda.gov/ForPatients/Approvals/Fast/ucm405405.htm.

[146] PHSA §319F-2(c)(2)(A)(ii).

Section 3087. Paperwork Reduction Act Waiver During a Public Health Emergency

PHSA Section 319 authorizes the HHS Secretary to determine the existence of a public health emergency, which in turn authorizes certain further actions to enhance response flexibility, such as waivers of requirements for grant-making and hiring.[147] The Paperwork Reduction Act (PRA) ensures that federal agencies do not overburden the public with federally sponsored data collections. Among other things, the PRA requires review and preclearance of federal data collection proposals by OMB.[148] Such preclearance and other requirements could slow the collection of information needed to prepare for and respond to public health emergencies.

Provision

Section 3087 would add a provision to PHSA Section 319 to waive requirements for voluntary data collection under the Paperwork Reduction Act (PRA) if the HHS Secretary determines (1) that the conditions of a public health emergency under PHSA Section 319 are met or there is a significant likelihood that such conditions will arise, and (2) that applicable preparedness and response activities would necessitate a waiver of PRA requirements. A waiver becomes effective when the HHS Secretary posts a notice of such waiver on the HHS website. Its termination must be similarly posted. The duration of a waiver is a matter of Secretarial discretion in order to facilitate reasonable preparedness, response, and post-response activities.

Section 3088. Clarifying FDA Emergency Use Authorization

Under normal circumstances, drugs, devices, and biologics may be introduced into interstate commerce only if they have been approved, cleared, or licensed, respectively, by the FDA. But if the Secretary of HHS declares, pursuant to FFDCA Section 564, that an emergency exists due to a specified biological, chemical, radiological, or nuclear agent (which may include a naturally occurring disease outbreak), the HHS Secretary may temporarily authorize the use of unapproved products, or unapproved uses of approved products, for response to the emergency. This authorization is referred to as an Emergency Use Authorization (EUA).[149] This authority did not previously extend to drugs approved for use in animals.

Provision

Section 3088 provides a set of amendments that make EUA provisions applicable to animal drugs by referencing FFDCA Sections 504 (regarding animal drugs used in feeds), 512 (regarding requirements for approval of new animal drugs), and 517 (regarding animal drugs for minor animal species or minor uses).

[147] For more information, see HHS, "Public Health Emergency Declaration," http://www.phe.gov/Preparedness/legal/Pages/phedeclaration.aspx.

[148] For more information, see HHS, "Frequently Asked Questions about PRA / Information Collection," http://www.hhs.gov/ocio/policy/collection/infocollectfaq.html.

[149] For more information, see FDA, "Emergency Use Authorization," http://www.fda.gov/EmergencyPreparedness/Counterterrorism/MedicalCountermeasures/MCMLegalRegulatoryandPolicyFramework/ucm182568.htm.

Subtitle I-Vaccine Access, Certainty, and Innovation

A vaccine may be both a commercial product and a public good, and Congress has established several federal payment mechanisms, health insurance coverage requirements, and other incentives to support the production and use of vaccines in the United States. Some of these incentives are tied to recommendations of CDC and/or its Advisory Committee on Immunization Practices (ACIP). The ACIP is a group of medical and public health experts that develop recommendations on use of vaccines in the civilian U.S. population.[150] In contrast to FDA, which licenses vaccines when they are shown to be safe and effective for individuals, ACIP and CDC also consider epidemiology and vaccine availability, and may recommend routine use of a vaccine for only a subset of the population for whom FDA has licensed its use. Vaccine manufacturers have an interest in understanding the factors considered by ACIP and CDC, as well as FDA, in making vaccine use and licensing decisions.

The ACIP is not explicitly authorized in the PHSA or elsewhere in federal law. Its authority is based in general authority of the HHS Secretary to establish advisory committees.[151] However, the ACIP has been given explicit statutory roles under the PHSA and the Social Security Act (SSA). The ACIP's actions pursuant to these roles affect reimbursement for immunizations, and thereby 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 cost-sharing, immunizations recommended by the ACIP.[152] 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.[153]
- SSA Section 1928 establishes the Vaccines for Children (VFC) program, which provides federally purchased vaccines free of charge to eligible children.[154] VFC vaccines are those for which the ACIP has issued a recommendation for use in children.

In 1986, in order to stabilize the pediatric vaccine market, Congress waived the liability of manufacturers (in most cases) and established the National Vaccine Injury Compensation Program (VICP) to compensate persons injured by certain vaccines.[155] Initially the list of covered vaccine types and associated compensable injuries and time frames (called the "Injury Table") was provided in law.[156] Prior to the enactment of the Cures Act, the HHS Secretary could, through rulemaking, create or modify compensable injuries and time frames for vaccines on the Injury Table, but could not add additional vaccine types. An exception existed for new vaccines that were recommended by CDC for routine use in children, which were automatically included in the Injury Table. In 2013, the Advisory Commission on Childhood Vaccines (ACCV), which advises

[150] Advisory Committee on Immunization Practices (ACIP), http://www.cdc.gov/vaccines/acip/index.html.

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

[152] 42 U.S.C. §300gg-13(a)(2). Regulations are at 45 C.F.R. §147.130.

[153] 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.

[154] 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.

[155] Health Resources and Services Administration (HRSA), "National Vaccine Injury Compensation Program," http://www.hrsa.gov/vaccinecompensation/.

[156] PHSA Title XXI, Subtitle 2.

on the VICP, informed the HHS Secretary that the VICP authority could discourage the growing use of vaccines for pregnant women, as the law did not allow for addition of such vaccines to the Injury Table unless they were also recommended for routine use in children, and did not clearly cover injury to an infant born to a woman who was vaccinated during pregnancy.[157]

Sections 3091-3093. Predictable Review Timelines of Vaccines by the ACIP, Review of Processes and Consistency of ACIP Recommendations, Encouraging Vaccine Innovation

Provisions

Section 3091 requires the 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. If ACIP does not issue recommendations regarding such vaccine at such meeting, it must provide an update on the status of its review. The section also requires the ACIP to make recommendation in a timely manner regarding (1) a vaccine designated by FDA as a breakthrough therapy to treat a serious or life-threatening disease or condition (pursuant to FFDCA Section 506), or (2) a vaccine that could be used in a public health emergency.

Section 3092 requires the CDC Director to review, as specified, ACIP processes 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.

Section 3093 requires the CDC Director to ensure that the agency's infectious disease centers and divisions coordinate their immunization program and policy efforts, including through consultation with stakeholders. The section also requires the HHS Secretary, within one year of enactment, to publish and provide to Congress a report on ways to promote innovation in the development of vaccines against infectious diseases, including the processes to determine priority needs, and on obstacles (and proposed remedies) to vaccine innovation. The HHS Secretary may consult with specified stakeholders, including vaccine developers, in producing this report.

Section 3093 also amends PHSA Sections 2111 and 2114 (which authorize the VICP petition process and vaccine injury table) to require the HHS Secretary to incorporate into the table of covered vaccines any vaccine recommended by CDC for routine use in pregnant women. It clarifies that both the woman and a child or children in utero when the vaccine was administered are eligible for compensation.

Title IV- Delivery

Sections 4001 through 4008 of Title IV address the federal policies to promote the adoption and use of EHR technology. They are based on the provisions in S. 2511, the Improving Health Information Technology Act, which was reported by the Senate HELP Committee on April 5, 2016. These eight sections are discussed below.

