WORLDWIDE CLINICAL TRIALS

OVERCOMING THE CHALLENGES OF PEDIATRIC CLINICAL RESEARCH

Children are severely underrepresented in the world of clinical research.

Children make up **27%** of the world's population.

ONLY 16.7% of World Health Organization-registered clinical trials are pediatric.[1]

ONLY 20% of approved medications included label information for pediatric use before the U.S. Food and Drug Administration initiated incentive programs for pediatric clinical studies.[2]

NOW, AT LEAST 50% of all drug products may still lack labeling with pediatric information.[3]

This is bad news for kids, even though they have long relied on and often benefitted from off-label use of the vast majority of drugs prescribed to them.

Drugs without proper clinical research in pediatric patients greatly raises the risk of unexpected adverse events, suboptimal therapy, and inappropriate dosing.[4]

211,209 visits to the emergency room were by **children 12 or younger** due to adverse drug reactions in 2008 alone.[5]

FDA incentive programs have removed some of the hurdles to completing these critical pediatric trials.

Companies can receive 6 extra months of marketing exclusivity for completing pediatric studies requested by the FDA.[5]

The FDA granted marketing exclusivity to 189 drug sponsors extending studies to children from 1998-2012.

57% of the 189 drugs granted exclusivity received new or expanded pediatric indications, and **92%** received new pediatric labeling information.[6]

The Foundation for the National Institutes of Health offers some trial funding for the study of off-patent drugs in children.[7]

To Design an Effective Pediatric Clinical Trial, You Must Consider:

- Additional regulatory oversight, consent/assent procedures, and trial ethics requirements
- Dose-finding with small blood/serum volumes and minimal clinical data
- Equipment, medical techniques, and staff prepared to appropriately manage pediatric patients
- Dosing methods that are safe, palatable, and easy to take
- Growth and development stages, such as puberty, tooth development, liver maturity, metabolic differences, and more
- Quality of life, schedule, and number of interventions
- Techniques for recruitment, retention, communication, and comfort for families
- Multiple age groups, as appropriate:
  - Preterm infants
  - Full-term neonates up to 28 days of age
  - Infants and toddlers under the age of 2
  - Children aged 2-11
  - Adolescents aged 12-18

MAKE YOUR PEDIATRIC CLINICAL RESEARCH A SUCCESS WITH **WORLDWIDE CLINICAL TRIALS**

Our experts offer uncommon support in the clinical, technical, and regulatory requirements underpinning pediatric clinical development programs.

Our successes in rare diseases, study site development, and operational agility can transform the experience for our clients and the families involved in our studies.

Find out how we turn our expertise and unflagging devotion to quality into seamless, effective pediatric research programs. Learn more about **Worldwide's uncommon commitment to pediatric clinical trial solutions**.

CONTACT US

WORLDWIDE.COM | +1 610 964 2000

WORLDWIDE CLINICAL TRIALS

REFERENCES

1. Joseph PD, Craig JC, Caldwell PHY. Clinical trials in children. Br J Clin Pharmacol. 2015;79(3):357-369.
2. U.S. Food and Drug Administration. "Drug research and children." U.S. Food and Drug Administration. Updated 05/04/2016. Accessed online 04 April 2019: https://www.fda.gov/Drugs/ResourcesForYou/Consumers/ucm143565.htm.
3. Committee on Drugs; American Academy of Pediatrics. Policy Statement: Off-label use of drugs in children. Pediatrics. 2014;133(3):563-567.
4. Department of Health and Human Services, Food and Drug Administration. Regulations requiring manufacturers to assess the safety and effectiveness of new drugs and biological products in pediatric patients. Final Rule; 21 CFR Parts 201, 312, 314, 601. Docket No. 97N-0165.
5. Clarke PE, Office of Communications. "FDA encourages pediatric information on drug labeling." U.S. Food and Drug Administration. Updated 01/08/2016. Accessed online 04 April 2019: https://www.fda.gov/Drugs/ResourcesForYou/SpecialFeatures/ucm254072.htm.
6. Wharton GT, et al. Impact of pediatric exclusivity on drug labeling and demonstrations of efficacy. Pediatrics. 2014;134(2):e512-e518.
7. Crosse M. Pediatric Drug Research: The study and labeling of drugs for pediatric use under the Best Pharmaceuticals for Children Act. Testimony before the Subcommittee on Health, Committee on Energy and Commerce, House of Representatives. U.S. Government Accountability Office. 2007 May 22. GAO-07-898T. Accessed online 04 April 2019: https://www.gao.gov/assets/120/116714.pdf.

Last Update: 09042019