Rho
Giving flight to research

Orphan Drugs and Rare Diseases

www.rhoworld.com

General Experience

> 125 Trials

> 23,000 Subjects

> 2,000 Sites

Indications

We have experience with numerous rare diseases and orphan indications, including:

- Organ Transplantation (multiple types)
- Cholera
- Hypophosphatasia
- HIV/AIDS
- Chronic Myelogenous Leukemia
- Sickle Cell Anemia
- Pulmonary Hypertension
- Cystic Fibrosis
- Sjögren's Syndrome
- Hemophilia A & B
- Von Willebrand Disease
- Multiple Myeloma
- Hepatocellular Carcinoma
- Glioblastoma Multiforme

- We have extensive experience submitting Orphan Drug Designation Applications as well as experience with Accelerated Approval programs and securing Priority Review for applications.
- Because of our work with industry-sponsored orphan drug programs and federally-funded research in rare diseases, we have experience working with small patient populations and a track record of successful enrollment in special sub-populations.
- We can help you create a regulatory strategy that will best utilize all available regulatory pathways.
- Our programs have included work with foundations, public-private partnerships, and research consortiums.

FOLLOW US ON **Twitter @rhoworld**

Meet Our Experts

David Shoemaker, Ph.D.

Senior Vice President, Research and Development

Dr. Shoemaker has over 25 years of experience in research and pharmaceutical development. He has served as an advisor for multidisciplinary, matrix managed project teams and has been involved with products at all stages of the development process. His primary activities include designing and overseeing the execution of early stage development (Pre-IND/CTA) and late stage to-market strategies; analysis of critical scientific, clinical, and regulatory issues; identifying optimal regulatory pathways; and designing and assessing integrated nonclinical, chemistry, manufacturing, and controls (CMC), and clinical programs.

He has extensive experience in the preparation and filing of all types of regulatory submissions including primary responsibility for four BLAs and three NDAs. He has managed or contributed to dozens of INDs/CTAs and over a dozen successful NDAs, BLAs, and MAAs. Dr. Shoemaker has moderated dozens of regulatory authority meetings for all stages of development and supported several companies at FDA Advisory Committee meetings. Dr. Shoemaker has authored or overseen dozens of Orphan Drug Designation applications, has developed several successful Accelerated Approval programs, and has secured several Priority Review applications.

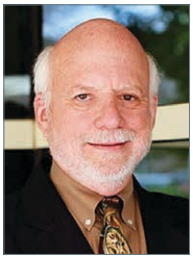
Karl Whitney, Ph.D.

Director, Product Development

Dr. Whitney has over 10 years of experience in the pharmaceutical industry, specializing in clinical and regulatory aspects of pharmaceutical development. During this time, he has led multiple integrated drug-development programs spanning the development spectrum, by planning, managing, and overseeing concurrent manufacturing, nonclinical, clinical, and regulatory activities. Dr. Whitney has also led the preparation of or helped prepare IND applications, clinical and nonclinical final study reports, pre-IND and NDA meeting packages, annual reports, Investigator Brochures, Fast-Track and Orphan-Drug applications, NDAs prepared in the electronic Common Technical Document (eCTD) format, and numerous other documents. He has also provided strategic advice to clients on a broad array of drug development strategy issues. Dr. Whitney's therapeutic areas of expertise include CNS disorders such as drug addiction, pain, Huntington's disease, and schizophrenia as well as tuberculosis.

David Iklé, Ph.D.

Principal Investigator



Dr. Iklé has more than 25 years of experience as a senior biostatistician in a variety of areas of application, including manufacturing research and development, statistical process control, clinical trial design and analysis, and bioinformatics, and has co-authored more than 60 peer-reviewed publications in clinical and translational science. Dr. Iklé is Co-Principal Investigator of the Statistical and Data Coordinating Center (SDCC) for the Immune Tolerance Network (ITN), responsible for biostatistical leadership of its clinical trials in solid organ transplantation. In addition, he is the Principal Investigator of the Statistical and Clinical Coordinating Center (SACCC) for three consortia funded by the National Institute of Allergy and Infectious Diseases (NIAID): Clinical Trials in Organ Transplantation (CTOT), Clinical Trials in Organ Transplantation in Children (CTOT-C), and the Genomics in Transplantation Cooperative Research Program (GTCRP). Thus, he provides leadership for more than 25 clinical and observational studies designed to improve clinical outcomes from transplants, to reduce the burden on patients of immunosuppressive medications, and to develop genomic and proteomic biomarkers predictive of long-term transplant survival.