The Health Information Technology for Economic and Clinical Health (HITECH) Act of 2009 authorized Medicare and Medicaid incentive payments to acute-care hospitals and physicians

[157] See letters to the HHS Secretary from the ACCV regarding compensability of in utero injuries from vaccines, HRSA, "Reports and Recommendations," 2013, http://www.hrsa.gov/advisorycommittees/childhoodvaccines/reportsrecommendations.html.

who attest to being meaningful users of certified electronic health record (EHR) technology.[158] The law instructed the HHS Secretary to make the measures of "meaningful use" more stringent over time, which CMS has done in stages.

Stage 1 of meaningful use requires eligible hospitals and physicians to use EHR technology to meet a series of meaningful use objectives that generally involve capturing and storing structured patient data (e.g., vital signs, medications, lab test results). Providers must use EHR technology that has been tested and certified as having the capability to perform these functions. Testing and certification entities are authorized by the HHS Office of the National Coordinator for Health Information Technology (ONC).

Stage 2 of meaningful use requires eligible hospitals and physicians to use their EHR technology to perform more advanced functions, such as giving patients access to their electronic health information and exchanging patient data during transitions of care (e.g., a hospital discharge to a rehabilitation facility, or a physician referral).

Beginning in 2015, hospitals and physicians that are not meaningful EHR users are subject to a Medicare payment adjustment (i.e., penalty) unless they qualify for a hardship exception.

CMS published a final rule in October 2015 modifying the meaningful use Stage 2 objectives and establishing the objectives for Stage 3, which hospitals and physicians must meet by 2018.[159] The agency made significant changes to the meaningful use program in response to the concerns of health care providers about the challenges and burdens they face in making EHR technology work. For example, CMS eliminated several clinical documentation objectives, and instead focused on a few objectives that capture more advanced uses of the technology (e.g., CDS, health information exchange).

CMS also published an accompanying final rule (the 2015 Edition final rule) that expands the certification program.[160] In addition to certifying the next generation of EHR technology that hospitals and physicians need to achieve meaningful use Stage 3, the program will be able to certify health information technology (HIT) products with a different combination of capabilities and functionalities that meet the needs of other types of health care providers and settings that are not eligible to participate in the EHR incentive program.

The 2015 Edition final rule for the certification program established new transparency requirements for HIT developers. It also seeks to improve interoperability, for example, by requiring certified HIT products to adopt new and updated vocabulary and content standards for structured health information, including a common clinical data set composed of standardized data elements, and by improving the testing of the ability of HIT systems to transmit, receive, and use standardized clinical documents.

ONC released a national interoperability roadmap in October 2015—developed over an 18-month period with input from numerous stakeholders—to coordinate efforts around achieving HIT interoperability.[161] The roadmap establishes interoperability goals for the next 10 years, with 2017

[158] P.L. 111-5, Division B, Title IV; 123 Stat. 467.

[159] Centers for Medicare & Medicaid Services, "Medicare and Medicaid Programs; Electronic Health Record Incentive Program - Stage 3 and Modifications to Meaningful Use in 2015 through 2017; Final Rule," 80 *Federal Register* 62761, October 16, 2015.

[160] Office of the National Coordinator for Health Information Technology, "2015 Edition Health Information Technology (Health IT) Certification Criteria, 2015 Edition Base Electronic Health Record (EHR) Definition, and ONC Health IT Certification Program Modifications; Final Rule," 80 *Federal Register* 62601, October 16, 2015.

[161] Office of the National Coordinator for Health Information Technology, *Connecting Health and Care for the Nation:*

set as the deadline for individuals and health care providers along the care continuum to be able to send, receive, find, and use core clinical data. ONC expects the roadmap to evolve in partnership with the public and private sectors as technology and policy dictate.

The roadmap discusses the payment and regulatory drivers for promoting interoperability, as well as the central policy and technical components of a fully interoperable nationwide health information infrastructure. A key challenge is overcoming legal and governance barriers to trusted information exchange by getting stakeholders to agree to and follow a common set of standards, services, policies, and practices that facilitate exchange and use of electronic health information without limiting competition.

Medicare Access and CHIP Reauthorization Act of 2015 (MACRA)[162]

MACRA declared it a national objective to achieve widespread interoperability of certified EHR technology by the end of 2018. The law defines interoperability as the ability of health information systems to not only exchange clinical information but to also use the information based on common standards in order to improve care and patient outcomes.

In addition, MACRA instructed the HHS Secretary, within one year of enactment, to submit a report to Congress on ways to help health care providers compare and select certified EHR technology, such as through surveying EHR users and vendors and making such information publicly available.

Finally, MACRA required the HHS Secretary, in consultation with stakeholders, to establish interoperability metrics to measure progress toward achieving the national objective of widespread interoperability of certified EHR technology by July 1, 2016. If that objective is not met by December 31, 2018, the HHS Secretary will have until December 31, 2019, to submit a report to Congress identifying the barriers to widespread interoperability and providing recommendations for achieving it.

Information Blocking

ONC released a report to Congress on health information blocking in April 2015.[163] The report defined information blocking as knowingly and unreasonably interfering with the exchange or use of electronic health information, and examined the nature and extent of the practice based on available evidence. It also detailed the actions that ONC is taking, in coordination with other federal agencies, to address information blocking. Finally, the report identified gaps in authority that limit the ability of ONC and other federal agencies to effectively target, deter, and remedy such conduct.

MACRA requires eligible hospitals and physicians, beginning April 2016, to indicate through meaningful use attestation (or some other process specified by the HHS Secretary) that they have not knowingly and willfully taken any action to limit or restrict the interoperability of their certified EHR technology.

Patient Access

A Shared Nationwide Interoperability Roadmap, Final Version 1.0, October 2015, https://www.healthit.gov/sites/default/files/hie-interoperability/nationwide-interoperability-roadmap-final-version-1.0.pdf.

[162] P.L. 114-10, §106(b), 129 Stat. 138.

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

The Health Insurance Portability and Accountability Act (HIPAA) Privacy Rule gives individuals the right of access to inspect, obtain a copy of, and transmit to a third party a copy of their health information.[164]

One of the meaningful use objectives that must be met by hospitals and physicians using certified EHR technology is to provide individuals with the ability to view, download, and transmit (VDT) their electronic health information. As part of meeting that objective, the 2015 Edition final rule for the certification program requires EHR developers to publish application programming interfaces (APIs); that is, programming instructions to enable other software application developers to produce apps giving individuals access to their clinical data.

Patient Matching

ONC released a report on patient identification and matching (i.e., linking patient records with the correct individual) in February 2014. It recommended standardizing patient attributes for the purpose of information exchange, coordinating activities among organizations, and introducing EHR certification criteria for capturing patient identification standards.

Patient matching was addressed in the 2015 Edition final rule for the HIT certification program. Certified EHR systems must be able to create a summary-of-care document that includes the following standardized patient data: first name; last name; previous name; middle name (including middle initial); suffix; date of birth (year, month, and day are required fields; hours and minutes are optional); address; phone numbers (home, business, cell); and sex.

