synteract®
BRINGING CLINICAL TRIALS TO LIFE

Broad-based Pediatric Experience to Support the Littlest Ones

Highlights of our history:

260+ PEDIATRIC CLINICAL TRIALS | 25+ INDICATIONS

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 ~100 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%

As seen in the June 2018 issue of PharmaVoice

Pediatric Drug Development: Regulatory Updates to Know

All applications for marketing authorization 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 by:

- 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 legislation. It will evaluate pediatric and orphan regulations to better understand why rewards do not seem to be driving development for rare diseases. Findings are expected to be delivered in 2019, enabling the next Commission to make informed decisions about policy options.

Dr. Martine Dehlinger-Kremer
VP, Pediatric Development,
Synteract

Meanwhile, the European Commission and EMA have started to streamline application and implementation of the regulation, including making changes to deferrals and revisiting PIP processes and other aspects.

A revised and revoked class waivers list will be effective in July 2018. Applications for new medicines or variations of marketing authorizations will be validated against it. Waivers, specifically in oncology, will no longer be automatic. Regulators will expect companies to have considered product mechanism of action and pediatric needs prior to decision.

Regarding multi-stakeholder discussion of pediatric needs, the EU Commission and EMA held a workshop with patients, academia, healthcare professionals, and industry on March 20, 2018. Potential improvements to implementing the regulation were discussed. An action plan addressing challenges will be published mid-2018. The EMA, EU Commission, and stakeholders will need to commit to implementation within two years.

ICH E11 (R1) "Clinical Investigation of Medicinal Products in the Pediatric Population," enforced in February 2018, aims to advance pediatric research globally. Addendum R1 reflects latest technical, scientific, and regulatory approaches and recognizes topics where consensus had not been achieved, including:

- Ethical considerations
- Age classification and pediatric subgroups
- Pediatric formulations
- Common scientific approaches to aid discussions in different regions
- Pediatric extrapolation and introduction of modeling and simulation
- Practicalities in design and execution of trials, including feasibility, outcome assessments, and long-term clinical aspects.

We expect global progress to continue. ICH E11 A "Pediatric Extrapolation Guideline," discussed at the ICH meeting in Geneva (November 2017), is expected to reach Step 2a by November 2020. ICH S11 "Safety Testing in Support of Development of Pediatric Medicines" is expected to reach final stage, Step 4, in June 2019.

These are important updates to know and promising steps toward making better medicines available to treat children. PV

Editor's Note: Dr. Martine Dehlinger-Kremer is an observing member of the Coordinating Group of Enpr-EMA, the European Network of Pediatric Research at the European Medicines Agency, and is a member of Working Parties of Enpr-EMA. She is also President of EUCROF, the European CRO Federation, and a Board Member of EFGCP. Contact Synteract at +1 760-268-8200 to leverage its three decades of pediatric experience to advance your clinical trial.

Synteract *is an innovative, full-service contract research organization supporting biopharmaceutical companies in all phases of clinical development to help bring new medicines to market. Synteract has conducted nearly 4,000 studies on six continents and in more than 60 countries, working with more than 26,000 investigative sites and nearly 750,000 patients. The CRO offers a notable depth of expertise in oncology and neuro-degenerative indications, as well as rare and orphan, pediatric, and immunotherapy studies.*

For more information, visit synteract.com.

About Synteract:

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