Introduction to Biological and Small Molecule Drug Research and Development: Chapter 11. Lysosomal storage disorders: current treatments and future directions

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Lysosomal storage disorders (LSDs) represent a group of about 50 genetic disorders caused by deficiencies of lysosomal proteins. The missing lysosomal protein causes a build-up of toxic metabolites in the cells of patients, leading to progressive multisystem disease and premature death. Although individually rare, the combined prevalence of all lysosomal disorders is estimated to be 1 in 8000 births. This chapter describes progress in several different LSD treatment modalities including enzyme replacement therapy, haematopoietic stem cell therapy, chaperone (enzyme stabilization) therapy, and substrate reductions therapy, and highlights new treatment directions for the future.

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