

# EFFICACY AND SAFETY OF HIGH-DOSE TWICE WEEKLY SEBELIPASE ALFA IN SEVERE-ONSET WOLMAN DISEASE: A CASE REPORT

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Case studies - with patient consent

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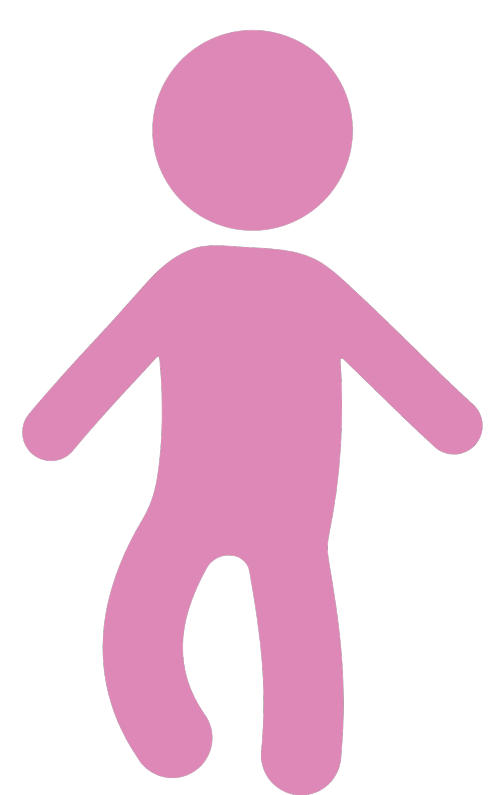
## BACKGROUND AND IMPORTANCE

- **Lysosomal-acid-lipase (LAL) deficiency** is a rare metabolic disease (0.2:10,000) characterized by lysosomal accumulation of cholesterol esters and triglycerides, with a severe and rapidly progressive form, Wolman Disease (WD), usually fatal in the first 6-12 months of life.
- **Sebelipase-alfa (SA)** is a recombinant human LAL administered weekly, initially at 1 mg/kg, with a gradual increase according to response, thus avoiding serious **hypersensitivity** reactions. Twice weekly dosing with rapid escalation had not been previously described.

## OBJECTIVE

To describe the **efficacy** and **safety** of high dose SA administered **twice weekly** in severe onset WD.

## METHODS

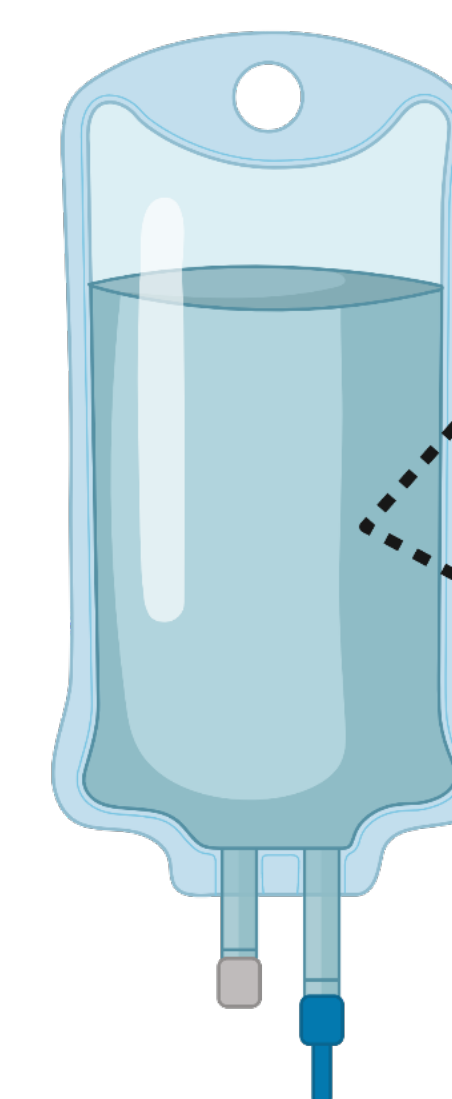


3-month-old woman

- **Diagnosis: WD** with secondary **hemophagocytic syndrome**.
- Admission to **paediatric critical care unit**.
- Since admission: anaemia, thrombocytopenia, hyperferritinemia, altered liver function tests and lipid profile, and massive hepatosplenomegaly.

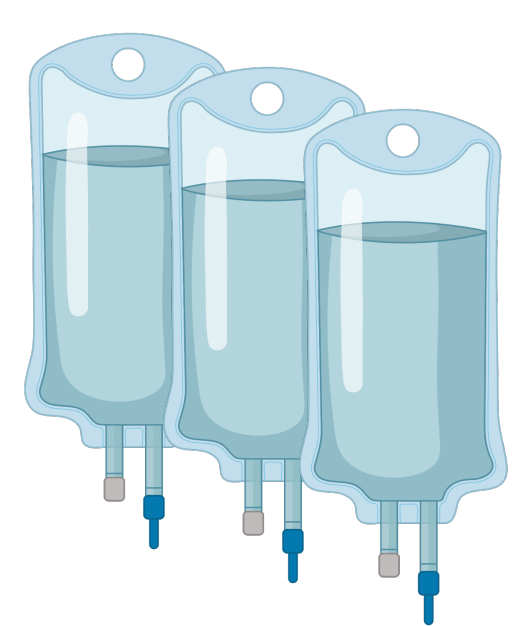


**Rapid deterioration and critical situation**, with severe **respiratory** and **kidney failure** → High dose SA was started



