
Contents

<i>Preface</i>	<i>v</i>
<i>Contributors</i>	<i>xi</i>

PART I REVIEWS AND OVERVIEW CHAPTERS

1 An Overview of Recent Therapeutics Advances for Duchenne Muscular Dystrophy.....	3
<i>Jean K. Mah</i>	
2 Clinical Manifestations and Overall Management Strategies for Duchenne Muscular Dystrophy.....	19
<i>Takeshi Tsuda</i>	

PART II SKELETAL AND CARDIAC MUSCLE IN DMD

3 Cardiac Involvement in Duchenne Muscular Dystrophy and Related Dystrophinopathies.....	31
<i>Sophie I. Mavrogeni, George Markousis-Mavrogenis, Antigoni Papavasiliou, George Papadopoulos, and Genovefa Kolovou</i>	
4 Characterization of the Inflammatory Response in Dystrophic Muscle Using Flow Cytometry	43
<i>Jenna M. Kastenschmidt, Ileen Avetyan, and S. Armando Villalta</i>	
5 Imaging Analysis of the Neuromuscular Junction in Dystrophic Muscle	57
<i>Stephen J.P. Pratt, Shama R. Iyer, Sameer B. Shah, and Richard M. Lovering</i>	

PART III OMICS APPROACHES

6 System Biology Approach: Gene Network Analysis for Muscular Dystrophy....	75
<i>Federica Censi, Giovanni Calcagnini, Eugenio Mattei, and Alessandro Giuliani</i>	
7 Proteomic Profiling of the Dystrophin-Deficient Brain	91
<i>Sandra Murphy and Kay Ohlendieck</i>	
8 Probing the Pathogenesis of Duchenne Muscular Dystrophy Using Mouse Models.....	107
<i>Alexander Morrison-Nozik and Saptarsi M. Haldar</i>	

PART IV EXON SKIPPING AND ANTISENSE OLIGONUCLEOTIDES (ASOs)

9 Exon Skipping Therapy Using Phosphorodiamidate Morpholino Oligomers in the <i>mdx52</i> Mouse Model of Duchenne Muscular Dystrophy.....	123
<i>Shouta Miyatake, Yoshitaka Mizobe, Hotake Takizawa, Yuko Hara, Toshifumi Yokota, Shin'ichi Takeda, and Yoshitsugu Aoki</i>	

10	Designing Effective Antisense Oligonucleotides for Exon Skipping	143
	<i>Takenori Shimo, Rika Maruyama, and Toshifumi Yokota</i>	
11	Identification of Splicing Factors Involved in DMD Exon Skipping Events Using an In Vitro RNA Binding Assay	157
	<i>Julie Miro, Cyril F. Bourgeois, Mireille Claustres, Michel Koenig, and Sylvie Tuffery-Giraud</i>	
12	The Use of Antisense Oligonucleotides for the Treatment of Duchenne Muscular Dystrophy	171
	<i>Karima Relizani and Aurelie Goyenvallé</i>	
13	PMO Delivery System Using Bubble Liposomes and Ultrasound Exposure for Duchenne Muscular Dystrophy Treatment.....	185
	<i>Yoichi Negishi, Yuko Ishii, Kei Nirasawa, Eri Sasaki, Yoko Endo-Takahashi, Ryo Suzuki, and Kazuo Maruyama</i>	

PART V BIOMARKERS AND DRUG DISCOVERY

14	Proton Nuclear Magnetic Resonance (^1H NMR) Spectroscopy-Based Analysis of Lipid Components in Serum/Plasma of Patients with Duchenne Muscular Dystrophy (DMD)	195
	<i>Niraj Kumar Srivastava</i>	
15	Test of Antifibrotic Drugs in a Cellular Model of Fibrosis Based on Muscle-Derived Fibroblasts from Duchenne Muscular Dystrophy Patients ..	205
	<i>Simona Zanotti and Marina Mora</i>	
16	Flow Cytometry-Defined CD49d Expression in Circulating T-Lymphocytes Is a Biomarker for Disease Progression in Duchenne Muscular Dystrophy	219
	<i>Wilson Savino, Fernanda Pinto-Mariz, and Vincent Mouly</i>	

PART VI LAST DISCOVERIES AND FUTURE PROSPECTIVES

17	Advanced Methods to Study the Cross Talk Between Fibro-Adipogenic Progenitors and Muscle Stem Cells	231
	<i>Luca Tucciarone, Usue Etxaniz, Martina Sandomá, Silvia Consalvi, Pier Lorenzo Puri, and Valentina Saccone</i>	
18	AAV6 Vector Production and Purification for Muscle Gene Therapy	257
	<i>Christine L. Halbert, James M. Allen, and Jeffrey S. Chamberlain</i>	
19	From gRNA Identification to the Restoration of Dystrophin Expression: A Dystrophin Gene Correction Strategy for Duchenne Muscular Dystrophy Mutations Using the CRISPR-Induced Deletion Method	267
	<i>Benjamin Duchêne, Jean-Paul Iyombe-Engembe, Joël Rousseau, Jacques P. Tremblay, and Dominique L. Ouellet</i>	

<i>Index</i>	285
--------------------	-----