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Will Newly Proposed FDA Guidance Discourage Pediatric Drug Development?

Josie Cooper

The Alliance for Patient Access, founded in 2006, is a national network of physicians dedicated to ensuring patient access to approved therapies and appropriate clinical care. AfPA accomplishes this mission by recruiting, training and mobilizing policy-minded physicians to be effective advocates for patient access. AfPA is organized as a non-profit 501(c)(4) corporation and headed by an independent board of di[1]rectors. Its physician leadership is supported by policy advocacy management and public affairs consultants.

In 2012, AfPA established the Institute for Patient Access, a related 501(c)(3) non-profit corporation. The Institute for Patient Access is a physician-led policy research organization dedicated to maintaining the primacy of the physician-patient relationship in the provision of quality health care. In furtherance of its mission, IfPA produces educational materials and programming designed to promote informed discussion about patient access to approved therapies and appropriate clinical care.

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The U.S. Food and Drug Administration could soon become more selective about which companies receive six months of patent exclusivity in exchange for developing pediatric drugs. If finalized, the guidance could have the unintended consequence of discouraging investment in drugs for infants and children.

The Role of Patent Exclusivity in Pediatric Drug Development

Manufacturers are currently required under the Pediatric Research Equity Act to conduct pediatric studies to receive FDA approval for certain drugs. Those same studies can also make them eligible for six months of patent exclusivity, which they can apply to any drug they manufacture.

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This approach incentivizes companies to develop and invest in drugs for infants and children, which are generally difficult to develop and, therefore, rare.

For decades, physicians often treated infants and children off-label, adjusting the dosage of medications studied and approved for adults rather than children. Policy reforms have helped turn the tide, including offering patent exclusivity and dramatically increasing the number of clinical studies dedicated to pediatrics. As a result, more medications are being developed and approved specifically for infants and children, and new pediatric labels are being added to existing drugs.

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Proposed Changes

However, new guidance proposed by the Food and Drug Administration would change the rules on exclusivity and pediatric drug development.

Specifically, it would ask manufacturers to conduct additional studies beyond those already required by the Pediatric Research Equity Act to obtain exclusivity. The draft guidance urges manufacturers to take a broader approach by conducting additional studies on children of varying ages. It also directs manufacturers to test multiple indications before sending the drug through the FDA approval process.

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Impact on Pediatric Drug Development

The FDA's guidance is well intended. Undoubtedly, generating more research on pediatric drugs would build a body of knowledge that benefits clinicians and patients alike. Nevertheless, raising the bar could also impact whether companies attempt to develop much-needed drugs for the pediatric population. Additional research demands additional time, human resources, and investment. Not all manufacturers will be ready or able to dedicate these resources.

Most physicians and parents would argue that they need more, not fewer, medications developed and approved specifically for infants and children. By making exclusivity, the driving incentive behind pediatric drug development, more challenging, the FDA may unintentionally encourage manufacturers to focus their attention and research dollars elsewhere.

This could leave young patients and their healthcare providers behind.

The proposed guidance was open to public comment through July 17, after which the Food and Drug Administration finalized it.

References:

1. https://www.fda.gov/drugs/information-consumers-and-patients-drugs/drug-research-and-children

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