Sections 4001-4008. Policies to Promote the Adoption and Use of EHR Technology

Provisions

Section 4001 ("Assisting Doctors and Hospitals in Improving Quality of Care for Patients") requires the HHS Secretary to develop, within one year of enactment, a strategy and recommendations for reducing the regulatory and administrative burdens of using EHR technology. In addition, it eases EHR documentation requirements by allowing physicians, as consistent with state law, to delegate electronic medical record documentation to non-physicians, provided certain criteria are met. It requires ONC to encourage the voluntary certification of HIT for use in medical specialties and sites of service, and to adopt certification criteria for HIT used by pediatricians. It also requires the HHS Secretary to submit to the HIT Advisory Committee of the ONC, within six months of enactment, a report on meaningful use statistics, as specified.

Section 4002 ("Transparent Reporting on Usability, Security, and Functionality") amends PHSA Section 3001(c)(5) to require the HHS Secretary, within one year of enactment, to issue a rule that requires HIT developers, as a condition of certification, (1) to provide assurances that they will not engage in information blocking; (2) not to prohibit or restrict communication regarding the usability, security, or functionality of their HIT product; and (3) publish application programming interfaces, among other things. Section 4002 also establishes Medicare EHR payment adjustment hardship exceptions for hospitals and physicians whose EHR technology has been decertified, and for physicians eligible for merit-based incentive payments (MIPS). Finally, this section adds a new PHSA Section 3009A, which establishes an EHR reporting program to help providers choose EHR products. It instructs the HHS Secretary to convene stakeholders to develop reporting criteria that reflect EHR product usability, interoperability, and security, and requires

[164] 45 C.F.R. §164.524.

EHR developers—as a condition of maintaining certification—to submit reports based on those criteria for each of their certified products. This section authorizes to be appropriated $15 million in total for the purposes of carrying out the information blocking and Medicare EHR payment provisions.

Section 4003 ("Interoperability") amends PHSA Section 3000(c) to require ONC, in collaboration with other federal entities, to convene stakeholders to develop and publish on its website a trusted exchange framework and a common agreement among existing health information networks to exchange electronic health information, as steps in achieving an interoperable nationwide health information network. It also requires the HHS Secretary to establish a digital contact directory for health care professionals, practices, and facilities. Finally, Section 4003 eliminates the existing HIT Policy Committee and HIT Standards Committee and adds a new PHSA Section 3002 to replace them with a single new committee—the HIT Advisory Committee—which assumes their responsibilities and duties, and is required to produce annual progress reports on advancing interoperability nationwide, as specified.

Section 4004 ("Information Blocking") defines the practice of information blocking; directs the HHS Secretary to identify via rulemaking reasonable and necessary activities that do not constitute information blocking; and authorizes the HHS Office of Inspector General (OIG) to investigate and penalize information-blocking practices by HIT developers, health information exchanges and networks, and health care providers. It establishes civil monetary penalties for developers, exchanges, and networks that engage in information blocking, and requires the OIG to refer to the appropriate agency health care providers who engage in information blocking to be subject to appropriate disincentives under federal law. OIG is authorized to refer instances of information blocking to the HHS Office for Civil Rights (OCR) if a HIPAA privacy consultation would resolve the matter. Section 4004 also requires ONC, in consultation with OCR, to issue guidance on common legal, governance, and security barriers that prevent the trusted exchange of electronic health information. It authorizes ONC to share information about information blocking investigations with the Federal Trade Commission. Finally, it requires ONC to implement a process for the public to report instances of information blocking or problems with interoperability.

Section 4005 ("Leveraging Electronic Health Records to Improve Patient Care") requires certified HIT to be able to transmit data to and receive data from clinical data registries, as defined. It also extends federal privilege and confidentiality protections to HIT developers who report and analyze patient safety information related to HIT use.

Section 4006 ("Empowering Patients and Improving Patient Access to Their Electronic Health Records") amends PHSA Section 3009 to facilitate patient access to their electronic health information by requiring the HHS Secretary to encourage partnerships between health information networks, health care providers, and other stakeholders to offer access through secure, user-friendly software. This section also requires the HHS Secretary, in coordination with OCR, to educate providers on using exchanges to provide patient access, and to issue guidance to exchanges on best practices for providing patient access. It requires ONC and OCR to develop policies that support dynamic technology solutions for promoting patient access, and to help educate individuals and providers on patients' rights under HIPAA. Finally, ONC may require that HIT standards and certification support patients' access to their electronic health information.

Section 4007 ("GAO Study on Patient Matching") requires that GAO conduct a study, within one year of enactment, to review the policies and activities of ONC and other relevant stakeholders, and to make recommendations regarding patient matching, the effectiveness of such efforts, and performance related to additional factors, such as

privacy and security of patient information. GAO must report its findings to Congress within two years of enactment.

Section 4008 ("GAO Study on Patient Access to Health Information") requires GAO to study patients' access to their own health information, including barriers to access (such as fees and formats), complications that health care providers experience when providing access, and methods patients may use for requesting their personal health information. GAO must report its findings to Congress within 18 months of enactment.

Section 4009. Improving Medicare Local Coverage Determinations

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 post-payment 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. 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 HHS Secretary has discretion to determine what specific items and services will be covered and under what conditions.

The HHS Secretary has authority to make Medicare coverage policy decisions both nationally and locally. LCDs are MAC decisions on whether, and under what circumstances, to cover a particular item or service on a contractor-wide basis. 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.

CMS's Medicare Program Integrity Manual (Chapter 13, Local Coverage Determinations) instructs MACs on LCD development. The process includes several mechanisms for local stakeholder input, including notice and comment 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 the provider community in the MAC's jurisdiction. MACs are responsible for ensuring that LCDs are consistent with all statutes, rulings, regulations, and national coverage decisions.

Provision

Section 4009 requires the HHS 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 proposed 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.

Section 4009 is effective for LCDs proposed or revised 180 days after the enactment date.

Section 4010. Medicare Pharmaceutical and Technology Ombudsman

Prior to the passage of the Cures Act, the HHS Secretary was 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.

Medicare law requires the HHS Secretary to conduct a satisfaction survey at least every five years of beneficiaries, as well as providers and suppliers who submitted appeals (SSA Section 1869(e)) and to submit a report to Congress on the results of the survey.

In addition, SSA Section 1808(c) requires the HHS Secretary to appoint a Medicare Beneficiary Ombudsman. The Medicare Beneficiary Ombudsman was created to identify and address systemic issues that affect Medicare beneficiaries, but the Medicare Beneficiary Ombudsman did 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 may "identify issues and problems in payment or coverage policies."

Provision

Section 4010 requires the HHS Secretary to provide within 12 months of enactment a pharmacy and technology ombudsman within CMS. The pharmacy and technology ombudsman is required to 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 is subject to the same prohibition on advocacy and authority to identify issues as the Medicare Beneficiary Ombudsman.

Section 4011. Medicare Site-of-Service Price Transparency

Some Medicare-covered items and services can be provided either in a physician's office, in a hospital outpatient department, or in a freestanding or hospital-operated ambulatory surgical center (ASC); 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 (including 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 with those of free-standing physician offices.

