NIH's thrust for more drugs and cures[1,2] is echoed in Congress as +$9B and faster approval

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The 21st Century Cures Act could be a harmful step backward

by Susan F. Wood and Diana Zuckerman, WP, November 19, 2105

Precision medicine is the next big thing in health care, and it's also one of the few health goals that Congress and the White House agree on. But while we await treatments targeting the precise genetic makeups of individuals and diseases, medical researchers still are not paying enough attention to the most important kinds of differences among patients: those of sex, age and race.

A clear example of this disconnect is the 21st Century Cures Act, which was passed overwhelmingly by the House of Representatives and is being scrutinized by the Senate. The stated goal of the bill is wonderful: to stimulate the development of new cures for a range of diseases. Many medical schools and patient organizations are supporters, since the proposal would provide almost $9 billion more for the National Institutes of Health — including a boost for precision medicine. The 360-page bill offers other potential benefits as well. But an immediate impact would be to ignore how differences between men and women and younger and older patients influence the safety and effectiveness of many medical products.

Throughout the 20th century, most medical research was conducted on relatively young, healthy men. In recent years, researchers have realized that treatments often affect women and older patients differently than men or younger patients. These differences can affect safety and effectiveness. The sleeping pill Ambien, for example, makes women drowsier for longer periods than it does men, putting them at risk if they drive the next morning. Since most medications are taken by people older than 65 and women of all ages, it makes sense to analyze the effects of age and sex on the drugs' safety and effectiveness before they can be sold.

But the 21st Century Cures Act is based on the assumption that there will be more cures if drugs and devices are studied more quickly by testing them on fewer patients — in some cases, on just a handful. Unfortunately, such studies would be too small to allow safety and effectiveness findings to be broken down for subgroups such as men, women, young adults and seniors.

This embrace of smaller, more preliminary studies could drastically lower scientific standards. When fewer people are studied, it is more likely that a drug will seem safe and effective even if it has dangerous side effects for many patients — who may not have been included in those small studies.

In addition to allowing smaller studies, the House bill would encourage the Food and Drug Administration to determine a drug or device's effectiveness based on "clinical experience," which the bill defines to include the experience of one or more doctors or patients. Scientists call these anecdotes and note that just because one doctor has had success treating a few patients with a particular drug does not prove it is either safe or effective. Worse, the bill specifies that after studying only small groups of patients, drug manufacturers could sell a new treatment to anyone, even if the patient was not among the types studied. In fact, hospitals would be paid extra to make it financially feasible to prescribe more expensive new drugs to Medicare patients, even if the drugs were never studied on patients older than 65 (the age of most Medicare patients).

Similarly, lifesaving medical devices, such as heart valves, could be approved based on case histories, which are written descriptions of the experiences of just one or two patients. They are unlikely to be good predictors of how a treatment helps or harms most patients.

The recalls of drugs such as Vioxx and devices such as metal-on-metal hips in recent years have made clear that inadequate testing can produce ineffective and harmful products. And since new drugs tend to be much more expensive than older ones, the costs of widely used, unproven medical products can be enormous in both human and economic terms.

These sections in the 21st Century Cures Act go in the opposite direction of the push for precision medicine and what we've learned about differences between male and female and older and younger patients. The General Accountability Office has concluded that the NIH needs to make a priority of analyzing data related to sex differences. Just three years ago, the House and Senate overwhelmingly passed legislation that directed the FDA to ensure that men and women, old and young, are studied, with results analyzed to see which treatments are safest and most effective for whom. Why is Congress undermining that law?

Congress should surely increase funding for research to find 21st-century cures, but the price should not be returning to early-20th-century standards, when unproven medical products were widely available and often put all Americans at risk.

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