An Update on Infants and Clinical Trials Diversity

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The Alliance for Patient Access (allianceforpatientaccess.org), 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 directors. Its physician leadership is supported by policy advocacy management and public affairs consultants. In 2012, AfPA established the Institute for Patient Access (IfPA), a related 501(c)(3) non-profit corporation. In keeping with its mission to promote a better understanding of the benefits of the physician-patient relationship in the provision of quality healthcare, IfPA sponsors policy research and educational programming.

Earlier this summer, the Food and Drug Administration (FDA) issued draft guidance entitled, "Enhancing the Diversity of Clinical Trial Populations — Eligibility Criteria, Enrollment Practices, and Trial Designs Guidance for Industry." This guidance was not focused on any one age group - and not specifically on the enrollment of the infant population - but about broadening eligibility criteria to improve retention of clinical trials patients and access to clinical trials.

Where are we now with infants and participation in clinical trials?

The NIH's Eunice Kennedy Shriver National Institute of Child Health and Human Development notes that four million infants are born each year in the United States. Of those, about 380,000 are born each year prematurely, per information available from the March of Dimes. This is important because premature birth (birth before 37 weeks of pregnancy) and its complications are the number one cause of death of babies in the United States. Yet, new treatment options for newborns are poorly lagging. In fact, the last currently available drug tested and approved specifically for newborns was approved about 20 years ago. (1)

In 2018, the Coalition for Clinical Trials Awareness (CCTA) dedicated its annual Clinical Trials Awareness Week (April 30-May 4, 2018) to the need for age diversity in research on new drugs. Andrew Rosenberg of the Newborn Health Initiative brought an economic perspective to the issue. About 200,000 newborns require admission to NICU for prematurity treatments, Rosenberg explained, costing about $26 billion each year. There are also some conditions for which drugs are needed in neonates that do not occur in older children and adults, he noted, heightening the need for new drug discovery, and thus clinical trials conducted specifically for infants. (2)

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Patient groups acknowledge the inherent difficulties in conducting clinical trials on infants, including "pharmaceutical companies and institutional review boards continue to shy away from studying infants because they are fragile, cannot spare many blood samples, and are vulnerable to permanent injuries — injuries that, in the past, have been awarded large malpractice verdicts." (3)

However, researchers and pediatricians in the health care community agree that there is no excuse not to study infants and proper dosage for this vulnerable population.

Infant-Focused Policy Solutions

One solution discussed has been components of the legislation "Promoting Life-Saving New Thera-pies for Neonates Act of 2017" (which has yet to be reintroduced in the 116th Congress). It pro-

posed to amend the Federal Food, Drug, and Cosmetic Act to require the FDA to award the sponsor of a new drug or biological product for the treatment of newborns a neonatal drug exclusivity voucher upon approval of the medication. A neonatal drug exclusivity voucher would be a transferable voucher for a one-year extension of all existing patents and marketing exclusivities for a brand name medication. (4)

Advocates generally support providing incentives such as exclusivity as a way to incentivize re-search that targets treatments tailored to difficult to reach populations - such as newborns.

Another Solution

The draft guidance also focuses in part on making trials less burdensome on participants. In the case of infants, this would have to apply to patients and their families. Trial designs and recruitment should focus on reimbursement of necessary expenses, travel times, locations, and frequency of visits, and further development of online tools to ease the burden of clinical trial participation. One often hears of families with newborns that require additional clinical care or hospitalization, while also managing childcare for multiple children and concerns with balancing a dual-income household. These considerations would provide both financial and logistical stability as families determine if clinical trial participation is right for their child and family.

Policies must be developed to encourage research in treatments for newborns, including finding ways to expand clinical trials targeting these patients. The status quo will have serious ramifications for the health care available to future generations.

Public comment to the FDA's draft guidance may still be submitted at: *https://www.fda.gov/regulatory-information/search-fda-guidance-documents/enhancing-diversity-clinical-trial-populations-eligibility-criteria-enrollment-practices-and-trial*.

References:

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4. H.r.2641 - 115th Congress (2017-2018): Promoting Life .https://www.congress.gov/bill/115th-congress/house-bill/2641 (accessed August 08, 2019).

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