Provision

Section 4011 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 2018 and in each year thereafter, the HHS Secretary will make information available to the public via a searchable website on (1) the estimated Medicare payment amounts for the items and services provided under both the hospital OPPS fee schedule and the ASC payment system, and (2) the estimated amount of beneficiary liability for each 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 HHS Secretary would include notice of the availability of such information in the annual explanation of Medicare benefits sent to all beneficiaries. The HHS 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 HHS Secretary would transfer $6 million from the Supplemental Medical Insurance Trust Fund to the CMS Program Management Account for FY2017; these funds would remain available until expended.

Section 4012. Telehealth Services in Medicare

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.

Medicare Part A does not cover services furnished through telehealth. Medicare Part B does cover "telehealth services," which are defined under Social Security Act Section 1834(m)(4)(F) as a set of service codes corresponding to various primary care and psychiatric visits furnished by physicians and other practitioners. With some exceptions, Part B telehealth services must be provided through live videoconferencing.

Under Medicare Part B telehealth, the facility where the beneficiary is located is referred to as the "originating site," and the site where the practitioner is located is referred to as the "distant site." CMS makes a payment to the physician or other practitioner at the distant site for rendering the telehealth service, and it pays a separate facility fee to the originating site. Under Social Security Act Section 1834(m)(4)(C), only certain categories of providers and suppliers may serve as telehealth originating sites. Further, within those categories, only providers or suppliers that are located in a documented health professional shortage area or in a county that is not included in a Metropolitan Statistical Area, or are participating in federal telemedicine demonstration projects, are eligible to be originating sites.

Provision

Section 4012 requires CMS and the Medicare Payment Advisory Commission (MedPAC) to conduct evaluations and submit information to Congress concerning telehealth.

CMS is required, no later than one year after the date of enactment of the provision, to provide information on (1) subpopulations of Medicare beneficiaries whose care would be most improved by the expansion of telehealth services; (2) activities by the CMS Center for Medicare and Medicaid Innovation (CMMI) that examine the use of telehealth; (3) the types of high-volume Medicare services that might be suitable for telehealth reimbursement; and (4) barriers that might prevent the expansion of telehealth services under Part B.

MedPAC is required, no later than March 15, 2018, to provide information identifying (1) the telehealth services that are reimbursable under Medicare Parts A and B under current law; (2) telehealth services that currently are reimbursable by private health insurance plans; and (3) potential ways to incorporate into Medicare Parts A and B telehealth services that are not paid for under those programs but are paid for by private health insurance plans.

Section 4012 also expresses the sense of Congress that eligible telehealth "originating sites" should be expanded. Section 4012 provides that any expansion of telehealth services in Medicare 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

the applicable conditions for Medicare coverage and payment if the same service were provided in person; and should involve clinically appropriate means for delivering services.

Title V-Savings

Section 5001. Savings in the Medicare Improvement Fund

The Medicare Improvements for Patient and Providers Act (P.L. 110-275) established Social Security Act Section 1898, which makes funds available to the HHS Secretary "to make improvements under the original Medicare fee-for-service program under parts A and B … including adjustments to payments for items and services furnished by providers of services and suppliers under such original Medicare fee-for-service program." Many subsequent laws have modified the amount in the fund, but to date none of the monies have been expended. Most recently, the Comprehensive Addiction and Recovery Act of 2016 (P.L. 114-198) modified Section 1898 to make $140 million available "during and after 2021."

Provision

Section 5001 would change the amount available in the fund from $140 million to $270 million.

Section 5002. Medicaid Reimbursement to States for Durable Medical Equipment

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. Federal upper payment limit regulations specify that states cannot pay more in the aggregate for certain types of services than the amount that would be paid for the services under the Medicare principles of reimbursement; the Medicare principles of reimbursement are based on methodologies that apply to regions and certain metropolitan areas, and may result in different payment amounts in different states. The Consolidated Appropriations Act, 2016 (P.L. 114-113) applies an upper payment limit to durable medical equipment (DME) under Medicaid for items and services furnished on or after January 1, 2019.

Provision

Section 5002 requires the federal upper payment limit on DME be implemented one year earlier – for items and services furnished on or after January 1, 2018.

Section 5003. Penalties for Violations of Grants, Contracts, and Other Agreements

Social Security Act Title XI identifies Medicare- and Medicaid-related anti-fraud provisions, which include penalties and exclusions on individuals and other entities that engage in certain types of federal health program misconduct (federal health programs include Medicare and Medicaid, as well as other programs that provide health benefits or insurance funded by the federal government). Under Social Security Act Section 1128A, the HHS OIG is authorized to impose civil monetary penalties (CMPs) and assessments on individuals, organizations, agencies,

or other entities, that engage in improper conduct related to federal health care programs, including penalties for knowingly presenting or causing to be presented to a federal or state employee or agent false or fraudulent claims (beneficiaries are not subject to civil penalties under Social Security Act Section 1128A).

For example, penalties may 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. Social Security Act 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.

Social Security Act 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. The HHS OIG has permissive authority to exclude entities or individuals from federal health programs under a number of circumstances such as: convictions for certain fraud misdemeanors, theft, embezzlement, breach of fiduciary duty, or other financial misconduct; convictions for interference or obstruction of criminal investigations; 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

Section 5003 amends Section 1128A of the Social Security Act by adding new subsections (o), (p), (q), (r), and (s). Under Section 5003 any person (including organizations, agencies, or other entities, but excluding beneficiaries) who commits improper conduct related to grants, contracts, or other agreements funded by HHS is subject to CMPs as follows:

Knowingly presents or causes to be presented a specified claim that the individual knows or should know was false is 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 is 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.

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 is subject, in addition to other penalties prescribed by law, to 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 are also subject to assessments of up to three times the total amount of the funds or property obligated to the HHS Secretary in lieu of damages sustained by the United States or a specified state agency;

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 is 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 are 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.

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 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 are 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 are subject to assessments of up to three times the total amount of the funds or property obligated to the HHS Secretary in lieu of damages sustained by the United States or a specified state agency).

Fails to grant timely access, upon reasonable request (as defined in regulations issued by the HHS Secretary), to the HHS OIG for conducting audits, investigations, evaluations, or other statutory functions related to grants, contracts, and other agreements with HHS is 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, Section 5003 authorizes the HHS Secretary to exclude individuals who knowingly commit improper conduct related to HHS-funded grants, contracts, and other agreements from participation in federal health care programs and to direct appropriate state agencies also to exclude these individuals from participation in any state health programs.

The Social Security Act Sections 1128A(c), (d), (g) and (h) apply to Section 5003 CMPs or assessments as they do to penalties, assessments or proceedings under Social Security Act Section 1128A(a). Section 5003 also specifies that in applying the Social Security Act Section 1128A(d), references to claims under Social Security Act Section 1128A(d) are treated as a reference to claims as defined in Section 5003.

Section 5003 defines the following terms applicable to Social Security Act Section 1128A(o) and (p):

Department means the Department of Health and Human Services (HHS).

Material means having a natural tendency to influence, or be capable of influencing, the payment or receipt of money or property.

Other agreement includes 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).

Program beneficiary means, 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 HHS Secretary provides funding, an individual who applies for, or who receives, such benefits or assistance from a grant, contract, or agreement. Program beneficiary does 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.

Recipient includes a sub-recipient or subcontractor.

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 HHS Secretary.

