

# UCB Transplant of Inherited Metabolic Diseases With Administration of Intrathecal UCB Derived Oligodendrocyte-Like Cells

NCT02254863

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Status	RECRUITING
Phase	Phase 1
Sponsor	Joanne Kurtzberg, MD
Enrollment	40 participants

## Key Eligibility Criteria

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### Inclusion (16)

- Patients must be age e1 week to d21 years.
  - Patients must have one of the following inherited metabolic diseases detected by enzyme or mutation analysis, and confirmed by repeat testing on a separately obtained sample:
    - Adrenoleukodystrophy (ALD) Batten Disease Hunter Syndrome (MPS II) Krabbe disease (Globoid Leukodystrophy) Metachromatic Leukodystrophy (MLD) Niemann Pick disease type A or B Pelizaeus-Merzbacher disease (PMD) Sandhoff disease Tay Sachs disease. Alpha Mannosidosis Sanfilippo (MPS III)
  - Patients must have neurologic evidence of their disease, either clinically or via neuroimaging or neurophysiological testing. Examples of evidence of neurologic involvement include, but are not limited to the following:
    - Abnormal EEG, Brainstem Auditory Evoked Response (BAER), and/or Visual Evoked Potentials (VEP).
- ... and 11 more (see full listing online)

### Exclusion (12)

- Prior organ, tissue, or stem cell transplant within 3 years of study entry.
  - Prior participation in any gene or regenerative cell therapy study.
  - Inability to have an MRI scan or lumbar puncture.
  - Intractable seizures.
  - Chronic aspiration.
- ... and 7 more (see full listing online)

## Locations (1 total)

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Duke University Medical Center, Durham, North Carolina, United States