
Preface

Duchenne muscular dystrophy (DMD) is an X-linked degenerative skeletal muscle disease caused by mutations in the dystrophin gene. This fatal disease occurs in 1 out of 5000 male births, and the average life expectancy for affected individuals is around 25 years.

In the last decades, many efforts have been pursuing the development of a cure or of a safe prenatal test. Unfortunately, dystrophin is a very large gene containing 79 exons, and its size complicates the specificity of potential targeted molecular treatments.

The aim of this book is to provide a comprehensive overview to the experimental approaches used to study DMD, from different standpoints. In particular, we intend to point out the very last progresses and scientific achievements. In this context, the recent discoveries achieved through *in vivo* gene editing proved to be promising in restoring dystrophin expression, at least ameliorating skeletal muscle symptoms.

A specific focus on “Omics” techniques in gene expression, protein expression, miRNAs, and long non-coding RNA analysis, is included. Also, we provide a detailed and up-to-date account of the experimental studies of the structural/functional changes affecting the skeletal and cardiac muscles, related to the progress of the disease. Finally, we consider important ongoing preclinical studies and clinical trials.

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Duchenne Muscular Dystrophy

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