

---

## Preface

The purpose of this volume of *Methods in Molecular Medicine* is to set forth examples of the great variety of techniques and applications that are now emerging in the field of nonviral gene therapy. The book emphasizes not only specific approaches to gene delivery but, in particular, the best current methods to prepare, handle, and characterize gene delivery agents. These topics are of very broad importance since gene therapy evolves from its mostly academy-based experimental and clinical research to the ever increasing number of industry-driven programs directed toward commercial development. Successful introduction of nonviral gene therapy agents into the clinic should be expected to require rigorous manufacturing and analytical methods that readily meet the regulatory guidelines under which new drug candidates are reviewed for marketing approval. Exactly what those guidelines will prove to be certainly depends on the established guidelines for review of both biological and chemical therapeutics. Additionally, many new techniques are being devised and applied to gene therapy research; these techniques will be instrumental in developing and characterizing successful gene delivery agents.

*Nonviral Vectors for Gene Therapy: Methods and Protocols* has two main sections. To start with, there is a series of chapters on specific protocols for the synthesis, characterization, and application of gene delivery agents. Several chapters address the topic of materials to bind with DNA to form the compact condensed phases that facilitate cellular delivery. Variations on this theme are addressed by using peptide conjugates, synthetic polymers, and lipids. Increasingly refined methods for the characterization of delivery systems and their complexes with DNA are described both as part of synthesis protocols and as separate topics. One still relatively new analytical technique is atomic force microscopy; this should rapidly gain attention for its applicability to the characterization of DNA condensation. Subsequent chapters describe approaches to gene transfer in vivo, including direct delivery by intratumoral injection or indirect delivery by cell-specific targeting of DNA complexes.

The latter section of *Nonviral Vectors for Gene Therapy: Methods and Protocols* consists of a series of review-format chapters that provide extensive additional information for those preparing and characterizing gene trans-

fer agents. These chapters contain additional information on the use of novel materials to complex DNA. In a highly detailed series of chapters from the Middaugh Laboratory at the University of Kansas, a broad range of spectroscopic techniques is discussed in the context of characterizing nonviral gene delivery agents. Finally, a short review on renal gene therapy discusses an area not well represented elsewhere in treatments of gene therapy. For these topics, the coverage presented here points out a variety of opportunities for bringing new approaches to bear on the development and application of nonviral vectors in the research lab and, eventually, in the clinic.

I regret to note that during the preparation of this book, Dr. Jean-Michel H. Vos of the University of North Carolina at Chapel Hill passed away. We are fortunate to have had him contribute to this volume. His work is being continued by his colleagues, as well as those who will draw on the Vos lab's studies in their own research.

As with any project of this nature, it would not have been possible without an incredible amount of effort and patience on the part of each and every contributor, the Series Editor, Professor John M. Walker, and Craig B. Adams at Humana Press. Thank you to everyone who has supported this project, and thank you to the readers whose interest in this volume make the efforts to produce it all worthwhile.

***Mark A. Findeis***



<http://www.springer.com/978-0-89603-712-0>

Nonviral Vectors for Gene Therapy

Methods and Protocols

Findeis, M.A. (Ed.)

2001, XI, 399 p., Hardcover

ISBN: 978-0-89603-712-0

A product of Humana Press