Addressing the Growing Need for Medications for Children

Pediatric development plans are a requirement for all new medicines, indications, dosing forms, regimens, and routes of administration, and pediatric clinical research is on the rise. Additionally, all applications for marketing authorization for new medicines must include the results of studies in children as described in an agreed-upon pediatric plan, unless the medicine is exempt because of a deferral or waiver.

Understanding the Diversity of Pediatric Clinical Trials

At Synteract, we are at the forefront of working with sponsors in pediatric drug development with broad experience in 310+ studies across 6 continents, in 62+ countries.

Conducting Pediatric Clinical Trials with Heart

Pediatric drug development requires special care in recruitment to address ethical, scientific, and logistical challenges. We understand the compassionate, specialized clinical and regulatory expertise required when working with vulnerable pediatric populations and their families. We have experience working with international pediatric networks, opinion leaders, patient advocacy groups, and regulatory bodies, with many of our executives actively involved in the industry, and can capitalize on relationships with global partners invested in this patient population.

We can assist with all aspects of pediatric drug development, including medical and regulatory strategy; trial design; creation, submission, follow-up and updates of PSPs and PIPs, and more. Our clinical experts will guide you through evolving global regulations and data requirements each step of the way.

A - Respiratory: 16%
B - Endocrinology: 14%
C - Gastroenterology: 10%
D - Infectious Disease: 10%
E - Hematology: 9%
F - Dermatology: 7%
G - Metabolic: 7%
H - Dental: 5%
I - Neurology: 4%
J - Nutrition: 3%
K - Oncology: 3%
L - Cardiology: 2%
M - Ophthalmology: 2%
N - Psychiatry: 1%
O - Cosmetic: 1%

Highlights of our history:

310+ PEDIATRIC CLINICAL TRIALS
25+ INDICATIONS
Pediatric Drug Development: Regulatory Updates to Know

All applications for marketing authorizations for new medicines must include the results of studies in children as described in the pediatric plan, unless the medicine is exempt because of a deferral or waiver. Pediatric legislation internationally has led to better medicines for children, but gaps still exist that regulatory agencies want to close.

How will pediatric drug development requirements impact sponsors?

In August 2017, the United States passed the FDA Reauthorization Act and with it the Research to Accelerate Cures and Equity for Children Act (RACE). RACE will eliminate exemptions and improve opportunities for cancer drugs development for children:

- Requiring companies to do PREA studies in children when the molecular target of their drug is relevant to children’s cancer
- Ending exemption of PREA obligations for cancer drugs with orphan designations if the molecular target of the drug is relevant to children’s cancer

In August 2018, the FDA will publish a list of molecular targets substantially relevant to growth and progression of pediatric cancer. It will also publish a list of molecular targets for which pediatric study requirements will be automatically waived.

Other recent actions include:

- Congress reauthorized through 2020 the Rare Pediatric Disease Priority Review Voucher program in the 21st Century Cures legislation.
- In the EU, the Commission report on 10 years EU Pediatric Regulation (October 26, 2017) showed encouraging impact of the Pediatric Regulation overall, though the regulation appears most effective when adult and pediatric needs overlap.

Global Progress Continues

Fewer advances have been made in diseases that are unique to children. While some instances of over- or under-compensating drug developers with financial rewards exist, overall benefits seem to outweigh costs.

Therefore, the European Commission does not currently recommend re-opening the leg-

Editor’s Note: Dr. Martine Dehlinger-Kremer is an observing member of the Coordinating Group of Orphan-EMA, the European Network of Pediatric Research at the European Medicine Agency, and a number of Working Parties of Orphan-EMA. She is also President of EUCROF, the European CRO Federation, and a Board Member of Enpr-EMA. Contact Synteract at +1 760-208-8209 to leverage its three decades of pediatric experience to advance your clinical trial.

Synteract.com

Synteract is an innovative, full-service contract research organization supporting biopharmaceutical companies across all phases of drug development to help bring new medicines to market. Synteract has conducted 4,000 studies on six continents and in more than 60 countries, working with more than 26,000 investigative sites and 750,000 patients. It has contributed to more than 240 product approvals. Synteract offers a notable depth of expertise in oncology, general medicine, dermatology, and neuroscience indications, as well as rare and orphan, pediatric, and immunotherapy studies.

For more information on how we can support your clinical trial, please visit www.synteract.com/Therapeutic-Expertise/Pediatrics or ContactUs@synteract.com.

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