Washington University in St. Louis
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LYSOSOMAL ENZYME GLCNAC-1-PHOSPHOTRANSFERASE WITH ENHANCED ABILITY TO PHOSPHORYLATE LYSOSOMAL ENZYMES

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Lysosomal storage disorders result in severe, multi-system dysfunction and frequently, early death. Currently, a number of lysosomal storage disorders are treated with enzyme replacement therapy (ERT), which requires periodic infusions of the deficient enzyme. However, in a number of instances the content of the mannose-6-phosphate, which is critical for lysosomal uptake and use of the enzyme, is low in the replacement enzymes, thus impairing the efficacy of the product. Dr. Kornfeld has developed a mutated lysosomal enzyme GlcNAc-1-phosphodiesterase which produces lysosomal enzymes for ERT that has a higher content of mannose-6-phosphate. By significantly increasing mannose-6-phosphate, this should improve the uptake of these critical lysosomal enzymes by the deficient cells of affected patients and make ERT more effective for a variety of lysosomal storage disorders including but not limited to Fabry disease, Pompe disease, Alpha-mannosidosis, and Gaucher's Disease.