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21st Century Cures

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One role of the SGIM's advocacy efforts in research and health policy involves closely following proposed legislation that might impact SGIM researchers. Since last spring, the Research Subcommittee of the SGIM Health Policy Committee has been following bipartisan legislation titled the 21st Century Cures Act.

Last July, the US House of Representatives overwhelmingly passed the 21st Century Cures Act (HR 6) by a vote of 344-77. The bill, sponsored by Representative Fred Upton (R-MI), had unanimous support (51-0) in the House Energy and Commerce Committee. The bipartisan legislation seeks to expand funding for medical research and streamline approvals and regulations for drugs and devices. In its most widely discussed provision, the bill will establish a temporary innovation fund for the National Institutes of Health (NIH) and the Food and Drug Administration (FDA) with a goal of supporting major challenges in biomedical research that have the potential to lead to therapeutic breakthroughs. The fund is a mandatory appropriation, which means that it is not subject to the annual budgets that must be approved by Congress. The fund will direct $1.75 billion per year for five years to the NIH and $100 million per year for five years to the FDA. This innovation fund will be offset (i.e. matched with cuts elsewhere in the federal budget) by future savings, with the idea that money spent now will develop treatments and cures that will lower spending on health care in the future. The legislation also proposes to increase the authorized ceiling on appropriations to the NIH by about 3% per year for three years and includes provisions to invest more resources in the next generation of scientists.

Another major focus of the legislation is to streamline the drug and device approval process at the FDA with the goal of accelerating the pace of approvals for promising therapies. Nonetheless, these provisions have led to some concerns that standards for approvals could be lowered too much. Examples of such provisions include allowing expanded approval of drugs based on biomarkers and blood tests rather than relying on improved clinical outcomes. In seeking to get effective therapies to market, the legislation would also allow consideration of drug approvals based on clinical experience and "real world evidence" from patients rather than well-designed clinical trials. Antibiotics could be approved based on animal and *in vitro* studies and very small trials in patients. Approvals of medical devices, which some already view as too lenient, would be relaxed further, allowing high-risk devices to be approved based on case studies, registries, and articles in the medical literature while also allowing changes to devices with only notification but not review by the FDA.

David Kessler, MD, a former FDA commissioner, coauthored an editorial in the *New York Times* where he described the FDA as a flexible and creative institution where new drug approvals have become the fastest in the world. He and his coauthors raised concerns that the 21st Century Cures Act could substantially lower the standards for approval of many medical products resulting in unnecessary risk of injury or death. Another provision highlighted by critics of the bill is its proposal to eliminate disclosure requirements intended to limit the influence of pharmaceutical companies on physicians, with a provision allowing physicians to receive speaking fees and gifts from companies without disclosing them as long as they are for medical education.

The bill includes several other provisions intended to accelerate the development of new therapies.[1] First, it seeks to improve access, sharing, and use of de-identified health data generated in research and clinical settings with a goal of increased research collaboration. Second, it removes regulatory uncertainty for the development of new medical apps for cell phones and devices that are designed to improve clinical care with a goal of speeding the creation and adoption of these tools. Third, it provides new incentives for the development of drugs for rare diseases.

Other specific pieces of the House legislation relevant to SGIM researchers include requirements that NIH issue a strategic plan and also work on streamlining the grant process for researchers and decrease administrative burdens. The legislation also improves loan repayment programs for NIH researchers and encourages the NIH director to expand programs for young emerging scientists at NIH. It also seeks to refocus national efforts on making information technology systems interoperable and to increase price transparency at the site of service for Medicare beneficiaries.

Lamar Alexander (R-TN) and Patricia Murray (D-WA) from the Senate Health, Education, Labor and Pensions Committee have been working on similar legislation, currently identified as the Senate Innovation Initiative. The goal of this

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bipartisan initiative is "to examine how we get drugs, devices and treatments from the discovery process through the regulatory process into our medicine cabinets and doctors' offices."[2] The Senate bill is expected to be released and discussed this year. Although the legislation was reportedly on a "parallel track" with the House bill, the Senate bill will likely be different. Senate Republicans have expressed a preference for funding that remains discretionary instead of mandatory. They are also sure to be examining the language addressing regulation closely. Moreover, in light of the recent budget deal passed in December 2015, which provides a $2 billion (6.6%) increase in NIH funding, the Senate may feel less urgency to push forward legislation whose primary aim is to increase NIH funding.

There are certainly things to like about 21st Century Cures legislation, particularly the promise of direct funding to the NIH that is not subject to appropriations. That said, it is not yet clear if this funding will focus solely on drug discovery or if it may also be used to fund health care delivery research. The expansion of de-identified health data also holds great promise for SGIM researchers, as does expanded loan repayment programs, but concerns about other provisions remain. The Health Policy Committee Research Subcommittee will be watching closely as the Senate crafts and deliberates its legislation in the coming year.

References

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