Commentary

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A Brief Note on Molecular Therapy

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Introduction

Molecular therapies for neuromuscular disorders are rapidly expanding and hold promise for devastating diseases with few options for treatment. Diseases targeted in this review include Duchenne, Becker, and the limb girdle muscular dystrophies. Strategies to repair defective genes involve exon skipping using antisense oligonucleotides or mutation suppression requiring small molecules that permit dose-dependent readthrough of stop codons. Clinical trials using these products have been completed or are underway. Gene replacement strategies targeting muscular dystrophies have been introduced into the clinic, and delivery potential is expanding by administration throughout the extremity using a vascular approach.

An exciting development in gene therapy employs a scheme for gene expression through recombination approaches, expanding the potential for delivering genes usually considered too large for packaging into adeno associated virus. Also discussed in this review are therapeutic genes that do not replace but facilitate functional recovery through alternate pathways such as myostatin inhibition. When confronted with the wide array of targets for molecular therapy, it is easy to forget that only a minority of cancer patients will ever be eligible for these treatments. The vast majority of patients with advanced, malignant, solid tumors are treated with non-targeted chemotherapy. It is well known that two patients with the same tumor will not necessarily respond in the same way to the same chemotherapy regimen. It is likely that molecular differences between tumors underlie these differential responses. Indeed, given that the mechanisms of action of most chemotherapy agents are related either to enzymes involved in DNA repair, e.g. ERCC1 and platinum-based chemotherapy, or to the transport of molecules across the cell membrane, it seems reasonable that specific markers predicting chemotherapy response could be discovered.

Prospects for Molecular Therapy

Although many therapeutic strategies for lymphedema effectively reduce excess volume, minimize complications, and optimize function, the disease currently lacks a cure. For these reasons, there has been emphasis on the possible application of effective molecular therapies. Among these, the most exciting to date is therapeutic lymphangiogenesis, which is based on insights into the developmental biology of the lymphatics. While it is clear that exercise conditioning is beneficial in cardiac disease, whether modulation of these molecular targets, PI3K/Akt, C/EBPβ, or paracrine factors such as PGF or IL-6, has the potential to influence cardiac physiology in a clinical context is far from established. Moreover, there are potential risks inherent in a molecular therapy that seeks to identify and target these relevant molecular targets. First, it is quite clear that physiologic hypertrophy represents a coordinate response of the myocardium to a physiologic trigger and this is effected not only at the level of the cardiocyte but also in terms of mitochondrial function, extracellular matrix content, and vascularity.

Targeting one component of this response without acknowledging the others is potentially dangerous. For example, one might create a cardiac phenotype that has increased contractile capability but is energy and/or substrate limited (although the fact that transgenic animals overexpressing PI3K activity appear to do well and transgenic animals overexpressing PGF are protected against failure secondary to pressure overload is encouraging). In addition, the link to a cell proliferation signal (via CITED4), while potentially quite exciting, raises questions about the complexity of ventricular remodeling in the context of the adult heart. Moreover, exercise is a dose-dependent and intermittent stimulus and designing a molecular therapy that recapitulates this unique physiology will be challenging.