

Raymond A. Huml, MS, DVM *Editor*

Rare Disease Drug Development

Clinical, Scientific, Patient, and Caregiver Perspectives

This book provides a broad overview of rare disease drug development. It offers unique insights from various perspectives, including third-party capital providers, caregivers, patient advocacy groups, drug development professionals, marketing and commercial experts, and patients.

A unique reference, the book begins with narratives on the many challenges faced by rare disease patient and their caregivers. Subsequent chapters underscore the critical, multidimensional role of patient advocacy groups and the novel approaches to related clinical trials, investment decisions, and the optimization of rare disease registries. The book addresses various rare disease drug development processes by disciplines such as oncology, hematology, pediatrics, and gene therapy. Chapters then address the operational aspects of drug development, including approval processes, development accelerations, and market access strategies. The book concludes with reflections on the authors' case for real-world data and evidence generation in orphan medicinal drug development.

Rare Disease Drug Development is an expertly written text optimized for biopharmaceutical R&D experts, commercial experts, third-party capital providers, patient advocacy groups, patients, and caregivers.

"I frequently receive communications from the families of patients whose lives have been transformed by the success of a new rare disease therapy. Each letter is an emotional validation of why we do this work and why people like Raymond A. Huml, DVM, who have a passion for rare disease drug development are critical for our society."

Alistair Macdonald
Chief Executive Officer (CEO) of Syneos Health (SYNH)

"Dr. Raymond A. Huml's comprehensive book will bring new understanding of rare diseases to researchers and clinicians. This essential text highlights innovation and progress to date with an important emphasis on patient centricity."

Lynn O'Connor Vos
VosHealth LLC
Former President and CEO, Muscular Dystrophy Association

"If you are the parent of a child with a rare disease, you likely realize that those pioneering clinical treatments and research in this area are real heroes. Dr. Raymond A. Huml, editor and co-author of this book, as well as his family and co-authors, are some of those heroes. In his latest book, Rare Development Drug Development, Ray and his team become a force multiplier to help develop and deploy more treatments for more rare conditions."

Thomas Pike, former CEO of Quintiles Transnational Corp.

"Raymond A. Huml has pioneered this impressive work by gathering experienced colleagues to contribute chapters according to their specialized knowledge. Touching illustrations add emotion to the penetrating discussions. This book is a must read for those in biopharma and for the many students looking for practical commentary about drug development and rare diseases. In turn, this treatise is an important public health contribution."

Sir Dennis Gillings, CBE, FMedSci

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