Under Section 5003, a specified claim under Social Security Act Section 1128A 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 [Social Security Act Section 1128A(i)(2), defines a claim as an application for payments for items and services under a federal health care program] and that:

(1) is presented or caused to be presented to an officer, employee, or agent of HHS or any agency thereof or any specified state agency; or

(2) is made to a contractor, grantee, or any other recipient if the money or property is to be spent or used on HHS's behalf or to advance an HHS program or interest, and if HHS:

(A) provides or has provided any portion of the money or property requested or demanded; or

(B) will reimburse the contractor, grantee, or other recipient for any portion of the money or property which is requested or demanded.

In addition, the term obligation as used in Social Security Act Section 1128A(o) means 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 or regulation, or from the retention of any overpayment.

Section 5003 also requires the following conforming amendments be made:

by adding "specified claims" to "claims" in Social Security Act Section 1128A(e); and

(A) in Social Security Act Section 1128A(f) in the matter before paragraph (1), inserting "or specified claim (as defined in subsection (r)) after "district where the claim"; and inserting "or, with respect to a person described in subsection (o), the person)" after "claimant"; and

(B) in the matter following paragraph (4) inserting "(or, in the case of a penalty or assessment under subsection (o), by a specified State agency (as defined in subsection (q)(6)),' after "or a State agency".

Section 5004. Reducing Overpayments of Infusion Drugs

Although most outpatient prescription drugs are covered under Medicare Part D, Medicare covers certain drugs and biologicals under Part B. Biological products are derived from living organisms rather than inorganic chemical compounds. Part B drugs and biologicals include drugs furnished incident to physician services, immunosuppressive drugs following a Medicare-covered organ transplant, erythropoietin for treating individuals with anemia who have end-stage renal disease, certain oral anti-cancer drugs, and drugs administered through DME. Medicare providers and suppliers purchase Part B drugs, and then are paid by Medicare after administering the drugs to beneficiaries.

Generally, Medicare reimburses physicians and other providers, such as hospital outpatient clinics, for Part B drugs and biologicals at 106% of the volume weighted average of each drug's average sales price (ASP) billed under the same billing code. Health care providers also are paid separately for the administration of Part B drugs and biologicals to patients.

Some Part B drugs and biologicals however, such as blood products, vaccines, and drugs administered through DME are reimbursed differently. Drugs administered through DME, such as infusion pumps, are reimbursed at 95% of the drug's average wholesale price (AWP) in effect on October 1, 2003. A drug or biological's AWP is a commercially published reference price, but not an average paid by purchasers or charged by wholesalers. AWP is considered a manufacturer's

suggested wholesale price to retailers and is published in drug pricing compendia. AWP is not defined in statute or regulation.

Section 303(b) of the Medicare Prescription Drug Improvement and Modernization Act of 2003 (MMA; P.L. 108-173) required the HHS Secretary to establish a competitive acquisition program for certain DME products in specified areas. Under the DME competitive acquisition program as described in the Social Security Act Section 1847, payment for DME items in competitive bidding areas is based on supplier bids, rather than on a Medicare DME fee schedule. Medicare Part B drugs administered through DME are included in DME competitive bidding. The DME competitive acquisition program started in nine metropolitan areas in January 2011 and has since expanded to 100 metropolitan areas.

Several HHS OIG reports, such as the February 2013 report, *Part B Payments for Drugs Infused through Durable Medical Equipment* (OEI-12-12-00310), have shown that under the Medicare Part B drug reimbursement methodology based on AWP the amount Medicare paid DME suppliers for some drugs furnished through DME was substantially greater than what it cost DME suppliers to purchase those drugs. Based on an April 2015 OIG study, *Implementing OIG Recommendations Could Have Reduced Payments for DME Infusion Drugs by Hundreds of Millions of Dollars* (OEI-12-15-00110), other Part B drugs furnished through DME, such as insulin (administered through an infusion pump), the amount it cost DME suppliers to purchase some drugs was considerably less than what Medicare paid for those drugs drug acquisition costs generally exceeded the Medicare payment rate.

Provision

Beginning on January 1, 2017, Section 5004 requires Medicare to reimburse DME suppliers for Medicare Part B infusion drugs and biologicals furnished through DME in the same manner as other Medicare Part B drugs; on the basis of 106% of the volume weighted average of the ASPs of drugs included in the same Medicare billing code. Section 5004 excludes Medicare Part B drugs and biologicals furnished through DME from the DME competitive acquisition program.

Section 5005. Increasing Oversight of Termination of Medicaid Providers

Prior to passage of the Cures Act, state Medicaid programs were required to promptly notify the HHS Secretary (and for physicians, also the state licensing board) when they terminated, suspended, otherwise sanctioned, or prohibited providers (or other individuals) from participating under the state Medicaid plan.

In addition, prior to passage of the Cures Act, states were required to terminate individuals or entities from their state Medicaid program when the HHS Secretary or another state Medicaid program terminated participating providers for cause -- fraud, integrity, or quality issues. States also may terminate providers for reasons other than cause such as inactivity, death, and failure to renew their license or revalidate enrollment, but states are not obligated to report non-cause terminations to the HHS Secretary. States also were required to deny claims for items or services provided by terminated individuals or entities for the duration of the termination and the HHS Secretary is required to recover the federal share of claims paid by states (or Medicaid managed care entities) to terminated providers.

The Patient Protection and Affordable Care Act (ACA, P.L. 111-148, as amended) Section 6401(b)(2) required the CMS Administrator to establish a process to notify all state Medicaid and CHIP programs within 30 days of the effective date of a provider termination by Medicare or any state Medicaid or CHIP program. To address the notification requirement, CMS established an Internet web-based portal which was replaced in 2014 by the “Termination Notification

Database." Both the web-based portal and the Termination Notification Database enable states to voluntarily report provider and other entity terminations and also to identify individuals and entities that other state programs terminated. In March 2014 and August 2015 reports, the HHS OIG identified shortcomings with the voluntary termination reporting such as that the state provider termination data included providers terminated for reasons other than cause and the reported data were often insufficient for other states to confidently use to identify terminated providers.

Prior to passage of the Cures Act, states were not required to enroll providers employed by Medicaid managed care entities and, potentially, terminate those providers for cause, although states were obligated to inform managed care plans of the requirement to screen all employees and contractors for federal health program exclusions. Managed care entities under contract to state Medicaid programs were required to identify providers in the plan's network, who were terminated or otherwise sanctioned by a state Medicaid agency or Medicare. In addition, state Medicaid programs were required to identify and report to the CMS administrator or the HHS Secretary terminations for cause and other sanctions on Medicaid managed care providers.

Provision

Beginning on July 1, 2018, Section 5005 requires states to submit information within 30 days of the effective date on participating providers of services or any other person under a Medicaid state plan or a waiver who was terminated. Following the notice to the HHS Secretary that a provider was terminated, states are required to submit to the HHS Secretary the following information, as appropriate, on terminated providers: (1) the terminated provider's name; (2) the type of provider; (3) the provider's practice specialty; (4) the provider's date of birth, Social Security number, national provider identification number, and state license or certification number; (5) the termination reason; (6) a copy of the termination notice; (7) the effective date of the termination; and (8) any other information the HHS Secretary requires.

The termination notice effective date is defined as the later of (a) the date on which the termination is effective, as specified on the termination notice, or (b) the date on which all applicable appeal rights have been exhausted or the timeline for appeal has expired.

Section 5005 requires the HHS Secretary within 30 days of notification of a provider termination to review the provider termination and, if appropriate, include the termination in Termination Notification Database or similar system that was developed under ACA Section 6401(b)(2).

