Phoenix

Demo Case 4
NanoGLA

ICMAB INSTITUT DE CIÈNCIA DE MATERIALS DE BARCELONA
EXCELENCIA SEVERO OCHOA CSIC

NANOMOL TECHNOLOGIES

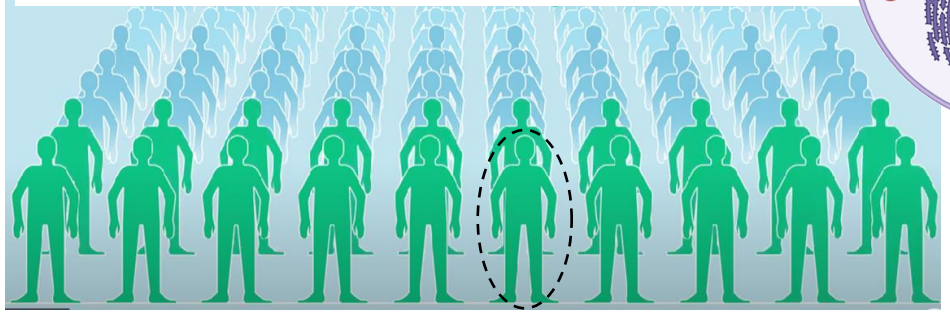
Leanbio

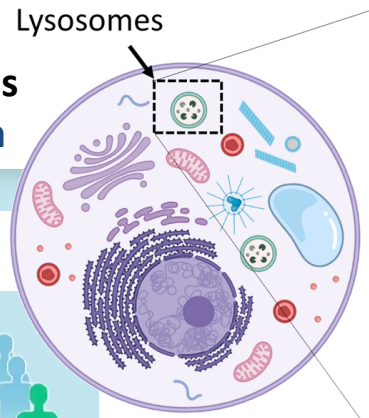
State of Art and Innovative solution

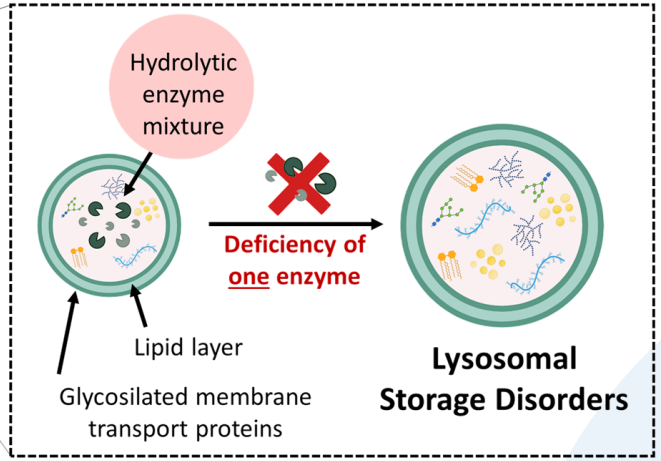
Lysosomal Storage Disorder's family > Fabry Disease

As a group, the cumulative incidence of LSDs represents a Serious global Health Problem

≈ 80,000 worldwide → ≈ 1,000,000 worldwide





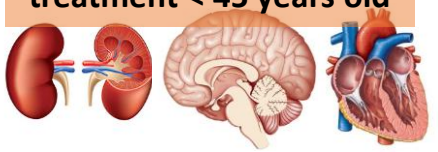


mainly treated by enzyme replacement therapy (ERT)

IV administration of the missing enzyme

- Poor enzyme biodistribution
- **No Blood Brain Barrier crossing**
- Need of frequent doses (EOW)
- High-cost treatment (>280 k€/year)

Life expectancy without treatment < 45 years old

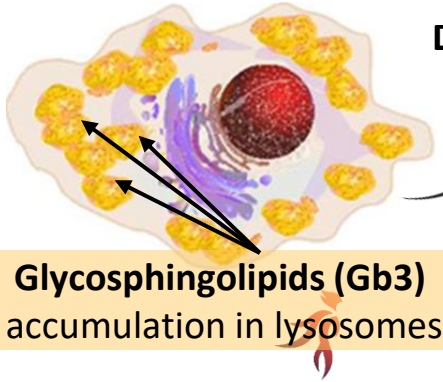


Dysfunctional organs
(kidney, heart, brain…)

deficiency/lack of a single protein (enzyme)

FABRY DISEASE

α-Galactosidase-A enzyme (GLA)

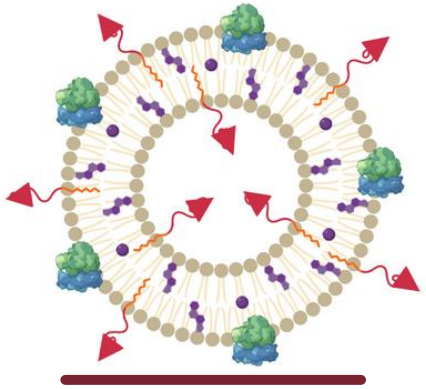


Glycosphingolipids (Gb3) accumulation in lysosomes

GA No 953110

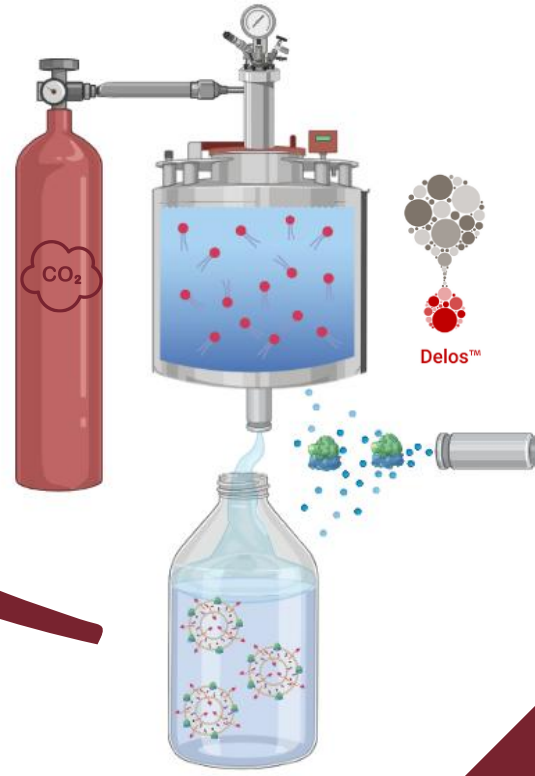
State of Art and Innovative solution

nanoGLA



RGD peptide targeting unit (Arginine-Glycine-Aspartic)
to enhance intracellular delivery (30-fold in vitro)

Active rh-GLA enzyme
α-galactosidase A, 110 KDa
(> 90% entrapment efficiency)



Orphan Medicinal Product Designation by EMA in 2021

PROTECTION

INTEGRIN TARGETING

INTRACELLULAR DELIVERY

IP protection of nanoGLA through a patent application, PCT/EP2022/051727 (2021)

J. Tomsen-Melero et al., *Targeted nanoliposomes to improve enzyme replacement therapy of Fabry disease,* Sci. Adv. 10, eadq4738 (2024)

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