Insight

Coming Together for a Cure

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In a rare showing of bipartisanship, policymakers are working together to achieve a universal goal: providing for more rapid development of and increased access to life-saving health care treatments. The House Energy and Commerce Committee passed H.R. 6, the 21st Century Cures Act, 51-0 on May 21, 2015. This legislation, introduced by Chairman Fred Upton (R-MI) and Rep. Diana DeGette (D-CO), combines a number of National Institutes of Health (NIH) and Food and Drug Administration (FDA) reforms and grants new authority to both in order to assist in more timely "discovery, development, and delivery" of new medical cures and treatments. While the bill is expected to be considered on the floor of the House in June, and the Senate is working on similar legislation, competing agenda items and limited time on the congressional schedule may keep it from being considered by the Senate until the fall at the earliest. President Obama voiced support for such work during his State of the Union address in January and requested $215 million for investment in a "Precision Medicine Initiative" in his FY2016 budget released in February.

FUNDING AND OFFSETS

The legislation is estimated to cost $13.2 billion over the next decade, most of which is from increased funding of $10 billion over the next five years for the NIH. The FDA will receive $550 million in additional funding through a transfer from the Treasury's General Fund into a newly created Cures Innovation Fund between 2016 and 2020; however, this is shy of the $880 million the FDA has estimated it will cost them to implement all of the bill's provisions.

The cost of the bill will be offset through various mechanisms. Much of the bill will be paid for by gradually delaying monthly Medicare reinsurance payments to Part D plan sponsors beginning in 2020. Currently, Part D reinsurance payments are paid in advance at the beginning of each month; transitioning the timing of such payments to the first of the following month is estimated to save $5 to $7 billion over the next ten years. Limiting federal Medicaid reimbursement for durable medical equipment to Medicare rates is estimated to save $2.8 billion, and reducing Medicare payments for x-rays using old equipment by up to 20 percent will save $200 million while encouraging the transition to more modern imaging technology. Finally, sales of oil from the Strategic Petroleum Reserve—8 million barrels per year between 2018 and 2025—will generate an estimated $5.2 billion.

TITLE I- DISCOVERY

The biggest investment in the bill is through the NIH—$10 billion over the 2016-2020 period—for basic, translational, and clinical biomedical research. This appropriation will support a newly created NIH Innovation Fund and stipulates that at least some of the money should be spent on: the newly-created Accelerating Advancement Program (which requires a dollar-for-dollar funding match by a participating institution); "early stage investigators"; "high-risk, high-reward research"; and no more than 10 percent for intramural research.[1]

The legislation specifies that such research should focus on addressing unmet medical needs by expanding knowledge regarding biomarkers, precision medicine, infectious diseases, and antibiotics. The NIH is also directed to establish and continuously update a five-year biomedical research strategic plan that ensures rare and pediatric diseases and conditions remain a priority. A loan repayment program and Capstone Award grant program are intended to provide support to scientists and encourage work in the biomedical field.

While the bill is intended to assist in the development and delivery of new medical cures and treatments for all, there is particular attention given to deadly and debilitating diseases disproportionately impacting children. The NIH is directed to establish a National Pediatric Research Network and is encouraged to establish a global pediatric clinical study network. Further, appropriate age groupings are to be determined for conducting clinical research studies and presenting findings.

Several reforms are intended to ease regulatory burdens and allow for improved data collection and information sharing. Scientific findings which result from NIH-funded research will be required to be publicly shared (with exceptions so as not to violate laws protecting privacy, confidentiality, proprietary information, and intellectual property rights). A Clinical Trial Data System will be created, and all information contained in clinical trial data registries must be standardized and easily usable by the public. Health privacy regulations will be amended to allow for use of protected health information by covered entities for research purposes, provided that such information is treated in a manner similar to if it were used for public health activities.

The Council for 21st Century Cures, a nonprofit public-private partnership, would be created to coordinate the efforts of all the stakeholders working on this mission and to disseminate information relating to such activities. The Council would also be responsible for identifying gaps and recommending further collaborative and developmental opportunities.

