## 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*

   *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*

12 Animal Models for Prenatal Gene Therapy: The Nonhuman Primate Model ....................................... 249
    *Citra N. Mattar, Arijit Biswas, Mahesh Choolani, and Jerry K.Y. Chan*

13 Choice of Surrogate and Physiological Markers for Prenatal Gene Therapy ...................................... 273
    *Juliette M.K.M. Delhove, Ahad A. Rahim, Tristan R. McKay, Simon N. Waddington, and Suzanne M.K. Buckley*
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