



Pharsight Capabilities in Pediatric Drug Development

Nearly half of pediatric trials for drugs used in adults fail to provide evidence of effectiveness¹. The challenges and complexities of conducting clinical studies in children are well recognized². Population modeling and simulation offers powerful methods for leveraging prior knowledge to support dosing, trial design and regulatory submissions in pediatric patients. As the advantages and benefits of pharmacometric analysis for improved drug development and regulatory decision-making have become increasingly well documented, FDA has challenged industry to more rigorously apply modeling and simulation to double the success rate of pediatric trials³.

Summary of Pharsight Expertise

Pharsight has expertise in quantitative pharmacology and population PK/PD modeling to reduce risk in pediatric drug development:

- Study design optimization
 - Dose selection
 - Prediction of drug exposure
 - Optimal sampling strategies to minimize the number of blood samples required in pediatric studies
- Modeling and simulation of PK/PD in pediatric patients to optimize safety and efficacy of treatments and demonstrate concentration-effect relationships.
- Bridging PK/PD data from adult to pediatric patients, animals to pediatrics
- Expertise with small molecules and biologics in neonates, infants, children and adolescents.
- Regulatory consultation for study design, submission and meetings with FDA/EMA.
- Pediatric project experience in multiple therapeutic domains/indications, including:
 - Anti-infectives, CNS, GI
 - Immunomodulation, Endocrine, Pain
 - Rare genetic diseases, orphan products
 - Formulation development, re-formulation
- More than 45 conference abstracts and peer reviewed publications in pediatrics.

Innovative & Informative Areas for Sponsor-Pharsight Collaboration in Pediatrics

- Study Optimization
 - Dosing recommendations
 - Optimal PK and PD sampling strategy
 - Clinical trial simulations
- Protocol Development
 - Protocol writing and review
 - Scientific rationale for sample size, power calculation, and trial success



Areas for Sponsor-Pharsight Collaboration in Pediatrics (continued)

- Scientific and Technical Writing
 - Regulatory-compliant PK/PD analysis
 - Technical and regulatory population PK report
 - Manuscript development

About Pharsight Consulting Services

Pharsight Consulting Services is a global team of experts dedicated to supporting strategic drug development decisions, submission-ready analyses and reporting for new drug approval.

- More than 40 expert staff in the disciplines of pharmacometrics, biostatistics, decision analysis and drug development strategy.
- Unrivalled therapeutic area domain experience, preclinical to post-approval: 80+ disease indications, 130+ drug classes.
- Unique ability to leverage leading Pharsight PK/PD software technology.

For more information about Pharsight, please visit us at www.pharsight.com.

Pharsight Contact

Pharsight Consulting Services would welcome the opportunity to collaborate with your organization. For more information, please contact:

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References

¹Benjamin DK et al. *Hypertension* 2008;51:834-840.

²Jadhav PR et al. 2010. Pediatric Drug Development and Clinical Pharmacology. In press.

³Gobburu JV. "How to Double Success Rate of Pediatric Trials?".

www.slideshare.net/JogaGobburu/how-to-double-success-rate-of-pediatric-trials.