**Sebelipase alfa**  
First dose: 3 mg/kg  
Subsequent doses: 5 mg/kg, twice weekly  
IV infusion over 240 min

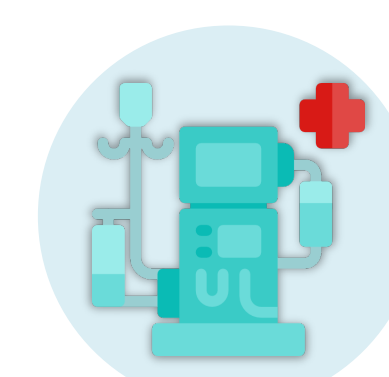
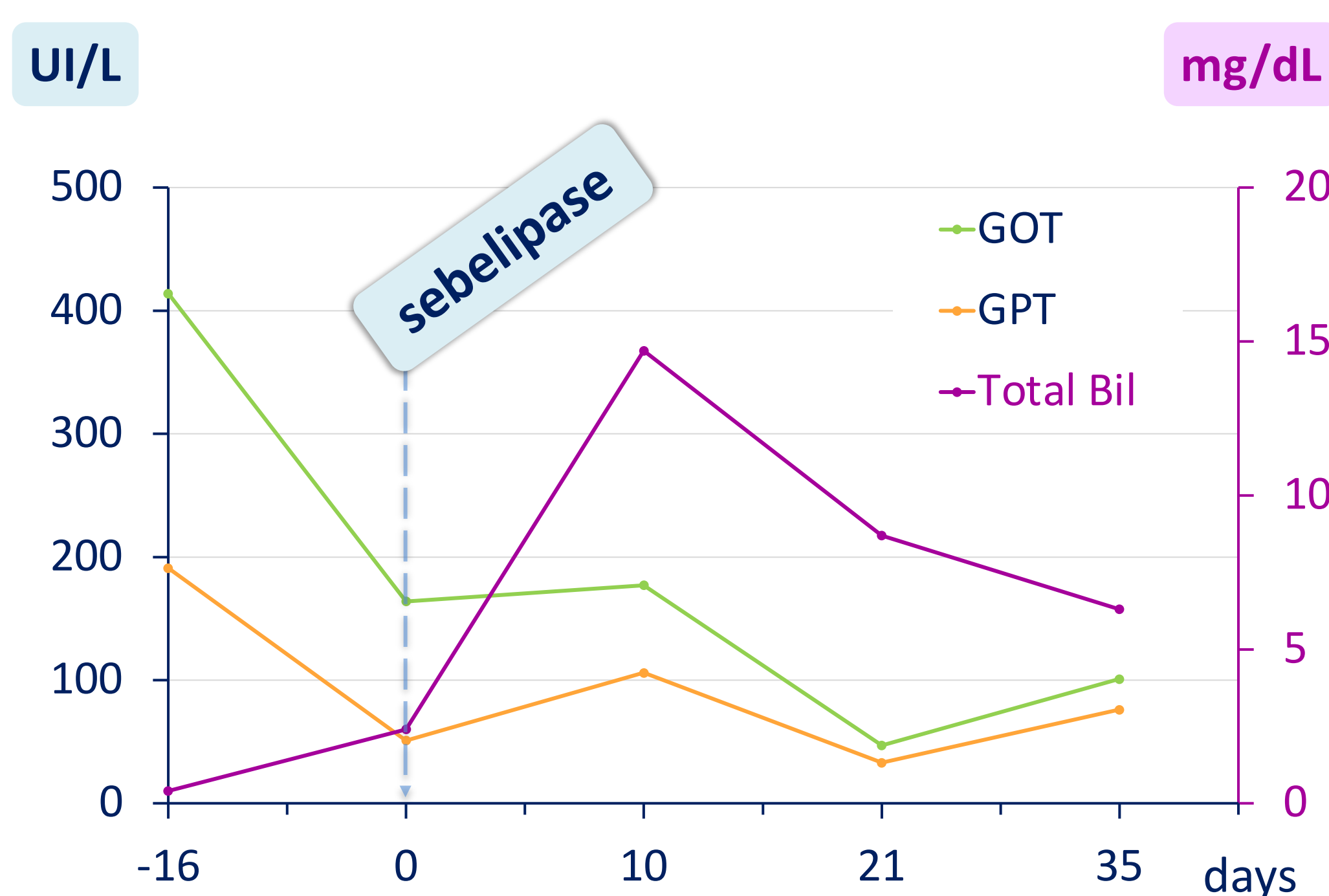
## RESULTS



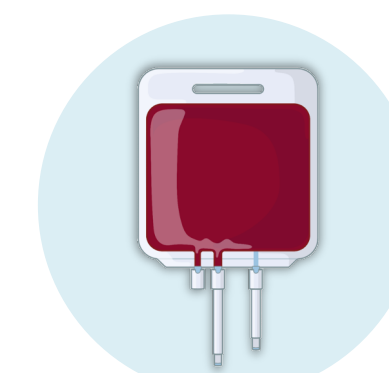
11 doses of sebelipase alfa over 35 days



No adverse effects were reported



**Mechanical ventilation and continuous hemodiafiltration:** required for 2.5 weeks



**Red blood cell and platelet transfusions** were needed repeatedly up to day +24



A **reduction in hepatosplenomegaly** was noticeable after one month



**Lipid profile** didn't reach normal values after 11 doses of SA

## CONCLUSION

Treatment with **high-dose twice weekly SA** has been an **effective** and **well-tolerated** therapy in an aggressive and severe presentation of WD so far, although it is necessary to maintain enzyme replacement for life