A Global Roadmap for Sanfilippo Syndrome Therapies

Sanfilippo syndrome is a rare and devastating form of childhood dementia.

There is currently no approved treatment or cure for this genetic condition.

Significant advances have been made, yet without further research, most individuals with Sanfilippo will never reach adulthood.

We're striving for a world without Sanfilippo. This roadmap aims to rapidly deliver solutions for all affected families.

STOP

disease progression

- ✓ Deliver gene, enzyme & other therapies as early as possible
- ✓ Enhance delivery of therapies to the brain & avoid the immune system
- ✓ Broaden options for ineligible patients & neglected subtypes

TREAT

the impact on the brain and body

- ✓ Pursue treatments to target brain cell function, inflammation & damage
- ✓ Understand influencers of disease severity & therapeutic windows
- ✓ Explore combination therapies

MANAGE

symptoms and quality of life

- ✓ Clinical & family guidelines to optimise management
- ✓ Research to harness families' lived experience
- ✓ Further understand impact & treatment of peripheral symptoms

ENABLERS *for success*

Data

NATURAL HISTORY STUDIES

PATIENT REGISTRY & CLINICAL DATA

INITIATIVES TO LINK AND SHARE DATA

Tools

CLINICAL TRIAL DESIGN

OUTCOME MEASURES & BIOMARKERS

PROGNOSTIC TOOLS

BIOLOGICAL SAMPLES

DISEASE MODELS

Connection

ADVOCACY FOR NEWBORN & CARRIER SCREENING

COLLABORATION & COMMUNICATION

SYMPOSIA & FOCUSED WORKSHOPS

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