AMCP Academy of Managed Care Pharmacy®
www.amcp.org

SUMMARY

21st CENTURY CURES ACT
PUBLIC LAW: 114 – 255

The 21st Century Cures Act (the Act) (H.R. 34) passed the House of Representatives on November 30, 2016, by a 392-26 vote and then the Senate on December 7, 2016, by a 94-5 vote. The President signed the Act into law on December 13, 2016 (becoming Pub.L No.114-255). The Act comprises an array of provisions aimed at improving and modernizing various aspects of the health care system, including three primary titles that address acceleration of medical product ***discovery, development, and delivery***. Overall, the Act intends to improve the health of Americans by promoting the discovery of new cures for cancer, improving mental health, and combating opioid addiction.

The Academy of Managed Care Pharmacy (AMCP) supports to provisions of the Act related to health care economic communications between health care decision makers and pharmaceutical manufacturers and combatting opioids addiction. AMCP is committed continue to playing an important role in the implementation of these provisions, particularly in regard to approval of medications and new health care technology and research initiatives. AMCP will continue to apprise members of any developments regarding the Cures Act and to seek member feedback, when necessary.

Introduction. The bill signed by the president was an agglomeration of several other bills, including the original 21st Century Cures legislation (H.R.6) which passed the House in 2015. Parts of H.R. 6 contained in the Act address medical and technology innovation, mental health reform provisions included in the Helping Families in Mental Health Crisis Act of 2016 (H.R. 2646), and several Medicare reforms. The Act's Medicare provisions reduce payments for the inpatient hospital setting, resulting in a potential savings of $760 million over the next decade. Other provisions give Medicare Advantage plans, long-term acute care hospitals, and suppliers of durable medical equipment relief from certain future changes in payment policy.

Below is a summary of provisions relevant to AMCP members.

Funding to Combat Opioid Abuse. The first title of the Act (Innovation Projects and State Responses to Opioid Abuse) builds on the recently enacted Comprehensive Addiction and Recovery Act (CARA), granting states $1 billion over the next two years for drug abuse prevention and treatment programs. AMCP supported certain provisions in CARA to reduce opioid misuse or abuse and is pleased to that the Act further addresses the issue of opioid abuse.

Discovery. This title instructs the National Institutes of Health (NIH) to establish an Innovation Prizes Program to fund areas of biomedical research "that could realize significant enhancements or improve health outcomes." It also enables the NIH to offer support in later phases of clinical trials, calls for more collaboration with the U.S. Food and Drug Administration (FDA) to "implement a system that allows further research on clinical trial data," and establishes a

nonprofit organization, Council for 21st Century Cures, to focus on accelerating medicine discovery, development, and delivery.

This title also authorizes Congress to appropriate up to $4.8 billion over 10 years for new research at NIH, including $1.8 billion for the cancer research "moonshot" championed by Vice President Biden, and $1.4 billion for the Precision Medicine Initiative. The latter, which was championed by President Obama, is collecting genetic data on one million American volunteers for use in developing new treatments. The FDA will receive $500 million to pay for its new responsibilities under the Act.

AMCP will monitor and analyze FDA regulations and guidance that implement the Act and the types of data and methodologies used to develop new medicines and treatments. This information will be important for pharmacists, physicians and nurses who make population health decisions for patients: it could inform medication coverage and utilization review decisions and analysis. AMCP will also monitor the manner in which FDA approves medications under the new law – it could significantly affect the level and type of post-marketing surveillance, as well as require additional precautions for products approved under this pathway relating to the use in certain populations.

Provisions in this title are also important for the development of precision medicine initiatives. In 2017, AMCP will convene experts to examine the impact of precision medicine on medication management and decisions on pharmacists, nurses, and physicians who work in population health settings. Precision medicine will require changes in the ways that population health care providers work with direct care providers to determine appropriate candidates for precision medicine and the factors that must be considered. AMCP will closely monitor regulations and guidance implementing this section.

Development. This title amends the Federal Food, Drug, and Cosmetic Act (FD&C Act) to require the FDA to "establish processes under which patient experience data may be considered in the risk-benefit assessment of a new drug." The Act requires the FDA to issue guidance regarding how to collect patient experience data. Such guidance documents shall address:

- Appropriate ways to collect data for use by the FDA in regulatory decisions;
- The process for patient submissions to FDA for draft guidance;
- The FDA process for responding to patient experience data submissions to FDA;
- The format and content for patient experience data submissions to FDA; and
- FDA's use of relevant patient experience data and related information when evaluating the risks and benefits of a drug.

Collection of patient experience data was included in the Prescription Drug User Fee Act reauthorization (PDUFA V), but no regulations from PDUFA V have been promulgated. PDUFA V began in 2012 and will expire at the end of 2017. Therefore, it is likely that the bill reauthorizing PDUFA in 2017 could include additional provisions.

The Act also requires the FDA to evaluate real world evidence submitted in support of a new indication for a previously approved drug, as well as submitted to help support or satisfy post-approval study requirements. AMCP will monitor implementation of these provisions because its members require information on the process and study design used to approve medications and other treatments. AMCP is also interested in the use of patient-reported outcomes data as a component of active post-market monitoring and tracking for medications and biologics.