TITLE II- DEVELOPMENT

The provisions relating to the development of new drugs and medical devices mostly impact the FDA. One interesting provision is a new requirement for the FDA to establish a framework for utilizing patient experience data in order to assess the risks and benefits of new treatments and incorporate such preferences into the approval decision-making process; essentially, this would allow for consideration of whether or not patients feel the risks and unwanted side effects of a drug are worth the potential benefits.

There is a Sense of Congress that the FDA should approve “breakthrough therapies” as quickly as possible. A provision amending current rules regarding patient access to investigational drugs would attempt to provide terminally-ill patients with a more transparent process and expanded access to therapies still awaiting approval. In order to accelerate the approval of new drugs, FDA would be required to issue guidance, based on input from the industry, for developing and qualifying biomarkers and other drug development tools which may be used as evidence demonstrating a drug’s effectiveness, as opposed to waiting for results from a lengthy clinical trial, as must be done now. FDA is instructed to issue periodic guidance to assist in the development of precision drugs and biological products. An FDA-certified third-party quality assessment program should be established to allow device companies to have their products reviewed in a more efficient manner and more quickly get products to patients. Registry data, peer-reviewed studies, and data collected in other countries would all be allowed to serve as valid scientific evidence to be considered in the FDA’s approval process for medical devices. Reporting requirements for medical devices will be eased if the Secretary determines the reports are no longer necessary, allowing for more targeted oversight of devices where it is most needed, and the humanitarian

device exemption will be increased from 4,000 to 8,000 people affected.

Drugs being developed for a rare medical condition affecting 200,000 people or fewer, known as orphan drugs, are already eligible for grant funding to assist in their development and deployment, and enjoy extra years of market exclusivity compared with non-orphan drugs. This legislation would allow for the use of evidence garnered from other drugs or clinical trial data for accelerated approval of new indications for a drug. Market exclusivity will be extended for 6 additional months for any drug which is approved for a new indication for the prevention, diagnosis, or treatment of a rare disease or condition. The Rare Pediatric Disease Priority Review Voucher (Pediatric PRV) Program, which allows for any one of a manufacturer's drugs being developed for a limited set of conditions to be reviewed for approval in just six months, rather than the typical ten months, is reauthorized. While this program, currently set to expire next year, can speed the drug approval process for certain types of conditions, the cost of a voucher is not cheap and may limit the program's effectiveness. The priority voucher fee has ranged between $2.5 and $5.5 million and must be paid in addition to the normal new drug user fee.[2] Only three Pediatric PRVs have been granted since the program was created in 2012, and both have subsequently been sold from the grantee to another pharmaceutical company.[3],[4]

Other provisions aim to help in the development of new antibacterial or antifungal drugs, particularly for treating infectious diseases with high mortality rates, and to improve access to and utilization of vaccines. Recent news stories regarding increased antibiotic resistance and the spread of measles throughout the country earlier this year highlight the importance of such provisions.

Information about drugs and devices that may be provided by manufacturers to the public is highly regulated and limited, often making it difficult for patients to fully grasp the intended uses of a drug and its potential risks and benefits. This legislation calls on the FDA to issue guidance to facilitate the dissemination of responsible, truthful, and non-misleading scientific and medical information not included on the approved label for a drug or device. Regulatory burdens relating to software used by health care companies for administrative and financial purposes—not relating to the actual provision of care—should be eased so as to allow for continued innovation and to provide clarity to software developers; such administrative software should not be regulated in the same manner as medical devices. Additional provisions relating to the meaningful use of electronic health records are included in the "Delivery" title, discussed below.

Finally, the limitation on the number of people FDA may hire, currently set at 500 employees, for biomedical research will be lifted to allow for increased staffing as necessary to implement all of these new requirements imposed on the agency under this legislation. Further, the ban on FDA employees attending scientific conferences will be lifted with the intent of keeping them informed of the most up-to-date developments in the industry.

