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Sommario/riassunto

About 7 million people worldwide are suffering from various inherited neuromuscular diseases. Gene therapy brings the hope of treating these diseases at their genetic roots. Muscle Gene Therapy is the only book dedicated to this topic. The first edition was published in 2010 when the field was just about to enter its prime time. The progress made since then has been unprecedented. The number of diseases that have been targeted by gene therapy has increased tremendously. The gene therapy toolbox is expanded greatly with many creative novel strategies (such as genome editing and therapy with disease-modifying genes). Most importantly, clinical benefits have begun to emerge in human patients. To reflect rapid advances in the field, we have compiled the second edition of Muscle Gene Therapy with contributions from experts that have conducted gene therapy studies either in animal models and/or in human patients. The new edition offers a much needed, up-to-date overview and perspective on the foundation and current status of neuromuscular disease gene therapy. It provides a framework to the development and regulatory approval of muscle gene therapy drugs in the upcoming years. This book is a must-have for anyone who is interested in neuromuscular disease gene therapy including those in the research arena (established investigators and trainees in the fields of clinical practice, veterinary medicine and basic

biomedical sciences), funding and regulatory agencies, and patient community.
