

clinical trial on Vanishing White Matter disease (VWM, CACH syndrome)

Objectives	Study the effect of an antioxidant molecule, guanabenz, on children with VWM.
Budget	€2 million, including €500,000 financed by ELA
Duration	4 years
Description	<p>Analysis of the safety and efficacy of guanabenz in delaying or halting disease progression in children with early-onset VWM.</p> <p>The trial aims to include 40 patients under the age of 10.</p> <p>Project partner: D' Marjo van der Knaap, University of Amsterdam VUmc, Netherlands.</p>
Impacts	<p>The goal is to benefit all children with early-onset forms of the disease.</p> <p>Expected results include improving the patients' quality of life and potentially restoring their mobility.</p> <p>This represents the first treatment trial available to these patients.</p>



"A promising clinical trial on CACH syndrome" video

https://youtu.be/uF2x4Nk_1rQ

