THE OFFICIAL NEWSMAGAZINE OF THE AMERICAN ACADEMY OF PEDIATRICS
AAP News

FDA highlights imperative to obtain evidence through pediatric clinical trials

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While it is challenging to conduct clinical trials in children, pediatric trials are essential to establish the safety and effectiveness of drugs in children. Without evidence from pediatric trials, pediatricians must rely on adult studies or anecdotal data to make treatment decisions about using drugs off-label in individual patients.

Evidence of safety and effectiveness obtained from adults may provide information on a drug's potential effects in pediatric patients but generally is not adequate to understand safe and effective use in the entire pediatric population. If a disease is similar in adults and pediatric patients (or an age cohort of pediatric patients, such as adolescents), efficacy observed in adults may translate to pediatric patients. In general, however, information about dosing and safety still will be needed. If the disease presents or progresses differently in children, data from pediatric patients may be needed to confirm drug efficacy.

Pediatric dosing for a medicine may differ from adults not only due to body weight and proportionality differences but also due to immaturity in organ systems responsible for the drug's absorption, distribution, metabolism and excretion.

Similarly, a drug's safety may differ based on age-related vulnerabilities, such as effects on growth, pubertal maturation and neurocognitive development that are not discernable in adults.

Pediatric trials should not be delayed unless there is a compelling scientific rationale or safety concern. Delays can result in prolonged off-label use of products in children without adequate information about dosing, safety and efficacy in pediatric patients.

Perceptions that children should not be enrolled in clinical trials impact study feasibility. Regulatory safeguards are in place for children involved in research to ensure risks are limited and higher risks are adequately justified by the potential for benefit. Preliminary adult data can be used to assess the risk/benefit profile to inform pediatric enrollment in a clinical trial.

Ultimately, the decision to proceed with a pediatric trial relies on scientific, clinical and moral judgment and must be grounded in a desire to advocate for the well-being of children.

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