PATIENT SAFETY BLOG

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Victims of Rare Diseases See New Focus on "Orphan Drug" Research

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A recently introduced bill in the House of Representatives seeks to update legislation for “orphan” diseases and drugs. “Orphan” status denotes disorders that are extremely rare—generally afflicting 6,000 or fewer patients.

Pharmaceutical companies have no financial incentive to develop drugs and treatments for them because there aren’t enough users to pay the costs and sustain the consumer market. In order to encourage the development of drugs and other treatments for orphan diseases, the government provides incentives it doesn’t grant to more common disorders, such as easier and faster FDA approval, and extended periods for developing companies to market the drugs exclusively.

Another advantage lawmakers hope to grant to orphan drug development is the routine use of a “surrogate endpoint.” Before drugs are given FDA approval, generally they must pass rigorous clinical muster; they must be tested in studies that identify the risks and side effects as well as the benefits of the treatment using meaningful numbers of real subjects. A surrogate endpoint substitutes for a real, observable, provable clinical result—the surrogate endpoint doesn’t necessarily have a guaranteed relationship with a clinical result like actual cure or extension of life, but it has a “biomarker,” according to the National Institutes of Health, that researchers can accept as indicative of clinical benefit, harm or the lack thereof.

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Surrogate markers are used if the number of subjects that might be suitable for a clinical trial is so small that it wouldn't result in a statistically significant result. It would be impractical to conduct a clinical trial in such circumstances, but the people who suffer—the people stricken with an orphan disease—still need treatment, so the surrogate endpoint, the more relaxed standard, is critical to their well-being.

The proposed law is called the Unlocking Lifesaving Treatments for Rare-Diseases Act, or ULTRA. Specifically, according to the FDA Law Blog, it would permit the FDA to approve an application for a drug designated both as an orphan drug and as a fast-track product by allowing the surrogate endpoint standard.

Currently, the FDA is able to "fast track" approval for a product that addresses a serious or life-threatening illness, and when it provides a meaningful therapeutic benefit to patients beyond the existing treatments.

If a product meets the criteria, the FDA may grant marketing approval based on a demonstrated effect on a surrogate endpoint reasonably likely to predict clinical benefit. Also, the manufacturer must commit to completing studies after marketing approval that confirm its benefits.

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