Orphan Drugs and Rare Diseases

General Experience

- **150+ STUDIES**
- **30K+ PATIENTS**
- **2,200+ SITES**

Indications

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

- 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
- Glioblastoma Multiforme
- Thrombocytopenia
- Metastatic melanoma
- Fragile X Syndrome
- Diffuse Systemic Sclerosis
- Parotid Salivary Hypofunction
- Niemann-Pick Disease Type C
- Pemphigus Vulgaris
- Non-Hodgkins Lymphoma (NHL)
- Hepatocellular Carcinoma
- Diffuse Systemic Sclerosis
- Organ Transplantation

KEY FACTS

- 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.

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. David has moderated dozens of regulatory authority meetings for all stages of development and supported several companies at FDA Advisory Committee meetings. He 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. | *Assistant Vice President, Product Development*

Dr. Whitney has over 15 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. Karl 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. His therapeutic areas of expertise include a number of orphan indications including Huntington's disease, tuberculosis, and hemophilia. Dr. Whitney also has experience working with gene therapy products.

JAMIE ARNOTT, R.N., B.S.N., O.C.N. | *Project Director*

Ms. Arnott has extensive experience from both the CRO and sponsor perspective in the oversight and management of clinical trial operations and outsourcing with more than 10 years' experience in project management and over 18 years' experience in healthcare as a practitioner and manager. Prior to her tenure at Rho, Jamie was the Director of Clinical Trial Operations for a biotechnology company where she provided oversight and management for all clinical activity for up to four concurrent INDs. She has broad therapeutic experience including orphan diseases like multiple myeloma and cystic fibrosis.

CAITLIN HIRSCHMAN, R.N., B.S.N. | *Clinical Team Lead*

Ms. Hirschman has over 14 years clinical research experience across Phase 1-4 studies in a variety of therapeutic areas including orphan indications such as cystic fibrosis, hepatic encephalopathy, and respiratory distress syndrome. Caitlin has lead site identification and selection; management of CRAs, including leadership of multiple global clinical study teams; development of clinical protocols, case report and other data collections forms, informed consent forms, site and monitor training tools, and clinical monitoring plans. Prior to beginning her clinical research career, she practiced as a Registered Nurse in the Medical Intensive Care Unit where she built a strong base of knowledge in a number of medical therapeutic areas including pulmonology, gastroenterology, cardiology, immunology, endocrinology, hematology, and infectious diseases.