Easing approvals for breakthroughs may have consequences

By ACSH Staff — March 29, 2012

Patients and pharmaceutical companies alike have been pressing the FDA to hasten the approval of new medications that could provide important innovations for treating devastating diseases. Now, Congress is considering a bill, the Advancing Breakthrough Therapies for Patients Act, that would accelerate the process of developing and reviewing what are considered to be breakthrough drugs.

A breakthrough drug is considered any medication for a serious or life-threatening condition that has been shown to provide a significant improvement over current therapies in preliminary clinical trials. Under this bill, the FDA would provide guidance in the development of such drugs and would allow for clinical trials to use fewer patients over a shorter period of time. According to one of the bill’s authors, Sen. Orrin Hatch (R-UT), This bipartisan bill ensures that when it comes to treating patients suffering with cancer or other devastating illnesses, science and patient care will not be slowed down by government red tape.

Other measures to speed up drug approvals are also under review by the House and Senate, and are modeled on the successful efforts that allowed for the swifter development of drugs for HIV/AIDS a process that used surrogate endpoints to measure effectiveness. In a controversial proposal, former FDA commissioner Andy von Eschenbach suggested that the FDA should consider only safety when approving drugs, measuring efficacy later, through post-marketing trials.

In response to this latest proposal, ACSH's Dr. Josh Bloom sees both pros and cons. Moving efficacy trials to the post-marketing phase would, on the one hand, allow us to have new drugs faster, he says. But we will certainly see more problems with them. And while he thinks that working toward speedier approval of drugs for unmet medical needs is admirable, they are kidding themselves if they think this is some sort of brilliant new paradigm that will duplicate the AIDS progress, he notes. That was a massive 15-year war that included companies that are no longer in existence, not to mention those that have severely cut their research and development staff. To expect a repeat of this is naïve and unrealistic.

ACSH's Dr. Ruth Kava adds that the downside of this proposal is that people may be prescribed drugs that are safe but ineffective. How will that improve their health?