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| Autore | Moro Enrico |
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| Sommario/riassunto | Lysosomal storage disorders are a heterogenoeus group of rare genetic conditions affecting worldwide population and often exhibiting severe clinical manifestations. During the last two decades, the joined collaboration between scientists and clinicians has allowed to offer valuable therapeutic options to affected patients. Therefore, the tight connection between basic science and clinical medicine represents the gold standard approach to these disorders. In this context, the present book collects a piece of current scientific advances in the knowledge of disease pathogenesis and in the development of novel diagnostic and therapeutic strategies for some of these diseases. Altogether, these articles define and recapitulate which essential steps are required during the clinical management of a rare inherited disorder and describe forthcoming advances and a breakthrough in the field of lysosomal diseases. |