Gaucher Disease

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In September of 2007 Gaucher Disease received a commendation in the Haematology category of the 2007 British Medical Association Medical Book Competition!

Although rare in the general population, Gaucher disease is the most prevalent of the lysosomal storage disorders, making research into this particular orphan disorder an invaluable prototype for the diagnosis, research, and treatment of others. Like many rare conditions, Gaucher disease has benefited from the explosion of medical research in the last decade, the amount of new information on this disease is staggering and the rate of new discoveries has left previous books on the subject unable to provide useful, up-to-date information.

The most current, fully comprehensive reference to date, Gaucher Disease provides valuable information for academic and industry scientists, and clinicians. Outlining the latest research on the biochemical mechanisms and pathology of lysosomal storage disorders, this book covers diverse topics including animal models, crystallography, imaging and radionuclide evaluation. It not only addresses the developmental basis for current treatments like the now widely available enzyme replacement therapy, but also includes chapters introducing new therapies on the horizon. With contributions from world-renowned experts in substrate reduction therapy, pharmaceutical chaperone therapy, hematopoietic stem cell transplantation and gene therapy, as well as chapters on a second generation of enzyme replacement therapy, this book explores the full spectrum of possibilities offered by the most recent advances in medicine. Some of the most interesting aspects of the book include the discussions on patient management, those touching on the ethics of research, and the societal aspects of treating rare diseases with expensive therapy.

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