

Foreword

The Co-Editors-in-Chief are excited to introduce a new IOS Press journal “*Translational Science of Rare Diseases*” (TRD) which has the focus on research enterprises moving studies in Rare Diseases from the laboratory to clinical trials. Our mission is to build on the numerous advances in fundamental research leading to important translational studies that coupled with increasing interest in developing new pharmaceutical agents, the repurposing of existing agents, and the remarkable progress in therapeutic devices hold great promise for improving the health of individuals affected with these rare disorders, their families and caregivers, and the general public. In the first issue we have focussed on some of the metabolic disorders. For future issues we look forward to receiving independent, original research articles as well as in-depth review of specific rare diseases and therapeutics, and opinion pieces from regulators, patient advocates and leading industry and academic researchers focused on rare diseases.

Taking the broadest perspective, we wish to engage the energies of the international community including fundamental, translational, and clinical trial design to advance studies related to “rare diseases”. Disease-modifying and gene replacement strategies for many disorders are advancing through translational studies. These strategies present fundamental challenges that require efforts of the research community, funding agencies, pharmaceutical companies, advocacy organizations, and individuals affected by the disorders and their families. Our goal is the creation of a platform that will create conversations leading to effective treatment and, ultimately, a cure.

We look forward to great progress.
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