The FDA must also identify "precision" drugs to treat serious or rare diseases and expedite their development, establish a program for priority review of breakthrough medical devices and train FDA employees on the "least burdensome appropriate means" concept in their review of medical devices. At the same time, the Act would remove FDA authority in certain regards, such as requiring the agency to rely on third-party certification of the safety and effectiveness of medical devices as meeting FDA criteria. AMCP will monitor these provisions and determine the impact on AMCP members and patient access to safe and effective medical technology.

Protecting Data and Patient Information in Biomedical Research

The Act establishes a straightforward way for NIH-funded investigators to share their data. The Act resolves data sharing issues by allowing the NIH director to require that data from NIH-supported research be published, giving all scientists the opportunity to use these data as quickly as possible to advance biomedical research.

The Act contains advances in research privacy protections. Certificates of confidentiality, previously available to researchers upon request, will now be provided to all NIH-funded scientists conducting research that involves the collection of *identifiable*, sensitive information. The certificates will provide stronger protections against the disclosure of the names of participants or any other identifiable data gathered during research. In addition, the Act will allow the NIH to withhold biomedical information about individuals that could be used to re-identify them through requests for records filed under the Freedom of Information Act.

Enhancing the Rigor and Reproducibility of Scientific Research

The Secretary of the Department of Health and Human Services (HHS) is tasked with convening a working group to develop recommendations for a formal policy to enhance the rigor and reproducibility of NIH-funded scientific research. The working group shall consider, as appropriate:

- Pre-clinical experiment design, including analysis of sex as a biological variable;
- Clinical experiment design;
- Applicable levels of rigor in statistical methods, methodology, and analysis, and
- Data and information sharing.

The Act requires the Director of NIH to consider the working group's recommendations and develop or update policies as appropriate within 18 months and then submit those changes to Congress within two years. AMCP is interested in this provision because rigor associated with

research and reproducibility is critical to ensuring review of data by health care professionals who make population health decisions based on evidence.

Health Care Economic Information (Section 3037)

Section 3037 amends the Federal Food, Drug, and Cosmetic Act's (FDCA) prohibition on misbranding by expanding the scope of off-label information that manufacturers can share with payers. Specifically, the provision broadens an *existing* safe harbor for sharing "health care economic information" (HCEI) with formulary committees, expanding eligible recipients to additional types of payer entities. Although the expanded language refers to off-label information about drugs and biologics, the same principles might provide a road map in the event that FDA—or Congress in this year's FDA PDUFA reauthorization—clarifies the parameters for the sharing of health care economic information about off-label uses of devices.

This section takes an important step towards creating a value- and outcomes-based health care system that will give patients the medicines they need while ensuring the wise use of health care dollars. This section modernizes Section 114 of the Food and Drug Administration Modernization Act (FDAMA114) of 1997, which was designed as a safe harbor for manufacturers to share health care economic information with entities that make formulary and coverage decisions. AMCP actively supports proactive communication of HCEI between health care decision makers and biopharmaceutical manufacturers. In March 2016, AMCP held a forum to develop recommendations on how Section 114 should be clarified and expanded to provide the clarity necessary to truly operationalize it with stakeholders representing population health decision makers, biopharmaceutical manufacturers, providers, patients, health economists, academia, and others. The formal recommendations from the forum were published in the July 2016 issue of the *Journal of Managed Care and Specialty Pharmacy*. Also, in October, AMCP organized a briefing on the issue for Congressional staff who focus on health care.

AMCP is pleased that the changes in *Section 3037* align with the several recommendations developed during the AMCP forum and AMCP is appreciative of their inclusion. However, AMCP and its stakeholders are concerned that even with the proposed changes and modernization, FDA guidance is still needed. Therefore, AMCP urges Congress to encourage the FDA to provide guidance to clarify FDAMA Section 114 and provide the level of clarity necessary for biopharmaceutical manufacturers and population health decision makers to truly operationalize these proactive communications. Furthermore, AMCP encourages the FDA to carefully consider the consensus recommendations developed during the AMCP forum as a starting point for guidance development as they represent the current thinking of stakeholders.

Combination Product Innovation

This section aims to improve the regulation of medical products that contain both a drug or biologic and a device, known as combination products. It would require that FDA meet with sponsors and agree early in development how to best study the combination product to meet the standard for approval. This section also clarifies how dispute resolutions work when the different

centers of FDA do not agree, and it includes provisions for reporting on combination product regulations.

AMCP will carefully monitor the implementing guidance and regulations, as we are concerned that combination products often result in an extension of patent life for older medications with little added clinical benefit but additional costs.

Limited Population Pathway
This section provides FDA with more flexibility to approve antimicrobial drugs based on a limited population if the drug treats a life-threatening infection.

If FDA approves a drug based on a limited population, the labeling and advertising of an antimicrobial drug shall contain "Limited Population" along with a proprietary name of the drug. This section also gives FDA the authority to review and approve promotional materials of a drug approved based on a limited population at least 30 days prior to drug dissemination.

