

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
- INTERACT meeting planned for early 2026

PROJECTED WORLDWIDE REVENUE OF \$300M-\$400M FOR GM1 ERT

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