Sant Joan de Déu
Barcelona · Hospital

Dear colleague,

We have opened a clinical trial that recruits patients for an open-label study in male pediatric patients with cerebral X-LINKED ADRENOLEUKODYSTROPHY (cALD) to assess the effect of a new experimental treatment on the progression of cerebral lesions. The primary objective is to evaluate whether this therapy can halt disease progression if administered prior to HSCT.

The study will last about 2 years, and will take place at Sant Joan de Deu Hospital (Barcelona, Spain) with periodic visits, during which we'll perform brain MRI, blood sampling, cardiological and neurological assessment.

Inclusion Criteria are:

- Males aged ≥2 and ≤12 years with a diagnosis of X-linked ALD based on genetic testing
- White matter involvement as determined by cerebral MRI lesions without Gadolinium enhancement at baseline, or with Gd enhancement at baseline.
- Mild or absent neurological symptoms (determined by key measures in the Neurological Function Scale assessed during the baseline visit)
- Loes score >0 and ≤10, GIS ≤2 (assessed during the screening visit)
- No signs or symptoms of adrenal insufficiency and morning cortisol and aldosterone levels within normal laboratory ranges for age, or appropriate steroid replacement if adrenal insufficiency is present. A history of adrenal insufficiency is not exclusionary if the foregoing is currently met.
- Glycated hemoglobin (HbA1c) within normal range

Please, let us know if you can provide interested and eligible patients. You can contact Drs. Chiara Alfonsi: chiara.a.86@hotmail.it and García-Cazorla: agarcia@hsjdbcn.org

Thank you in advance.
Best regards.

Neurometabolic Unit-Hospital Sant Joan de Déu, Barcelona