Delivery. This title helps deliver newly tested and approved drugs to patients. It also contains provisions to enhance interoperability of electronic health records systems can help improve a seamless patient experience.

Interoperability
The Act strengthens efforts to improve and enforce health information interoperability. Beginning in January 2018, vendors' relative interoperability will be evaluated, and by 2019, vendors not in compliance will lose certification. AMCP supports interoperability of medical records based on standard transactions with the ability of pharmacists to fully access and share information in a bi-directional manner. AMCP will continue its work with the Pharmacist Health Information Technology Collaborative to advance these efforts and determine the impact of the provisions in the Act.

Coverage and Payment Provisions
The Act includes a number of Medicare- and Medicaid-related provisions, many of which have implications for coverage and payment for medical devices and diagnostics.

Increased Transparency for Medicare Local Coverage Determinations (LCDs)
LCDs are made by Medicare Administrative Contractors (MACs) to cover (or limit coverage for) a particular item or service, such as a device, within the MAC's assigned geographic region. Beginning six months after enactment, MACs must post certain information on their websites at least 45 days prior to the effective date of a final LCD, including a summary of the evidence considered and an explanation of the rationale supporting the LCD. Although current Centers for Medicare & Medicaid Services (CMS) policy requires MACs to provide notice and opportunity for stakeholder comment on draft LCDs, the Act's provision enhances these existing procedural protections.

Medicare Pharmaceutical and Technology Ombudsman
The Act establishes a "pharmaceutical and technology ombudsman" within CMS to handle complaints, grievances, and other requests from pharmaceutical and medical device manufacturers seeking Medicare coverage for their products. The ombudsman would specifically be charged with addressing coverage, coding and payment, providing manufacturers with a potential new avenue to work with the agency to resolve access barriers arising from these issues. The new ombudsman must be in place no later than 12 months after the date of enactment of the Act. AMCP will carefully monitor regulatory activity in this area, particularly issues related to the responsibilities of this position.

Reducing Overpayments of Infusion Drugs
Section 5004 addresses findings from an HHS Office of Inspector General (OIG) report that determined Medicare has overpaid for certain drugs and underpaid for others by applying a new pricing methodology to better reflect actual transaction prices. The payment amount for Part B infusion drugs furnished through durable medical equipment will be set to Average Sales Price (ASP) plus 6% beginning on January 1, 2017. These drugs were previously paid based on 95% of the Average Wholesale Price (AWP) that took effect on October 1, 2003. The OIG concluded that applying the ASP+6 percent methodology to infused drugs would result in payment amounts that reflect actual transaction prices.

Medicare Part B Payment for Home Infusion Therapy and Durable Medical Equipment (DME) Infusion Drugs
The Act establishes a new Medicare benefit and payment system for "home infusion therapy," which encompasses the nursing, training and education, and remote monitoring services associated with administering certain infusion drugs in a patient's home. Pharmacies and other providers furnishing these services would receive a single payment for each day of infusion administration. The Medicare reimbursement formula for DME infusion drugs (i.e., drugs that are infused through DME-covered infusion pumps) is reduced from 95 percent of Average Wholesale Price to 106 percent of Average Sales Price, bringing payment for such drugs in line with other types of Part B-covered drugs.

Expansion of Telehealth Services under Medicare
The Act includes a "sense of the Congress" that telehealth services be expanded under the Medicare program. Toward this end, CMS must report certain relevant information to Congress, including the Medicare patient populations that may benefit from increased access to telehealth and any barriers that might prevent its expansion. It also calls for a Medicare Payment Advisory Commission report to Congress on telehealth.

Delay in Implementation of Payment Cuts to the Medicare DME Fee Schedule
The Act delays by six months the expansion of DME competitive bidding to rural geographic areas that were not originally subject to competitive bidding. Full implementation of these cuts, which have already been delayed by Congress this year, will take place on January 1, 2017,

instead of July 1, 2016. The extension of the current transition period for these rate reductions comes in response to industry concerns about the impact on patient access, particularly in rural areas. HHS is also directed to study the impact of the cuts on the number of suppliers and patient access.

Conclusion. The 21st Century Cures Act is breathtaking in scope and will affect patients, researchers, pharmaceutical companies, device manufacturers, payers and health care providers in new and in some cases dramatic ways. For researchers, the Act provides new funding and may streamline confusing regulations. For pharmaceutical companies and medical device manufacturers, the Act will offer expedited approval pathways for new drug indications, regenerative therapies, and "disruptive" technologies. For health care providers, the Act aims to make electronic records simpler and more user-friendly. For payers, increased communication with drug manufacturers regarding off-label information will help in determining overall value of a drug and its placement on a formulary. While the Act holds great promise to spur health care innovation, works still remains. For example, the Act spells out that implementation of the Act will be done through the various regulatory agencies. AMCP looks forward to providing feedback and guidance to these agencies as needed. Further, the appropriation of actual funding will be in the hands of the incoming Administration and Congress and AMCP will engage when necessary and seek feedback from members and stakeholders to ensure appropriate implementation.

January 13, 2017