Medical Xpress

Federal laws have enhanced pediatric drug studies

February 29 2012

Federal laws that motivate or require drug and biologic developers to conduct pediatric studies have yielded beneficial information to guide the use of medications in children, says a new report by the Institute of Medicine. Still, studies involving children continue to be limited, especially in certain areas such as medications' use in newborns and long-term safety and effectiveness in children. The report identifies ways that Congress and the U.S. Food and Drug Administration could further improve the utility of clinical information obtained from pediatric studies, including expanding innovative strategies to research drugs and biologics in children, using FDA's authority to require long-term pediatric studies of possible safety risks, and giving FDA flexibility to impose sanctions for unreasonably delayed studies.

Conducting research with children is inherently more difficult than with adults. Pediatric patients also offer drug companies a much smaller market and potential economic return. Clinicians often treat children with drugs that have been approved for use with adults but have not been studied with children, even though the drugs may have different risk-benefit profiles for pediatric patients. Recognizing a shortage in knowledge of how medications affect children, Congress has sought to increase pediatric studies of medications under two laws, the Best Pharmaceuticals for Children Act (BPCA), which offers companies economic incentives to study medications in children, and the Pediatric Research Equity Act (PREA), which requires such studies in specific situations. Both laws are due for reauthorization this year. As specified by Congress, FDA asked IOM to review certain aspects of studies that

have been conducted under the laws.

The laws have had positive effects, noted the committee that wrote the report. They have spurred the development of helpful information about the uses of therapies in pediatric patients and have expanded access to information from these studies, including FDA reviews of clinical data submitted by study sponsors. FDA reviewers generally have been thorough in assessing the efficacy of drugs, evaluating adverse events, and reaching conclusions about the safety profile of drugs studied in children, the committee concluded.

To further enhance the impact of the legislation, the report suggested that FDA consider more frequent use of its authority to require sponsors to undertake long-term follow-up studies after products have been approved for market. Because children's bodies and minds are continuing to develop and because some therapies for chronic conditions may be used for many years, pediatric studies of drug safety and effectiveness over the long term are important, but they are not commonly required.

Newborn children, especially premature infants, are particularly vulnerable to drug-induced harm and are especially difficult to study. Many drugs used with neonates are older medications, and the incentives and requirements of BPCA and PREA do not apply to them. To promote studies of newer and more widely used drugs that have not been adequately evaluated in these youngest patients, Congress could provide additional resources for short- and long-term neonatal drug studies through the existing BPCA program at the National Institutes of Health, the report says.

Congress extended the incentives of BPCA to biological therapies in 2010. It is too early to assess the effects of the law on this therapeutic category, but the committee found that most biologics either have been

or are being studied with children.

Although companies have stepped up their efforts to conduct research involving children in response to BPCA and PREA, it can still take a long time for needed information to be generated, the report noted. To improve the timeliness of pediatric studies, Congress could specify that sponsors submit their plans for pediatric studies at the end of phase II trials involving adults. To address concerns that FDA has limited ability to require completion of studies in children, Congress could consider providing FDA with more flexibility to impose sanctions, including monetary penalties, for unreasonably delayed studies.

Provided by National Academy of Sciences