

ENZYME  
REPLACEMENT  
THERAPY  
(ERT)

**FOR GM1**



**CUREGM1**  
FOUNDATION

# CURE GM1 HAS ENABLED THERAPEUTIC DEVELOPMENT IN GM1 GANGLIOSIDOSIS

## ENZYME REPLACEMENT THERAPY IS A PROVEN MODALITY IN LYSOSOMAL STORAGE DISEASE

- 17 ERTs have been approved for 12 LSDs with a remarkably high approval rate

## PROOF OF CONCEPT HAS BEEN DEMONSTRATED

- POC in two mouse models of disease
- Substrate reduction to near normal levels
- Behavioral analysis ongoing

## CURE GM1 IS LAYING THE GROUNDWORK TO IND

- Have identified a high producing cell line
- Currently manufacturing at small scale; plans in place for scale-up to generate material for IND-enabling tox and the clinic
- Pre-IND meeting planned for early 2026

PROJECTED WORLDWIDE REVENUE OF >\$300M FOR GM1 ERT

## SUPPORT US



GIVE TO SUPPORT  
GM1 ERT

[curegm1.org/donate](https://curegm1.org/donate)

## LEARN MORE

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GM1 ERT

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