

MODULATORS OF THE INTERFERON SIGNALLING PATHWAY FOR USE IN THE TREATMENT AND PREVENTION OF X-LINKED ADRENOLEUKODYSTROPHY (X-ALD)

A research group from CIBER, IDIBELL and Institutó Catalana de Recerca I Estudis Avançats has identified a novel therapy for the treatment of X-linked Adrenoleukodystrophy

The Need

X-ALD is a clinically heterogeneous disorder, leading to VLCFA accumulation and potentially severe cerebral inflammation, demyelination, neurodegeneration, and adrenal dysfunction. Current treatments (HSCT and ex-vivo gene therapy) are limited to selected patients at early stages and do not halt axonal damage, highlighting the unmet need for broadly applicable pharmacological therapies.

The Solution

The inventors have identified the potential of modulators of the interferon signalling pathway for the use in the treatment and prevention of X-ALD.

Innovative Aspects

- ✓ IFN pathway architecture provides multiple therapeutic intervention points.
- ✓ The invention also provides novel in-vitro methods for monitoring the severity and progression of X-ALD.
- ✓ The invention also provides in-vitro methods for screening and identifying potential candidate therapeutic compounds.

Stage of Development:

In vitro and post-mortem patient functional studies:

- Analytic validation in human samples from patients (post-mortem cALD brain).
- Mechanistic studies in X-ALD fibroblasts.

Intellectual Property:

- European patent application in 2026.
- Suitable for international extension (PCT application)

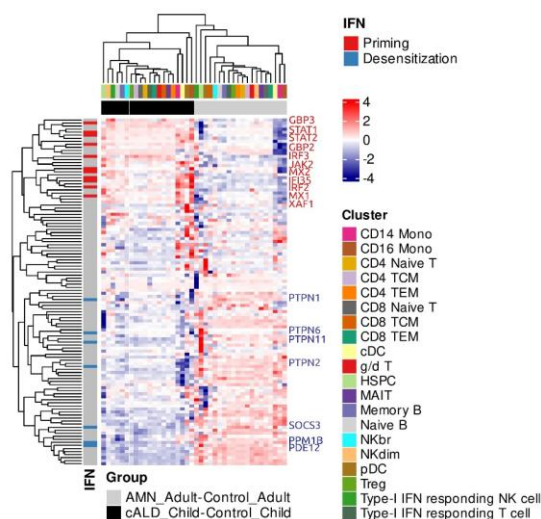


Figure 1. Phenotype specific IFN signature, priming and desensitization scores

Aims

Looking for a partner interested in a license and/or a collaboration agreement to develop and exploit this asset.

Contact details