

PRESS RELEASE

‘JAMA Neurology’ publishes the results from Phase 1b/2a clinical trial of the neuroprotective drug ApTOLL for ischemic stroke

- The results from APRIL study, previously presented at the International Stroke Conference 2023, indicate that ApTOLL showed good safety in 151 acute ischemic stroke patients.
- The article also highlights that the preliminary efficacy results show that ApTOLL significantly reduces mortality and long-term functional disability.
- ApTOLL will enter in late-stage trials for ischemic stroke.

Madrid (Spain), 20 June 2023 – The prestigious scientific journal *JAMA Neurology* has published today the positive results from APRIL, the Phase 1b/2a clinical trial from [aptaTargets](#), which has evaluated the safety and efficacy of the groundbreaking neuroprotective drug **ApTOLL** in combination with endovascular treatment (EVT) in patients with acute ischemic stroke.

The **results from the study have proven to be clinically relevant**: the administration of 0.2 mg/kg of ApTOLL within 6 hours after acute ischemic stroke in combination with EVT was safe and well tolerated, and it was also associated with a reduction in mortality from 18% to 5% along with a reduction in functional disability at 90 days compared to the placebo.

APRIL was a double-blind, randomized, multicenter, placebo-controlled, Phase 1b/2a clinical trial conducted in 14 comprehensive stroke centers¹ in Spain and France, which started in 2020. In total, the study included **151 patients**: 32 in Phase 1b and 119 in Phase 2a. The study population were men and non-pregnant women between 18 and 90 years old with an incapacitating stroke at the time of randomization.

Patients received randomly ApTOLL or placebo intravenously prior to thrombectomy. The total population analyzed were 139 patients, from them 42 patients received 0.05 mg/kg of ApTOLL, 42 received 0.02 mg/kg of ApTOLL, and 55 received placebo. The primary endpoint was the safety as determined by death, symptomatic intracranial hemorrhage, malignant stroke and recurrent stroke. The secondary endpoints, concerning efficacy, included the mean final infarct volume measured at 72 hours, the stroke symptoms severity assessed by the National Institutes of Health (NIH) Stroke Scale at 72h, and disability at 90 days in terms of modified Rankin Score (mRS).

“The recently published data in *JAMA Neurology* and presented in February in the main opening session of the [International Stroke Conference](#) as innovative neuroprotectant agent with promising potential to improve the treatment of ischemic stroke and other CNS disorders” explains **Dr Marc Ribó**, interventional neurologist from the [Vall d’Hebron University Hospital](#) in Barcelona, principal investigator of the [Vall d’Hebron Research Institute](#) (VHIR) Stroke Research Group, Chief Medical Officer of [aptaTargets](#) and one of the lead authors of the article.



Stroke affect 1 out of every 4 adults over age 25 throughout their lives. This figure emphasizes that the disease is the second leading cause of death and the main cause of adult disability. **Acute ischemic stroke** accounts for 85% of all strokes. Although in recent years reperfusion treatments have been shown to consistently achieve arterial recanalization in 85% to 90% of cases, more than 50% of patients treated develop a moderate to severe disability. In this context, there is a clear medical need to develop new drugs with a neuroprotective effect to improve the results of stroke in parallel with reperfusion treatments.

“ApTOLL is the first neuroprotective drug that achieves positive efficacy results in clinical trials on ischemic stroke, showing a strong anti-inflammatory action by the inhibition of TLR4 receptor, and significantly reducing mortality and long-term disability”, highlights **Dr Macarena Hernández Jiménez**, Chief Scientific Officer of aptaTargets and first author of the article.

“The fact that one of the most prestigious scientific journals in this field is publishing these results highlights the importance of ApTOLL. The APRIL II study is currently being planned and expected to start by Q1 2024”, comments **David Segarra**, CEO and co-founder of aptaTargets.

ApTOLL obtained priority medicines (PRIME) designation from the European Medicines Agency (EMA).

Reference article:

Safety and Efficacy of ApTOLL in Patients with Ischemic Stroke Undergoing Endovascular Treatment

Hernández-Jiménez M, Abad-Santos F, Cotgreave I, Gallego J, Jilma B, Flores A, Jovin T, Vivancos J, Hernández-Pérez M, Molina C, Montaner J, Casariego J, Dalsgaard M, Liebeskind D, Cobo E, Castellanos M, Cardona Portela P, Masjuán J, Moniche F, Tembl J, Terceño Izaga M, Arenillas J, Calleja P, Olivot J, Calviere L, Henon H, Mazighi M, Piñeiro D, Pugliese M, González V, Moro M, García-Tornel A, Ribó M. *JAMA Neurology*, 2023. DOI: 10.1001/jamaneurol.2023.1660

.....

About aptaTargets

aptaTargets is a clinical stage biopharmaceutical company specialised in developing innovative therapies based on aptamer technology. Its lead drug is ApTOLL, a molecule with an immunomodulatory and anti-inflammatory effect that is being researched for acute ischemic stroke (completed Phase 1b/2a), in patients with suspected ischemic stroke in ambulances (Phase 2a), myocardial infarction (Phase 2a), intracerebral hemorrhage, and multiple sclerosis. Founded in 2014, aptaTargets is based in Madrid, Spain, and has successfully raised 17 million euros, including investments from venture capital (Inveready and CDTI through the Invierte programme are the current investors of reference), in addition to different competitive programmes in the European Union and the Spanish Ministry of Science and Innovation. More information: aptatargets.com

.....

¹ Vall d’Hebron University Hospital in Barcelona (Spain), La Princesa University Hospital in Madrid (Spain), Germans Trias i Pujol University Hospital in Badalona (Spain), A Coruña University Hospital Complex



(Spain), Bellvitge University Hospital in L'Hospitalet de Llobregat (Spain), Ramón y Cajal University Hospital in Madrid (Spain), Virgen del Rocío University Hospital in Sevilla (Spain), La Fe University Hospital in Valencia (Spain), Doctor Josep Trueta University Hospital in Girona (Spain), University Clinical Hospital of Valladolid (Spain), 12 de Octubre University Hospital in Madrid (Spain), Toulouse University Hospital (France), Hospital University Hospital of Lille (France) and Hôpital Fondation Adolphe de Rothschild (France).

.....

Media contact:

Gemma Escarré Comms
aptatargets@gemmaescarre.com
M +34 667 76 15 24

Follow us on [LinkedIn](#) and [Twitter](#)