

Children remain underrepresented in drug research, says expert

January 11 2017

Children continue to be underrepresented in drug and medical research, making them less likely to receive personalized health-care treatments for life-threatening conditions such as cancer and infectious disease, says Purdue University professor of chemistry Peter Kissinger. New tools are about to change this.

Prescribing and administering drugs is especially difficult because young children cannot describe their feelings with any precision, and supportive data from [clinical trials](#) has been lacking. Many drugs are also used off label.

"One area where we believe we can make a difference very quickly is in [pediatric intensive care](#) where patients have limited blood volume and their health status can change very quickly," Kissinger said. "Studies have shown that we can now collect blood samples of as little as 0.1 percent of an ounce in a preprogrammed way that reduces labor, blood waste, and infection risk, while still allowing quality measurements. Some chemical diagnostics can now be made with a million-fold reduction in volume compared to 1980. This opens a number of opportunities for pediatric health care, especially for very [young children](#)."

Kissinger reports that continued improvements in the way research is conducted with these [new tools](#) will have long-term benefits.

Research over the last five decades has enabled more evidence-based

decisions derived from [randomized controlled trials](#). These studies provide averages for subpopulations that guides therapy, but imprecisely. We are just beginning to have the potential for quality data to support decisions one patient at a time. While traditional physical data such as body temperature and heart rate have great value, chemistry is driving the bus.

"The reduction in volume is made feasible by parallel advances in mass spectrometry and immunoassays. For example, ambient ionization methods developed in professor Graham Cooks' group at Purdue enabled our team to help found Prosofia Inc. This measurement technology is especially viable for drug determinations," said Kissinger, who later began Phlebotics Inc. to improve blood sampling for personalized medicine. "The combination of more efficient sampling and better measurement instruments provides a real opportunity to improve both pharmaceutical research and care for hospitalized children. To make it work, the data must be integrated into a decision support system that fits hospital workflows, considering the entire team of caregivers."

Using such methods for treatments and [medical research](#) will be important to patient outcomes and to gain consent from parents for clinical trials.

"This is just the beginning of a multifactorial problem, which we are working together to address," he said. "There are still challenges and considerations in collecting real and meaningful data to improve health care outcomes for children."

Other reasons why children are often not the focus of clinical studies include:

- Children are not small adults. Their physical characteristics change very rapidly from birth to their late teens. The rate of

change confounds clinical trials of any length. For example, newborns vary biochemically month to month, and age is not a reliable metric to predict phenotype.

- The ethical challenges of clinical trials with children are daunting to some and require informed consent from parents and/or guardians. Unlike for adults, healthy children are not included.
- While Congress and the FDA have provided incentives, such as extended market exclusivity to pharmaceutical companies who add a pediatric component to trials for new drugs, the incentive is not often sufficiently compelling.

"Working with children's hospitals is a logical next step, but it is a very big step with respect to regulations, demonstrating safety and fitting into medical workflows," Kissinger said. "Fortunately, the underlying science is complete for many of these enhancements."

Networks of children's hospitals working together for pediatric research include the Eunice Kennedy Shriver National Institute of Child Health and Human Development, which is funded through the National Institutes of Health. It has organized a pediatric trials network and a neonatal research network among children's hospitals that share experiences and data. The 2003 Research to Accelerate Cures and Equity for Children Act was introduced to Congress requiring compliance with the Pediatric Research Equity Act in 2003. Last month the 21st Century Cure Act passed Congress and was signed into law, further supporting the potential for more precise and less error prone care.

"We have achieved much in the past decade to advance treatments for [children](#), but there is much more we can and should do," Kissinger said.

Provided by Purdue University

Citation: Children remain underrepresented in drug research, says expert (2017, January 11)
retrieved 29 April 2024 from
<https://medicalxpress.com/news/2017-01-children-underrepresented-drug-expert.html>

This document is subject to copyright. Apart from any fair dealing for the purpose of private study or research, no part may be reproduced without the written permission. The content is provided for information purposes only.