Pediatric Drug Development MasterClass

Monday, 27th May 2019, Basel, Switzerland

Trainer: **Klaus Rose, klausrose Consulting, Basel, Switzerland**

09.30h	Registration & Coffee
10.00h	Welcome
10.10h	Introduction: Pediatric Drug Development
11.00h	Operational basics of EU Pediatric Investigation Plans (PIPs)
11.45h	Operational basics of US initial Pediatric Study Plans (iPSPs)
12.30h	Break
13.00h	Negotiating PIPs
13.30h	PIP modifications
14.00h	PIPs: strategies beyond PIP modifications
14.30h	Break
15.00h	Interactive training: EMA/FDA websites, www.clinicaltrials.gov, & more
16.00h	Impact of US & EU pediatric legislation on drug development
16.45h	Wrap-Up
17.00h	End of Masterclass

Costs:

Industry CHF 600

Academia, patient advocacy groups CHF 300

Students can apply for reduced price at CHF 100

Registration at registration@congrex.com

Masterclass Pediatric Drug Development

Rationale & description

Regarding children, drug development faces two realities: the real world and the regulatory view. Usually, these two views are compatible, but drug development in children is burdened by flawed concepts and institutional conflicts of interest so far barely acknowledged. At this interface of law, medicine and regulatory affairs, all players including clinicians, scientists and other industry professionals need a compass to navigate the jungle of confusing information and regulatory requirements that often contradict common sense.

FDA and EMA approve drugs for "children" separately. The FDA defines "children" as <17y, the EU as <18y, as if "children" were another species. Both demand separate proof of efficacy in "children": the FDA initial pediatric study plan (iPSP) 60 days after the end-of-phase-2 (EoP2) meeting; the EU a "pediatric investigation plan" (PIP) years before marketing authoristation application (MAA). PIP negotiations take roughly one year. Without a PIP agreed by the EMA Pediatric Committee (PDCO) MAA is blocked.

Newborns are vulnerable and immature, but grow and mature fast. The FDA/EMA age limits are artificial, confering an inadequate, pseudo-physiological connotation to administrative age limits. The word "child" has different meanings. With "children" we associate small, vulnerable persons; but administratively, anybody <17/18y is a "child". US and EU pediatric laws are semantically based on the blur between different meanings of the word "child".

Regulatory "pediatric" challenges include

1) Negotiating "pediatric" development for new drugs with FDA and EMA. Companies should try to develop drugs without separate "pediatric" efficacy studies that mostly medically lack sense or even harm patients. Adolescents should be included into pivotal adult studies, even if this requires additional paperwork. The FDA is often more pragmatic, but the US Research to Accelerate Cures and Equity (RACE) for Children Act will lead to FDA "pediatric" demands in anticancer drugs from 2020 on.

2) Ongoing PIPs and clinical studies that cost millions per year and are medically questionable, but the EMA insists on their continuation. But the EMA will not dare to openly antagonize clinicians involved in such studies and ethics committees that can suspend them.

3) Strategic questions including collaboration with patient advocacy groups, challenging FDA/EMA demands with Institutional Review Boards/ ethics committees, in the media, in the courts, in the scientific press, and in the social media.

To deal with regulatory "pediatric" challenges, you need solid knowledge in pharmacology, science, developmental physiology, history of pediatric laws & FDA/EMA decisions, the outcome of FDA/EMA-demanded studies, and diplomacy. The masterclass will help.