Stem Cell Biology and Regenerative Medicine

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Regenerative Medicine for Degenerative Muscle Diseases

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Preface

Regenerative medicine—an emerging multidisciplinary field composed of clinicians, bioengineers, pharmacologists, surgeons, and others—seeks to develop new ways of repairing and replacing cells, tissues, and organs. A great example of regenerative medicine technology was pioneered by Anthony Atala, MD, who pioneered the world's first urinary bladder made from the patient's own cells and grown in a laboratory before being implanted into the patient. This process of regenerative medicine differs radically from conventional medicine by not only treating symptoms but by recreating damaged tissues and organs in the human body. Unthinkable only a few years ago, clinicians and scientists are rapidly exploiting nature's secrets to replace damaged tissue or to stimulate the body's own natural repair mechanisms to heal previously irreparable tissues or organs. For degenerative muscle diseases, this is a tough order, since skeletal muscle comprises the largest tissue type in the human body. Not only is skeletal muscle the largest "organ" in the body, but most degenerative diseases, such as the muscular dystrophies, are caused by rare inherited genetic mutations. So in this case, regenerative technologies must overcome two very difficult problems. The first problem is to create enough "medicine" to treat the entire volume of skeletal muscle in a human body, and the second is to replace defective genes. Both problems may seem insurmountable at first glance, but the history of medical progress, particularly in the field of genetics and gene therapy, tells us otherwise.

In this book, the first three chapters are dedicated to an overarching view of how genetics and regenerative technologies can synergistically work together to target skeletal muscle genetic diseases. The next five chapters explore how an understanding of cellular biology (particularly stem cell biology and cellular reprogramming) proffers alternative methods to gene replacement. Chapters 8 and 9 examine new ways to look at our conventional approaches (rehabilitation medicine and nutrition) when combined with regenerative technologies. And the final chapters introduce the concepts of "micro-RNAs" and how fish and dog models can be used to enhance our understanding of these devastating and often fatal diseases of skeletal muscle.

Altogether, these new concepts, technologies, and approaches should give patients, families, and doctors cutting-edge information about incredible regenerative advances already under way in laboratories and clinics worldwide.

Seattle, WA, USA Martin K. Childers

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Martin K. (Casey) Childers, D.O., Ph.D. is a professor in the Department of Rehabilitation Medicine and Investigator at the Institute for Stem Cell & Regenerative Medicine, University of Washington. He is a graduate of Seattle Pacific University (B.A., Music Performance), Western University (D.O., Medicine, Osteopathic), and The University of Missouri (Ph.D., Physiology & Pharmacology; residency, Rehabilitation Medicine). The Childers' laboratory works in two areas of investigation. In a series of preclinical studies, they address the hurdles required for systemic gene replacement delivery for patients with X-Linked Myotubular Myopathy (XLMTM). In other studies, they use a "disease-in-a-dish" approach with induced pluripotent stem (iPS) cells to study heart disease in patients with Duchenne muscular dystrophy (DMD). Dr. Childers' clinical medicine practice at the University of Washington Medical Center is dedicated to serve patients with neuromuscular diseases.