TITLE III- DELIVERY

Under this legislation, in order for health information technology to be deemed "interoperable," it must: have the ability to transfer and allow access to all of a patient's medical record to any and all other authorized health information technologies and authorized users, including the patient; and not block information in any way, including by charging excessive fees for the transfer of information. Standards will be established by the Office of the National Coordinator for Health Information Technology (ONCHIT) through contracts with standards development organizations by 2017 regarding "vocabulary and terminology", "content and structure", the "transport of information", "security", and "service". A Health IT Policy Committee will be created to make

recommendations to the Secretary of Health and Human Services (HHS) regarding such standards, but will not have the authority to actually make changes. Any electronic health records (EHR) that do not meet all certification standards by 2019 will be decertified, which would result in payment penalties for providers using such records; however, the Secretary will have the authority to exempt providers from such penalties for up to one year in order to allow the provider time to switch to a new vendor. By 2017, guidance will be published clarifying the relationship between Health Insurance Portability and Accountability Act (HIPAA) privacy and security protections and the interoperability information sharing requirements. Hardship exemptions will be permitted on a case-by-case basis for providers unable to meet EHR meaningful use requirements. A demonstration program will allow for studying the effects of implementing similar EHR incentive payments for Medicaid providers.

One year after enactment, the Centers for Medicare and Medicaid Services (CMS) must provide Congress with information relating to which Medicare patients, particularly dual-eligibles and those with chronic conditions, may benefit most from improvements in quality care by the expansion of telehealth services; which services are best suited for delivery through telehealth; and any barriers that might prevent or impede the expansion of telehealth services. The Medicare Payment Advisory Commission (MedPAC) is instructed to make recommendations on providing payment for telehealth services under Medicare Parts A and B, based on what Medicare Advantage (MA) insurers are doing. It is recommended that states collaborate and create licensure compacts with one another in order to facilitate the delivery of telehealth services across state lines. (One such type of compact has already been signed by seven states.[5])

A new pharmaceutical and technology ombudsman will be created at CMS in order to respond to complaints and grievances of drug and device manufacturers regarding coverage of new technologies in order to help ensure timely access to life-saving treatments.

A public website will be created which will allow Medicare beneficiaries to easily search and review pricing information, including the estimated beneficiary liability, for covered services at various facilities in order to shop around and find the best price.

CONCLUSION

As this bill continues to move through the legislative process, several provisions (or the lack thereof) will continue to garner attention, and efforts will be made to revise the legislation further. Regulators continue to insist that the FDA will not be adequately funded to meet all of its new demands, thus limiting the effectiveness of the legislation. While some are thrilled at the possibility of getting new therapies to patients more quickly through the allowance of new surrogate endpoints and expedited review pathways, some patient advocates are concerned that, rather than helping patients, the legislation will allow for “drugs and devices [to be approved] faster based on weaker evidence”, putting patients’ health at risk. Pharmaceutical and device manufacturers are hoping to restore a provision which would update the antiquated regulations limiting a manufacturer’s authority to communicate information about a product on social media beyond what is specifically approved by the FDA for its product label. While vast revisions to the 340B discount drug program were floated, the most controversial amendments to the program were ultimately not included in the Committee draft; though, some are concerned those proposals may be included in future legislation.

While some stakeholders will undoubtedly be opposed to specific provisions in the legislation and more could always be done, overall this legislation marks a reasonable first step towards an important goal. Easing

regulatory burdens, accelerating access to new medical treatments, facilitating greater access to patient data in order to allow for greater coordination of care and new research opportunities for the development of new treatments, and providing for increased price transparency to spur competition and drive down costs are all much-needed changes.

[1] Intramural research is research completely funded by and conducted by the federal government in federal labs by federal employees, as opposed to research done by an outside organization funded by a federal grant.

[2] http://www.raps.org/Regulatory-Focus/News/Priority-Review-Voucher/

[3] http://www.fdalawblog.net/fda_law_blog_hyman_phelps/2014/11/fda-clarifies-its-rare-pediatric-disease-priority-review-voucher-program.html

[4] http://www.streetinsider.com/Corporate+News/Retrophin+(RTRX)+Will+Sell+Pediatric+PRV+to+Sanofi+(SNY)+in+$

[5] http://www.modernhealthcare.com/article/20150520/NEWS/150519873