

Muscular Dystrophy

A Concise Guide

Raymond A. Huml
Editor

 Springer

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Muscular Dystrophy Raymond A. Huml, 2015-07-16 This practical and concise guide offers an overview of muscular dystrophy's complicated features, treatment options, and general resources. New treatments and a greater understanding of proteins and structures associated with MD are discussed along with long-term patient care. Also included are clinical and developmental challenges within the current regulatory landscape and recent scientific and clinical advances. Muscular Dystrophy offers clinicians, researchers, pharmaceutical executives, and patient advocacy groups an easy-to-read reference that provides the necessary perspectives of the caregiver and patient.

Rare Disease Drug Development Raymond A. Huml, 2021-11-08 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 patients 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.

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