Contents

Preface ................................................................. v
Contributors ........................................................... ix

1 The Concept of Prenatal Gene Therapy ...................... 1
Charles Coutelle and Simon N. Waddington

2 Candidate Diseases for Prenatal Gene Therapy .............. 9
Anna L. David and Simon N. Waddington

3 Vector Systems for Prenatal Gene Therapy: Choosing Vectors for Different Applications ............. 41
Charles Coutelle and Simon N. Waddington

Raul Alba, Andrew H. Baker, and Stuart A. Nicklin

5 Vector Systems for Prenatal Gene Therapy: Principles of Retrovirus Vector Design and Production ........ 85
Steven J. Howe and Anil Chandrashekran

Christopher J. Binny and Amit C. Nathwani

7 Vector Systems for Prenatal Gene Therapy: Principles of Non-viral Vector Design and Production .......... 133
Suet Ping Wong, Orestis Argyros, and Richard P. Harbottle

8 Use of Manipulated Stem Cells for Prenatal Therapy .......... 169
Jessica L. Roybal, Pablo Laje, Jesse D. Vrecenak, and Alan W. Flake

9 Animal Models for Prenatal Gene Therapy: Choosing the Right Model ..................... 183
Vedanta Mehta, Donald Peebles, and Anna L. David

Jessica L. Roybal, Masayuki Endo, Suzanne M.K. Buckley, Bronwen R. Herbert, Simon N. Waddington, and Alan W. Flake

11 Animal Models for Prenatal Gene Therapy: The Sheep Model ........................................ 219
Khalil N. Abi-Nader, Michael Boyd, Alan W. Flake, Vedanta Mehta, Donald Peebles, and Anna L. David

Citra N. Mattar, Arijit Biswas, Mahesh Choolani, and Jerry K.Y. Chan

13 Choice of Surrogate and Physiological Markers for Prenatal Gene Therapy .................. 273
Vedanta Mehta, Khalil N. Abi-Nader, David Carr, Jacqueline Wallace, Charles Coutelle, Simon N. Waddington, Donald Peebles, and Anna L. David

Charles Coutelle, Simon N. Waddington, and Michael Themis

16 Monitoring for Potential Adverse Effects of Prenatal Gene Therapy: Genotoxicity Analysis In Vitro and on Small Animal Models Ex Vivo and In Vivo ................................................................. 341
Michael Themis

17 Risks, Benefits and Ethical, Legal, and Societal Considerations for Translation of Prenatal Gene Therapy to Human Application .......... 371
Charles Coutelle and Richard Ashcroft

Index ............................................................................................................. 389
Prenatal Gene Therapy
Concepts, Methods, and Protocols
Coutelle, C.; Waddington, S.N. (Eds.)
2012, X, 392 p. 33 illus., 7 illus. in color., Hardcover
ISBN: 978-1-61779-872-6
A product of Humana Press