

Contents

<i>Preface</i>	<i>v</i>
<i>Contributors</i>	<i>xi</i>
PART I RETINAL GENE THERAPY VECTOR PRODUCTION AND BIOLOGICAL ASSAYS IN VITRO	
1 Small Scale Production of Recombinant Adeno-Associated Viral Vectors for Gene Delivery to the Nervous System.	3
<i>Joost Verhaagen, Barbara Hobo, Erich M.E. Ehlert, Ruben Eggers, Joanna A. Korecka, Stefan A. Hoyng, Callan L. Attwell, Alan R. Harvey, and Matthew R.J. Mason</i>	
2 Small and Micro-Scale Recombinant Adeno-Associated Virus Production and Purification for Ocular Gene Therapy Applications	19
<i>Christopher A. Reid and Daniel M. Lipinski</i>	
3 Design and Development of AAV-based Gene Supplementation Therapies for Achromatopsia and Retinitis Pigmentosa.	33
<i>Christian Schön, Elvir Becirovic, Martin Biel, and Stylianos Michalakis</i>	
4 Development of Multigenic Lentiviral Vectors for Cell-Specific Expression of Antiangiogenic miRNAs and Protein Factors	47
<i>Anne Louise Askou and Thomas J. Corydon</i>	
5 Design and In Vitro Use of Antisense Oligonucleotides to Correct Pre-mRNA Splicing Defects in Inherited Retinal Dystrophies	61
<i>Alejandro Garanto and Rob W.J. Collin</i>	
6 Three-Dimensional Co-Culture Bioassay for Screening of Retinal Gene Delivery Systems	79
<i>Ding Wen Chen, Kathleen Pauloff, and Marianna Foldvari</i>	
7 Retinal Gene Therapy for Choroideremia: In Vitro Testing for Gene Augmentation Using an Adeno-Associated Viral (AAV) Vector	89
<i>Maria I. Patrício and Robert E. MacLaren</i>	
PART II ASSAYS FOR GENE AUGMENTATION AND EDITING IN VIVO ON RODENT AND MACAQUE RETINA	
8 In Vivo Electroporation of Developing Mouse Retina	101
<i>Jimmy de Melo and Seth Blackshaw</i>	
9 Methods for In Vivo CRISPR/Cas Editing of the Adult Murine Retina	113
<i>Sandy S. Hung, Fan Li, Jiang-Hui Wang, Anna E. King, Bang V. Bui, Guei-Sheung Liu, and Alex W. Hewitt</i>	
10 AAV Gene Augmentation Therapy for <i>CRB1</i> -Associated Retinitis Pigmentosa	135
<i>C. Henrique Alves and Jan Wijnholds</i>	

11	Dual AAV Vectors for Stargardt Disease	153
	<i>Ivana Trapani</i>	
12	Optogenetic Retinal Gene Therapy with the Light Gated GPCR Vertebrate Rhodopsin	177
	<i>Benjamin M. Gaub, Michael H. Berry, Meike Visel, Amy Holt, Ehud Y. Isacoff, and John G. Flannery</i>	
13	CRISPR Repair Reveals Causative Mutation in a Preclinical Model of Retinitis Pigmentosa: A Brief Methodology	191
	<i>Wen-Hsuan Wu, Yi-Ting Tsai, Sally Justus, Galaxy Y. Cho, Jesse D. Sengillo, Yu Xu, Thiago Cabral, Chyuan-Sheng Lin, Alexander G. Bassuk, Vinit B. Mahajan, and Stephen H. Tsang</i>	
14	In-Depth Functional Analysis of Rodents by Full-Field Electroretinography	207
	<i>Vithiyanjali Sothilingam, Regine Mühlfriedel, Naoyuki Tanimoto, and Mathias W. Seeliger</i>	
15	Advanced Ocular Injection Techniques for Therapy Approaches	215
	<i>Regine Mühlfriedel, Marina Garcia Garrido, Christine Wallrapp, and Mathias W. Seeliger</i>	
16	Neutralizing Antibodies Against Adeno-Associated Virus (AAV): Measurement and Influence on Retinal Gene Delivery	225
	<i>Mélissa Desrosiers and Deniz Dalkara</i>	
17	Screening for Neutralizing Antibodies Against Natural and Engineered AAV Capsids in Nonhuman Primate Retinas.	239
	<i>Timothy P. Day, Leah C. Byrne, John G. Flannery, and David V. Schaffer</i>	
18	Subretinal and Intravitreal Retinal Injections in Monkeys	251
	<i>Daniyar Dauletbekov, K. Ulrich Bartz-Schmidt, and M. Dominik Fischer</i>	
PART III CLINICAL PROTOCOLS AND RETINAL GENE THERAPY VECTOR TESTING ON HUMAN RETINA		
19	Production of iPS-Derived Human Retinal Organoids for Use in Transgene Expression Assays	261
	<i>Peter M. Quinn, Thilo M. Buck, Charlotte Ohonin, Harald M.M. Mikkers, and Jan Wijnholds</i>	
20	AAV Serotype Testing on Cultured Human Donor Retinal Explants.	275
	<i>Thilo M. Buck, Lucie P. Pellissier, Rogier M. Vos, Elon H.C. van Dijk, Camiel J.F. Boon, and Jan Wijnholds</i>	
21	Human Retinal Explant Culture for Ex Vivo Validation of AAV Gene Therapy	289
	<i>Harry O. Orlans, Thomas L. Edwards, Samantha R. De Silva, Maria I. Patrício, and Robert E. MacLaren</i>	
22	Visual Acuity Testing Before and After Intravitreal Injection of rAAV2-ND4 in Patients	305
	<i>Bin Li and Chenmian Wu</i>	
23	Recording and Analysis of the Human Clinical Electroretinogram	313
	<i>Mathieu Gauvin, Allison L. Dorfman, and Pierre Lachapelle</i>	

24	Recording and Analysis of Goldmann Kinetic Visual Fields.	327
	<i>Mays Talib, Gislin Dagnelie, and Camiel J.F. Boon</i>	
25	Measuring Central Retinal Sensitivity Using Microperimetry	339
	<i>Mays Talib, Jasleen K. Jolly, and Camiel J.F. Boon</i>	
26	Inspection of the Human Retina by Optical Coherence Tomography	351
	<i>Thomas Theelen and Michel M. Teussink</i>	
27	Vector Shedding and Immunogenicity Sampling for Retinal Gene Therapy. . .	359
	<i>Alun R. Barnard, Anna N. Rudenko, and Robert E. MacLaren</i>	
	<i>Index</i>	373

Retinal Gene Therapy

Methods and Protocols

Boon, C.J.F.; Wijnholds, J. (Eds.)

2018, XVI, 380 p. 54 illus., 44 illus. in color., Hardcover

ISBN: 978-1-4939-7521-1

A product of Humana Press