INTERNATIONAL SYMPOSIUM ON NEUROMUSCULAR DISORDERS STATE-OF-THE-ART AT THE EDGE OF THE MILLENNIUM BRUSSELS, DECEMBER 11th, 1999

Introduction

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A staggering diversity of different disease entities and a relatively low prevalence of each of these are characteristic of neuromuscular disorders. Many disorders of muscle and nerve are hereditary; most of them are progressive and incurable. They are therefore associated with intolerable physical and psychological suffering of patients and their families and constitute a significant burden to society. Over the last 150 years, the nosological scope of these disorders has been progressively defined first by clinical observation and subsequently by neurophysiological, pathological, and biochemical studies. The discovery of the Duchenne muscular dystrophy gene and its protein product, dystrophin, in the late 1980's has ushered the hereditary neuromuscular disorders into the field of molecular genetics. Since 5-10 years, the medical community is witnessing how the molecular causes of the muscular dystrophies, the channelopathies, the metabolic myopathies, the congenital myopathies, the spinal muscular atrophies, and the hereditary neuropathies are being discovered at an explosive pace. Insights into the biology of muscle and nerve and into the pathogenesis of hereditary and inflammatory or autoimmune neuromuscular disorders are rapidly expanding thanks to advances in molecular genetics and immunology. This leads to ongoing adaptation and refinement of the nosological spectrum of most neuromuscular disorders. The implications for the development of rational treatment strategies are major. Over the last few years, spectacular progress has been made in the treatment of inflammatory demyelinating neuropathies by the introduction of plasma exchange and intravenous immunoglobulin. Prenatal and preclinical diagnosis and heterozygous carrier detection for many hereditary neuromuscular disorders are possible. Most exciting is the prospect of gene therapy for the muscular dystrophies, the feasibility of which is being demonstrated in animal models. The most striking example thereof is the discovery that upregulation of utrophin can functionally compensate for dystrophin deficiency in the mdx mouse.

In this issue of Acta Neurologica Belgica, selected papers from leading investigators in various fields of neuromuscular disorders in Belgium and abroad, presented at the International Symposium on Neuromuscular Disorders, held in Brussels on December 11th, 1999, are published. They illustrate how close collaboration between clinicians and scientists leads to exciting discoveries that pave the way to understanding neuromuscular disorders and to the final goal, which is to find a cure for as many of them as possible.