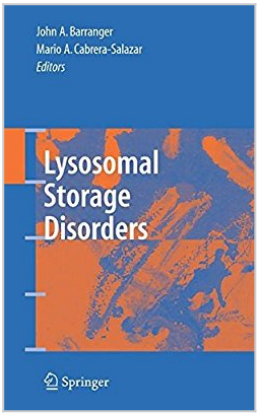

Lysosomal Storage Disorders

From John A Barranger

Lysosomal Storage Disorders From John A Barranger

The knowledge of lysosomal biology and the consequences of its dysfunction have increased dramatically in the past 60 years. This book describes the nature of the lysosomal dysfunction and diseases as well as potential future treatments and therapies. Disease specific chapters provide thorough reviews of the clinical features of lysosomal storage disorders, their molecular basis and the commercial or experimental therapeutic approaches sought in this area. This is an invaluable resource for researchers in biochemical and molecular genetics, enzyme therapy, and gene transfer.

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- Binding: Hardcover
- 564 pages

Editorial Review

Review

From the reviews:

"The authors have brought together an excellent group of contributors to provide an introduction to lysosomal storage disorders (LSDs). … It is oriented to serve individuals working in the field of LSDs, but it also would provide a good base of knowledge for graduate students, medical residents, or clinical genetics physicians who wish to update their understanding of the lysosome pathology and function. … With this unique book, the authors have provided a well thought out source of information on lysosomal disorders." (Luis F. Escobar, Doody's Review Service, July, 2009)

From the Back Cover

Lysosomal Biology and Storage Disorders

John A. Barranger and Mario A. Cabrera-Salazar

The knowledge of lysosomal biology and the consequences of its dysfunction have increased dramatically in the past 60 years. Research of these disorders has moved from diseases with unknown etiology to disorders with clear and defined pathophysiology and some of them have benefited from the development of disease specific therapeutics. ***Lysosomal Biology and Storage Disorders*** describes the nature of the diseases, the historical evolution of the field and future perspectives for the treatment of these clinical entities.

Organized as a textbook, ***Lysosomal Biology and Storage Disorders*** describes the nature of lysosomal dysfunction, the synthesis and targeting of lysosomal enzymes and the implications of the targeting mechanisms for the development of new therapies. Disease specific chapters provide thorough reviews of the clinical features of lysosomal storage disorders, their molecular basis and the commercial or experimental therapeutic approaches sought in this area.

Lysosomal Biology and Storage Disorders will be attract to all researchers in biochemical and molecular genetics, enzyme therapy, gene transfer, and others concerned with the models of genetic disease.

This book is dedicated to patients affected by Lysosomal Storage Disorders, and especially to the National Gaucher Foundation (USA) and the Colombian Association of Patients with Lysosomal Storage Diseases (ACOPEL for its Spanish Acronym). Both organizations will share in the sales of this book.

About the Editors:

John A. Barranger, M.D., Ph.D. is a Professor in the departments of human genetics, molecular genetics and biochemistry, and pediatrics at the University of Pittsburgh School of Medicine.

He has also served as director of the Human Gene Therapy Applications Laboratory, the Center for the Study and Treatment of Jewish Genetic Diseases, and the Comprehensive Gaucher Disease Treatment Center in this institution

Mario A. Cabrera-Salazar, MD is a Staff Scientist at Genzyme Corporation in Framingham, MA. He is involved in the development of enzymatic and gene therapies for lysosomal storage disorders and for neurodegenerative diseases in the department of Genetic Disease Science.

About the Author

John A. Barranger, M.D., Ph.D., is a tenured professor in the departments of human genetics, molecular genetics and biochemistry, and pediatrics at the University of Pittsburgh.

He is also director of the Human Gene Therapy Applications Laboratory, the Center for the Study and Treatment of Jewish Genetic Diseases, and the Comprehensive Gaucher Disease Treatment Center. Additionally, he is co-director of the Human Gene Therapy Center.

Previously, Dr. Barranger was a clinical associate at the National Institutes of Health (NIH), where, from 1976 to 1978, he served in the clinical investigations and therapeutics section, Developmental and Metabolic Neurology Branch (DMNB), National Institute of Neurological and Communicative Disorders and Stroke. He ultimately became chief of the molecular and medical genetics section of the DMNB and associate branch chief. Dr. Barranger received a doctorate and medical degree from the University of Southern California Los Angeles, and completed an internship and residency in pediatrics at the University of Minnesota.

Dr. Barranger has devoted his professional career to the study of inherited metabolic diseases, particularly lysosomal storage disorders. His research interests include biochemical and molecular genetics, enzyme therapy, gene transfer, and models of genetic disease. As a result of his investigation of the structure of glucocerebrosidase and receptors on macrophages, Dr. Barranger developed successful enzyme replacement therapy for Gaucher disease, an inherited, potentially lethal lysosomal disorder that affects more than 10,000 people in the United States.

This therapy received Food and Drug Administration approval in 1991 and serves as a treatment model for more than 50 related lysosomal storage disorders.

Currently, Dr. Barranger is focused on developing models of inherited diseases to study their pathobiology and treatment by enzyme and gene therapy.
Included among Dr Barranger's honors are the March of Dimes Health Career Award, United States Public Health Service Commendation Medal, Arthur S. Flemming Award and the Scientific Achievement Award of the National Gaucher Foundation.

In addition to consulting for government and industry, Dr Barranger is a member of numerous professional organizations, including the American Society of Biochemistry and Molecular Biology, American Society of Human Genetics, Society for Inherited Metabolic Disorders, and the Society for Pediatric Research.

Mario A. Cabrera-Salazar, MD is a Staff Scientist at Genzyme Corporation in Framingham. MA. He is involved in the development of enzymatic and gene therapies for lysosomal storage disorders and for neurodegenerative diseases in the department of Genetic Disease Science.

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