Under Section 5005, by July 1, 2018 states are required to include a provision in Medicaid and CHIP managed care contracts that managed care entities will terminate from their networks any providers of services or individuals terminated from Medicare or any state Medicaid or CHIP program.

Beginning on July 1, 2018, Section 5005 requires the HHS Secretary to prohibit payment to states for Medicaid expenditures for terminated fee-for-service (FFS) providers under a Medicaid state plan or waiver. The effective date for the prohibition is 60 days after the date on which terminated providers are added to the termination database or similar system required by ACA Section 6401(b)(2).

Beginning on July 1, 2018, Section 5005 requires the HHS Secretary to prohibit payments to states for managed care expenditures incurred by the state for Medicaid state plan services (or waivers) provided by terminated providers. Beginning on July 1, 2018, the HHS Secretary is prohibited from reimbursing states for the federal share of services provided by managed care entities unless the state has a contract with the managed care entity that complies with the Section

5005 requirement for Medicaid managed care contracts to include a provision requiring managed care entities terminate providers from the managed care entity network who were terminated from Medicare or other state Medicaid or CHIP programs.

By July 1, 2017, in consultation with state Medicaid directors, the HHS Secretary is required by Section 5005 to issue regulations establishing uniform terminology for describing the reasons providers are terminated from Medicaid or CHIP.

By January 1, 2017, Section 5005 requires states to require that Medicaid FFS and managed care entity (by January 1, 2018) providers to enroll with the state by submitting the following identifying information the providers: (1) name, (2) specialty, (3) date of birth, (4) Social Security number, (5) national provider identification number (if applicable), (6) federal taxpayer identification number, and (7) state license or certification number (if applicable). Participating FFS and managed care entity providers include entities that furnish items and services, order, prescribe, refer, or certify Medicaid eligibility for services under a Medicaid state plan or waiver.

Section 5005 specifies that certain Medicaid requirements must apply to states under CHIP in the same manner as they apply to state Medicaid programs. More specifically, Section 5005 requires state CHIPs to terminate providers if the providers were terminated by Medicare or other state Medicaid or CHIP programs. By January 1, 2017, Section 5005 requires state CHIPs to require participating FFS providers to enroll by submitting identifying information. In addition, Section 5005 requires the Secretary to limit federal matching payments to states if states have not implemented the requirement that managed care contracts include a provision agreeing to terminate providers who were terminated by Medicare or other state Medicaid or CHIP programs.

Before March 31, 2020, the HHS OIG is required to submit a report to Congress on the implementation of Section 5005. The report is required to include the following:

(1) an assessment of the extent to which providers who are included in the termination notification database as required by Section 5005 are terminated from participation in state Medicaid plans or waivers;

(2) information on federal financial participation paid to states in violation of Section 5005 prohibition on payments to states for terminated providers and payments to Medicaid managed care entities that were required to terminate providers who were terminated under Medicare or other state Medicaid plans or waivers;

(3) an assessment of the extent to which state contracts with Medicaid managed care entities comply with the new Section 5005 requirement that state managed care contracts include a provision barring terminated providers from participation in Medicaid and CHIP provider networks; and

(4) an assessment of the extent to which states are enrolling FFS and managed care providers participating in Medicaid or under a waiver as required by Section 5005.

Section 5006. Requiring Publication of Fee-for-Service Provider Directory

Provider directories—lists of the health care providers contracted to furnish care under a health care program—are useful in ensuring that eligible individuals have access to covered services. States have considerable discretion in how they communicate sources of available care under their fee-for-service (FFS) Medicaid programs to beneficiaries. Federal law currently does not require state Medicaid programs to publish FFS provider directories.

For Medicaid services furnished through managed care, by contrast, provider directories are federally required. Under new Medicaid managed care regulations that will take effect for

contract years beginning on or after July 1, 2017, the directories must cover a wider range of providers, include more information, and be available on the managed care entity's website.[165]

Provision

Section 5006 requires state Medicaid programs to publish annually and make available on their websites a FFS provider directory. The directory must identify participating physicians and may at state option include other participating provider types, listing at minimum the provider's contact information and specialty. For providers that participate in a primary care case management (PCCM) system, the directory must indicate whether the physician or other provider is accepting new Medicaid patients and the provider's cultural and linguistic capabilities.

The requirements of Section 5006 do not apply to any state where all Medicaid beneficiaries receiving services under the Medicaid state plan or waiver, except Indians or Alaska Natives, are enrolled in a comprehensive risk-based managed care organization (MCO) or similar prepaid health plan. Section 5006 will bring the administration of Medicaid FFS and managed care programs into closer alignment.

The state FFS provider directories must be published no later than January 1, 2017. If the Secretary of Health and Human Services determines that state legislation would be required for any particular state to have the authority to amend its Medicaid state plan to require the publication of a FFS provider directory, then the state will be considered compliant with the timing requirement of Section 5006 so long as it publishes its provider directory before the first day of the first calendar quarter after the close of the first regular legislative session beginning after the date of enactment of the Cures Act.

Section 5007. Fairness in Medicaid Supplemental Needs Trusts

Under federal Medicaid law, most trusts are counted as an asset in determining Medicaid eligibility for aged and disabled individuals and are subject to asset transfer rules. However, there are certain exceptions in current law to the general rule of counting trusts as an asset. Specifically, Medicaid does not count certain special-needs trusts and pooled trusts as assets and does not apply asset transfer rules to these trust types. This exception is commonly referred to as the "special needs trust exception." In order for a trust to meet this exception under Medicaid, a trust must contain the assets of an individual under age 65 (i.e., non-elderly individual) who meets the statutory definition of disability under SSA Section 1614(a)(3).

SSA Section 1917(d)(4)(A) permits only parents, grandparents, legal guardians, or a court to establish a special needs trust on behalf of a non-elderly disabled individual. Such trusts must contain assets of the disabled individual and the trust must be used to provide funding for certain expenditures that supplement Medicaid benefits, subject to certain limitations. Special needs trusts allow non-elderly individuals with disabilities to maintain their eligibility for Medicaid. When the beneficiary dies, the state receives the remaining proceeds of the trust equal to any amounts paid for medical assistance provided under the state Medicaid program.

Provision

Section 5007 makes a technical correction to the language regarding special needs and pooled trusts under Medicaid, which are exempt from asset counting and transfer rules, to allow non-

[165] 42 C.F.R. §438.10(h), 81 *Federal Register* 27867 (May 6, 2016).

elderly individuals with disabilities to establish a special needs trust on their own behalf. This provision is effective on or after the date of enactment.

Section 5008. Eliminating Federal Financial Participation with Respect to Expenditures under Medicaid for Agents Used for Cosmetic Purposes or Hair Growth

Outpatient prescription drugs are an optional Medicaid benefit, but all states cover prescription drugs for most beneficiary groups. Medicaid law requires prescription drug manufacturers who wish to sell their products to Medicaid agencies to enter into rebate agreements with the HHS Secretary on behalf of states. Under these voluntary rebate agreements, drug manufacturers pay a rebate to state Medicaid agencies for drugs purchased for Medicaid beneficiaries. Most drug manufacturers participate in the Medicaid drug rebate program.

