International Registry of Individuals with Lysosomal Storage Diseases

Inclusion:

Any diagnosis of a lysosomal storage disease

Parents, patients, or providers caring for a patient with a lysosomal storage disease may contact:

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What does participation require?

- 30-minute call with study team
- Ongoing permission for medical records review

ClinicalTrials.gov: NCT05619900

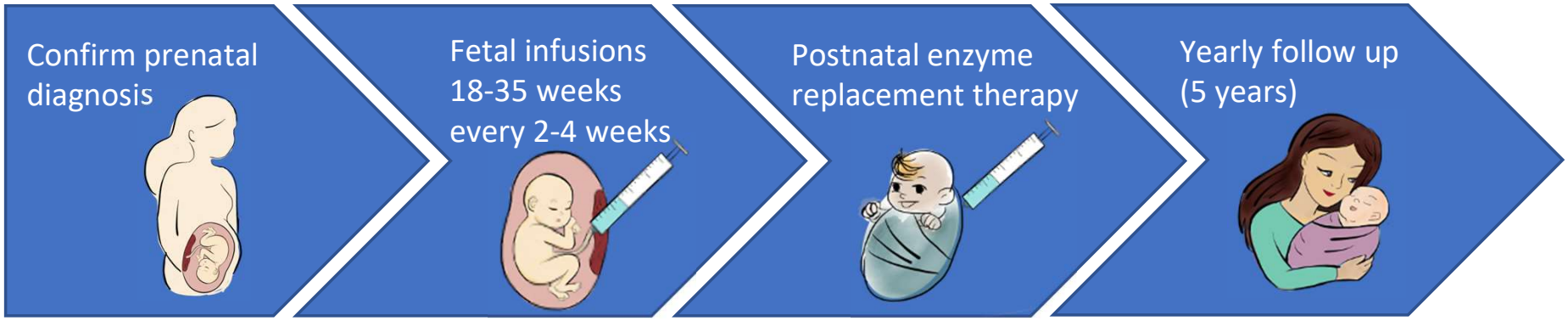
Program Goals

- Identify patient outcomes of therapies
- Improve the care of patients with lysosomal storage diseases
- Improve medical decision making
- Improve prenatal diagnosis
- Enable future cures

UCSF Benioff Children's Hospitals
Center for Maternal-Fetal Precision Medicine

In Utero Enzyme Replacement Therapy (ERT) for Lysosomal Storage Diseases

Phase 1 Clinical Trial at the University of California, San Francisco



Included Diseases

- MPS 1, 2, 4a, 6, 7
- IOPD
- Gaucher disease (types 2/3)
- Wolman disease

Rationale for Prenatal Therapy

- Prevent in utero demise
- Induce tolerance to missing enzyme
- Deliver enzyme to the brain
- Prevent organ damage

Primary Objectives

- Safety
- Feasibility

Learn more about our clinical trial
In Utero Enzyme Replacement Therapy (IUERT)
ClinicalTrials.gov: NCT04532047

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