

Muscular Dystrophy

A Concise Guide

Raymond A. Huml
Editor

 Springer

Muscular Dystrophy A Concise Guide

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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 care giver 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 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

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