



Dongsheng Duan (Ed.)

Muscle Gene Therapy

Methods and Protocols

Series: Methods in Molecular Biology

- Presents a comprehensive collection of state-of-the-art muscle gene therapy protocols from leaders in the field
- Focuses on gene delivery in less-covered large animal models, such as swine, ovine, canine, and non-human primates
- Contains tips from the experts highlighting key implementation advice and strategies

Gene therapy offers many conceptual advantages to treat muscle diseases, especially various forms of muscular dystrophies; however, it faces a number of unique challenges, including the need to deliver a therapeutic vector to all muscles throughout the body. In *Muscle Gene Therapy: Methods and Protocols*, expert researchers in the field present a collection of techniques aimed at bridging the translational gap in muscle gene therapy between the prevalent rodent models and vitally important larger animal models. Divided into three sections, this volume examines basic protocols for optimizing the muscle gene expression cassette and for evaluating the therapeutic outcomes, new developments in muscle gene therapy technology such as adeno-associated viral vector (AAV), oligonucleotide-mediated exon-skipping, and novel RNA-based strategies, and step-by-step guidance on muscle gene delivery in swine, ovine, canine, and non-human primates. Written in the highly successful *Methods in Molecular Biology™* series format, chapters include introductions to their respective topics, lists of the necessary materials and reagents, detailed, readily reproducible laboratory protocols, and tips on troubleshooting and avoiding known pitfalls. Authoritative and cutting-edge, *Muscle Gene Therapy: Methods and Protocols* serves as an invaluable resource for graduate students, post-doctoral fellows, and principle investigators pursuing the crucial advancement of muscle disease gene therapy in the hope of someday curing these debilitating disorders.

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