The Medicaid rebate program requires states to cover all of a participating manufacturer's drugs, but states have the option to not cover or restrict use of certain drugs, drug classes, or drug uses which are identified in the Social Security Act, Section 1927(d)(2). This list of Medicaid excluded drugs includes drugs used for cosmetic purposes or hair growth. When states elect to cover the statutorily excluded drugs, drug classes or drug uses, they receive federal financial participation (FFP), the federal share of state Medicaid expenditures. States are prohibited from receiving FFP when they cover certain statutorily excluded drugs, such as sexual or erectile dysfunction drugs, except when those drugs are medically necessary for other purposes.

Provision

Beginning with the enactment date of the Cures Act, Section 5008 prohibits states from receiving FFP for drugs used for cosmetic purposes and hair growth, except when those drugs are medically necessary.

Section 5009. Amendment to the Prevention and Public Health Fund

Section 4002 of the Patient Protection and Affordable Care Act (ACA, P.L. 111-148, as amended) established the Prevention and Public Health Fund (PPHF), to be administered by the HHS Secretary, and provided it with a permanent annual appropriation.[166] Prior to enactment of the Cures Act, appropriations to the PPHF were as follows:

- for FY2010, $500 million;
- for each of fiscal years 2012 through 2017, $1 billion;
- for each of fiscal years 2018 and 2019, $1.25 billion;
- for each of fiscal years 2020 and 2021, $1.5 billion; and
- for FY2022, and each fiscal year thereafter, $2 billion.

Provision

Section 5009 amends the PPHF appropriation as follows:

- for each of fiscal years 2018 and 2019, $900 million;

[166] See Appendix B in CRS Report R44505, *Public Health Service Agencies: Overview and Funding (FY2015-FY2017)*, coordinated by C. Stephen Redhead and Agata Dabrowska. PPHF authority is codified at 42 U.S.C. §300u-11.

- for each of fiscal years 2020 and 2021, $1 billion;
- for FY2022, $1.5 billion;
- for FY2023, $1 billion;
- for FY2024, $1.7 billion; and
- for FY2025 and each fiscal year thereafter, $2 billion.

This amendment decreases the total PPHF appropriation for FY2018 through FY2024 by $3.5 billion.

Section 5010. Strategic Petroleum Reserve Drawdown

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 695 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 an oil sharing program in times of emergency shortage. The current SPR oil inventory represents 149 days of net import coverage.

The President may authorize an SPR drawdown upon determining that a severe oil supply interruption exists nationally, or internationally, or is imminent. The Secretary of Energy also has limited authority to release oil from the SPR for a test drawdown.

Provision

Section 5010 directs the Secretary of Energy to drawdown and sell crude oil from the SPR in the amount of 10 million barrels during FY2017, 9 million barrels in FY2018, and 6 million barrels in FY2019, for a total of 25 million barrels. The resultant oil sales revenue is to be deposited in the general fund of the Treasury during the fiscal year corresponding to the year of sale.

Section 5010 amends SPR drawdown limitations as specified in subparagraphs (c) and (d) of EPCA Section 161 (h)(2). The amendment sets the minimum holding levels of the SPR at 450 million barrels, instead of the current minimum holding level of 500 million barrels.

Section 5011. Rescission of Portion of ACA Territory Funding

ACA Section 1323 provides that each U.S. territory can choose whether to establish a health insurance exchange by October 1, 2013. If a territory elects to establish an exchange, it could receive a portion of a $1 billion appropriation to provide financial assistance to individuals who obtain coverage through the exchange. If a territory does not elect to establish an exchange, it could receive an increase in Medicaid funds. No territory elected to establish an exchange.

Provision

Section 5011 rescinds $464 million from unobligated amounts of the $1 billion appropriation for U.S. territories that elect to establish an exchange.

Section 5012. Medicare Coverage of Home Infusion Therapy

Medicare Part B covers a variety of durable medical equipment (DME) when it is medically necessary and prescribed by a physician. Durable medical equipment (DME) 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. Infusion pumps are DME, and the drugs infused are covered supplies necessary for the functioning of the DME. Infusion pumps are covered by Medicare if, in part, the administration of the drug in the home is reasonable and necessary, an infusion pump is necessary to safely administer a drug, and either (a) the drug is administered by a prolonged infusion of at least 8 hours because of proven improved clinical efficacy, or (b) the drug is administered by intermittent infusion (each episode of infusion lasting less than 8 hours) that does not require the beneficiary to return to a physician's office prior to the beginning of each infusion and toxicity or adverse side effects of the drug are unavoidable without infusing it at a strictly controlled rate. The DME benefit does not include coverage of personnel to assist with the infusion (as the requirement that the administration of the drug in the home is reasonable is a condition of coverage).

Under a separate provision of law (not DME), Social Security Act, Section 1861(s)(2)(Z), Medicare Part B is required to cover intravenous immune globulin (IVIG) for the treatment of primary immune deficiency diseases in the home. However, the statutes do not cover the items and services necessary for the in-home administration of IVIG. The specific items and services are the supplies and in-home nursing services necessary to inject the IVIG intravenously.[167]

The Medicare IVIG Access and Strengthening Medicare and Repaying Taxpayers Act of 2012 (Medicare IVIG Access Act, P.L. 112-242) requires the HHS Secretary to establish a demonstration project to evaluate the benefit of providing payment for items and services needed for the in-home administration of IVIG. The IVIG demonstration began August 2014 and will end September 30, 2017. The Medicare IVIG Access Act also required the HHS Secretary to establish a per visit payment amount for items and services (including nursing services) needed for the in-home administration of IVIG based on national per visit low-utilization payment amount under the prospective payment system for home health services covered under Medicare.

In order to receive payments under Medicare Part B and retain a Medicare billing number, DME suppliers that furnish items of equipment or provide services under Medicare Part B must comply with quality and other Medicare conditions of participation requirements. Medicare conditions of participation for DME suppliers include being licensed in the state where suppliers are located and being accredited by an independent accreditation organization approved by CMS.

Provision

Section 5012 creates a new Medicare home infusion therapy benefit, effective January 1, 2021. Home infusion therapy is defined as a specific set of *items and services* furnished by a qualified home infusion therapy *supplier*, which are furnished in the *home* to an individual who is under

[167] U.S. Department of Health and Human Services, *Evaluation of the Medicare Patient Intravenous Immunoglobulin Demonstration Project: Interim Report to Congress*, March 2016, p. 2, https://innovation.cms.gov/Files/reports/ivig-intrtc.pdf.

the care of an *applicable provider* and who has a plan prescribed by a physician that specifies the type, amount, and duration of infusion therapy to be provided. The physician must also periodically review the plan.

Specifically, the *items and services* included in home infusion therapy consist of professional services, including nursing services, training and education (not included as part of the training and education associated with durable medical equipment), remote monitoring and monitoring services, and home infusion therapy drugs. A qualified home infusion *supplier* means a pharmacy, physician, or other provider or supplier licensed by the State in which she practices and who furnishes infusion therapy to individuals with acute or chronic conditions requiring administration of home infusion drugs, ensures safe and effective administration, is accredited, and meets other requirements determined by the Secretary; a home infusion therapy supplier may meet these qualification requirements by subcontracting with another pharmacy, physician, provider of services, or supplier who meets the requirements. An *applicable provider* (whom cares for the individual) is defined as a physician, nurse practitioner, and a physician assistant. *Home* has the same definition under the home infusion therapy benefit as under the durable medical equipment benefit. Home infusion drug is defined as a drug or biologic administered intravenously or subcutaneously for an administration period of 15 minutes or more, in the home of an individual through a DME pump, and does not include insulin pump systems or self-administered drugs or biologicals on a self-administered drug exclusion list. Prior to furnishing home infusion therapy, the physician who establishes the plan is required to notify the beneficiary of the options available for infusion therapy (home, physician's office, hospital outpatient department.)

