Enzyme replacement therapy for lysosomal storage diseases (Ramot)

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Ruth, Emeritus Navon, T.A.U Tel Aviv University, Medicine-Sackler Faculty, Human Molecular Genetics
Anthony Futerman, Weizmann Institute of Science (WIS)
Etty Osher, Sourasky Medical center
Naftali Stern, Sourasky Medical center
Ari Zimran

Lysosomal Storage diseases (LSD) are inherited genetic defects, resulting in an enzyme deficiency.

This deficiency prevents the lysosome from metabolizing cellular waste, and results in their accumulation in the cell. Excessive storage of proteins, saccharides and/or fats can cause permanent cellular and tissue damage, particularly in the brain, peripheral nervous system, liver, spleen, and bone marrow.

Additional information can be provided upon request.

Contact for more information:

Ariela Markel , VP Business Development, Healthcare , 02-6586608

Ramot at Tel Aviv University Ltd. P.O. Box 39296, Tel Aviv 61392 ISRAEL
Phone: +972-3-6406608
Fax: +972-3-6406675