



CLINICAL
TRIALS
AWARENESS
2018
WEEK

Understanding the Lack of Clinical Trials for Infants & Children

Newborns face an innovation desert when it comes to medical therapies. It's been nearly 20 years since a currently available drug was tested and approved specifically for the newborn population.

The Coalition for Clinical Trials Awareness and the National Coalition for Infant Health co-hosted a Washington, DC policy panel discussion on May 1, 2018 to explore the issue. Conducted at the United States Capitol, the event invited participants to consider why newborns don't have more options – and what advocates, parents and policymakers can do about it.



David Charles, MD
Coalition for Clinical
Trials Awareness



Andrew Rosenberg
Newborn Health Initiative



Jaszianne Tolbert, MD
Children's Mercy Hospital's
Experimental Therapeutics in
Cancer program



Joe Murray
debra of America

Summary

About 4 million babies are born each year in the United States, David Charles, MD, noted, yet 90 percent of the medications they take have never been tested for children and neonates. Calling the enrollment of children and infants in clinical trials “woefully inadequate,” Dr. Charles emphasized that the body metabolizes and responds to medications differently at different ages. Dr. Charles conducts clinical research for Parkinson’s patients at Vanderbilt University.

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. Prematurity is the leading cause of newborn mortality; the second leading cause of infant mortality. There are also some conditions for which drugs are needed in neonates that don’t occur in older children and adults, Rosenberg noted, heightening the need for clinical trials conducted specifically for infants.

“Kids get hand-me-down clinical trials,” explained JasziAnne Tolbert, MD, who specializes in pediatric hematology-oncology. Dr. Tolbert added, “We see kids as little adults.” The reality is far different.

Dr. Tolbert illustrated the importance of clinical trials for newborns and children by telling the story of Bailey. Doctors said, “Love your daughter as much as you can,” Dr. Tolbert explained. But after one month in a clinical trial, Bailey was in disease remission. Her mom thought the doctor was lying, Dr. Tolbert recalled, explaining, “She had never heard the words, ‘Your daughter is cancer free.’”

Joe Murray of debra of America conveyed the experiences of his daughter, Ella, who was born with a rare genetic skin disorder known as epidermolysis bullosa. “We were told there was no hope,” Murray recalled of his daughter’s first month of life. Murray depended upon patient advocacy groups, online research and webinars to learn about the disease and to find clinical trial opportunities for his daughter.



Steps Forward

The panel's discussion arrived at three key components for changing the landscape for neonates and clinical trials.



Congressional Action. The Promoting Lifesaving New Therapies for Neonates Act (H.R. 2641) would provide incentives for industry to invest in the development of neonate-specific drugs.



Patient Narratives. Joe Murray and Dr. Tolbert emphasized the power of storytelling in conveying the importance of clinical trials for neonates. “It doesn’t have to be a magic wand,” Murray explained, “It can start with one child, one story.”



Public-Private Partnerships. Industry-government collaborations can produce valuable research, panelists agreed. They can also play a critical role in heightening public awareness about the critical need for more treatments and clinical trials geared toward neonates.

Learn more about Clinical Trials Awareness Week 2018 at www.cctawareness.org.



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NCfIH National Coalition
for Infant Health

Protecting Access for Premature Infants through Age Two

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