Section 5012 requires the Secretary to implement a payment system for the new benefit described above. The payment is to be determined on a per-day basis, and is to vary by type of therapy and nursing utilization, and is to be adjusted by a geographic wage index, patient acuity, and complexity of drug administration. The payments will be updated yearly by the percent increase in the Consumer Price Index for all urban consumers for the 12-month period ending in June of the preceding year, adjusted for a measure of nationwide economic productivity. The per-day payment amounts determined in this way are prohibited from exceeding the cost of infusion therapy services provided in a physician's office. In addition, the Secretary has discretion to make adjustments to reflect outliers (or excessively costly patients) in a budget-neutral manner. In developing the payment system, the Secretary may consider the costs of providing infusion therapy, consult with suppliers, and consider payments for similar services under Medicare Part A or Medicare Advantage, and private insurance.

Section 5012 allows the Secretary to consider prior authorization requirements for home infusion therapy services.

Section 5012 requires the Secretary to designate organizations for accrediting home infusion therapy suppliers by not later than January 1, 2021. The Secretary is to consider the following factors in designating the accreditation organizations: (a) the ability of the organization to conduct timely reviews, (b) the ability of the organization to take into account the capacities of suppliers located in rural areas, (c) whether the organization has established reasonable fees for their accreditation services, and (d) such other factors as the Secretary determines appropriate. The Secretary is required to review the list of designated accreditation organizations, taking into account those factors specified above, and may, *by regulation*, modify the list of accreditation organizations. If the Secretary removes an organization from the list of accreditation organizations, any supplier that is accredited by the organization will be considered to have been accredited for the remainder of the effective period of accreditation, even after the organization is removed from the list of accrediting organizations. If an accrediting organization is designated by the Secretary before January 1, 2019, and a supplier is accredited by the organization before

January 1, 2021, then that supplier will be considered to have been accredited as of January 1, 2023 and for the remainder of the effective period of accreditation; this provision would allow suppliers that received accreditation early to avoid having to be re-accredited for an extended period.

Appendix. List of Acronyms

ACA: Patient Protection and Affordable Care Act (P.L. 110-148, as amended)

ACCV: Advisory Commission on Childhood Vaccines

ACIP: Advisory Committee on Immunization Practices

ASC: ambulatory surgical center

ASPR: Health and Human Services Assistant Secretary for Preparedness and Response

AST: Antimicrobial Susceptibility Testing

BARDA: Biomedical Advanced Research and Development Authority

BRAIN Initiative: Brain Research through Advancing Innovative Neurotechnologies Initiative

CACs: Carrier Advisory Committees

CARB: National Strategy for Combating Antibiotic-Resistant Bacteria

CBER: FDA Center for Biologics Evaluation and Research

CBRN: chemical, biological, radiological, or nuclear

CDC: Centers for Disease Control

CDER: Center for Drug Evaluation and Research

CDRH: Center for Devices and Radiological Health

CDS: clinical decision support

The Center: National Center for Medical Rehabilitation Research

CLIA: Clinical Laboratory Improvement Amendments of 1988

CMPs: civil monetary penalties

CMS: Centers for Medicare & Medicaid Services

CMMI: Center for Medicare and Medicaid Innovation

COW: certificate of waiver

DME: durable medical equipment

DOD: Department of Defense

EHR: electronic health record

EPCA: Energy Policy and Conservation Act of 1975 (42 U.S.C. 6241)

ETASU: elements to assure safe use

EUA: Emergency Use Authorization

FACA: Federal Advisory Committee Act

FAERS: FDA Adverse Event Reporting System database

FDP: Federal Demonstration Partnership

FDA: Food and Drug Administration

FDAAA: Food and Drug Administration Amendments Act of 2007 (P.L. 110-85)

FDAMA: Food and Drug Administration Modernization Act of 1997 (P.L. 105-115)

FDASIA: Food and Drug Administration Safety and Innovation Act (P.L. 112-144)

FFDCA: Federal Food, Drug, and Cosmetic Act

FFS: fee-for-service

FOIA: Freedom of Information Act

FY: Fiscal Year

GAO: Government Accountability Office

GS: General Schedule

HHS: Department of Health and Human Services

HELP: Senate Health, Labor, Education, and Pensions Committee

HIPAA: Health Insurance Portability and Accountability Act of 1986

HCT/Ps: human cells, tissues, and cellular and tissue-based products

HDE: Humanitarian Device Exemption

HIT: health information technology

HITECH: Health Information Technology for Economic and Clinical Health Act of 2009

IHS: Indian Health Service

IRB: Institutional Review Board

IACUC: Institutional Animal Care and Use Committee

IC: NIH Institutes and Centers

IDE: investigational device exemption

IEA: International Energy Agency

IOM: Institute of Medicine

LCD: local coverage determinations

MACRA: Medicare Access and CHIP Reauthorization Act of 2015

MACs: Medicare Administrative Contractors

MCO: managed care organization

MDA: Medical Device Amendments of 1976 (P.L. 94-295)

MedPAC: Medicare Payment Advisory Commission

MPFS: Medicare physician fee schedule

NAS: National Academy of Sciences

NCATS: NIH's National Center for Advancing Translational Sciences

NCDs: National coverage decisions

NDA: new drug application

NIH: National Institutes of Health (NIH)

NICHD: Eunice Kennedy Shriver National Institute of Child Health and Human Development

NLM: National Library of Medicine

OAPEC: Organization of the Arab Petroleum Exporting Countries

OCP: Office of Combination Products

OCR: HHS Office for Civil Rights

OIG: HHS Office of Inspector General

OIRA: Office of Information and Regulatory Affairs

OMB: Office of Management and Budget

ONC: Office of the National Coordinator for Health Information Technology

OSTP: White House Office of Science and Technology Policy

OT: Other transaction

OPPS: Medicare hospital outpatient prospective payment system fee schedule

PAHPRA: Pandemic and All-Hazards Preparedness Reauthorization Act of 2013 (P.L. 113-5)

PCCM: primary care case management system

PDUFA: Prescription Drug User Fee Act of 1992

PHI: protected health information

PHS: Public Health Service agencies

PHSA: Public Health Service Act of 1944

PMA: premarket approval pathway

PMI: Precision Medicine Initiative

PPHF: Prevention and Public Health Fund

PRA: Paperwork Reduction Act (44 U.S.C. Chapter 35)

R&D: research and development

REMS: risk evaluation and mitigation strategies

SAMHSA: Substance Abuse and Mental Health Services Administration

SBRS: Silvio O. Conte Senior Biomedical Research Service

SSA: Social Security Act

SPR: Strategic Petroleum Reserve

VA: Department of Veterans Affairs

VDT: view, download, and transmit

VFC: Vaccines for Children program

VICP: National Vaccine Injury Compensation Program

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