TECHNO OFFER

Treatment of rare Menkès disease

Menkès - Orphan disease - bioavailability of copper

CONTEXT

Menkès disease is a rare pathology of genetic origin which occurs at a rate of one in 300,000 births, i.e. two to three births each year in France.
It is characterized by a deficiency in ATPase 7A, which allows copper to pass through the intestine and the blood-brain barrier. This leads to a deficiency of cuproproteins, responsible for the severe brain damage of these patients.
Without treatment, their lifespan is less than 3 years and current treatments do not allow the passage of copper to the brain.

DESCRIPTION

The laboratory has developed new copper carriers immobilised on a support in order to make them biocompatible and stable.
Their stability has been tested and is more than 10 weeks.

In vivo, subcutaneous administration of these transporters reverts the Menkès phenotype in models of Menkès mice. At this stage, no toxicity is observed.

COMPETITIVE ADVANTAGES

New therapeutic alternative

- **Crossing of the blood-brain barrier and delivery of copper to the brain**

Stability

- **Subcutaneous implantation of a specific support allowing the delivery without repeating the injections.**

Markets & applications

Phamaceuceutical

- **Menkès disease**

Development stage

TRL 3 in vivo studies in progress

Intellectual property

In progress

Target partnership

Patent licensing

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