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FDA outlines ethical framework for including children in clinical trials

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New draft guidance from the Food and Drug Administration (FDA) describes an ethical framework for ensuring children are safeguarded when participating in clinical trials (http://bit.ly/3zwJ4J8). The framework reflects the FDA's human subject protection regulations, which outline limits on the risks to which children can be exposed in clinical investigations involving FDA-regulated products.

The FDA also has developed a guidance snapshot (https://www.fda.gov/media/161749/download), which provides an overview of the draft guidance. Clinical investigators, drug developers and institutional review boards can use the snapshot to proceed step-by-step through the ethical considerations and regulatory framework for including children in a clinical trial while referencing the guidance for details.

Once the scientific need for enrolling children in a clinical trial has been established, the potential risks of clinical trial participation need to be assessed. Most clinical trials involving investigational medical products present risks that exceed the lower-risk thresholds described in the FDA's regulations. When the risks are higher, the prospect of direct benefit to the child enrolled in the clinical trial and the balance of potential benefits and risks must be considered.

To assess the risks and potential benefits of a clinical therapeutics trial, data are needed to support the safety, efficacy and dosing of the medical product for children.

Donna Snyder, M.D., M.B.E., senior pediatric ethicist in the FDA's Office of Pediatric Therapeutics, shared insights about the assessment of potential benefit in a clinical trial in a guidance-related podcast (https://www.fda.gov/media/161781/download).

"The level of evidence to support prospect of direct benefit may vary based on the situation," Dr. Snyder said. "For rare diseases that are exclusively or primarily seen in children, or are more severe in children, nonclinical data alone might support prospect of direct benefit, but for conditions that exist in adults and children, we may want some adult data before initiating pediatric studies."

Additional considerations when preparing for a clinical trial involving children include conducting risk assessments for all research-related interventions or procedures in the trial (known as a component analysis of risk) and ensuring parents/guardians and children are informed about the trial via ongoing informed consent and assent processes.

Ethical standards like those described in the draft guidance are essential for sustaining clinical research by promoting integrity, quality and fairness, and building trust in the value of clinical research to advance development of medicines for children.

Comments on the draft guidance "Ethical Considerations for Clinical Investigations of Medical Products Involving Children" can be submitted until Dec. 27 at https://www.regulations.gov/document/FDA-2022-D-0738-0002.