

## FOREWORD

Amyotrophic lateral sclerosis (ALS) is a progressive neurodegenerative disease, characterized by degeneration of both upper motor neurons including motor cortex of the cerebrum and lower motor neurons in the brainstem and the spinal cord. Because of the rapid progression of muscular weakness and atrophy during the course of the disease and the lack of curative therapy with an estimated mortality of 30,000 patients a year worldwide, ALS is often said to be the most devastating neurodegenerative disorder in adults.

Since the approval of riluzole by the US Food and Drug Administration in 1995, many clinical trials have failed until the recent approval of edaravone. Both riluzole and edaravone are disease modifying drugs with limited benefits, and neither of them are curative. There are several on-going clinical trials with different mechanistic concepts. These include small molecules AMX0035 (combination of sodium phenylbutyrate and tauroursodeoxycholic acid) and mastinib (c-kit inhibitor), antisense nucleotide drug tofersen (antisense for superoxide dismutase 1), humanized monoclonal antibody ravulizumab-cwvz (antibody against C5 complement), and mesenchymal stem cell (MSC)-neurotrophic factor (NTF) cells as cell-based therapy. Furthermore, there are a large number of different potential therapies in basic research stage. Future therapies against ALS may well come out from these endeavors.

Novel groundbreaking therapy against intractable diseases like ALS can only originate from basic research based on the sufficient understanding of clinical features and disease pathophysiology. This book encompasses different aspects from basic research to clinical characteristics of ALS. While the covered areas may be limited as a single book of eight chapters, these chapters, contributed by practicing clinicians and active basic scientists, will inspire ALS researchers in laboratories and clinics, and lead to a further understanding of the disease and development of novel therapies that will eventually help patients suffering from this intractable condition.

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