

Promising Down syndrome research expands to second site in Brazil

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Credit: Case Western Reserve University

A Case Western Reserve University-led study of a promising medication for people with Down syndrome is going global.

Brazilian authorities' recent approval of a clinical trial in São Paulo not only increases the diversity of participants in the project begun in Cleveland, but also provides an opportunity to double their number. These two factors combined mean that the ultimate results will be that much more credible—and valuable—to pursuing successful treatments.

The trial is assessing the potential cognitive-enhancing effects of the drug memantine on adolescents and young adults with Down syndrome.

Leading the study is professor of pediatrics Alberto Costa, already renowned for his earlier pioneering work on Down syndrome, as well as his deep empathy for affected families. Costa, who grew up in Brazil, began pursuing this research just over two decades ago—after his daughter was born with the condition.

The current clinical trial has one additional connection to Brazil: It is funded through a nearly \$2.6 million grant from the Alana Foundation, part of Alana, a nonprofit organization based in São Paulo. The foundation previously awarded a grant of more than \$650,000 to support Costa's research on different medications' effects on Down syndrome with animal models.

"We are delighted that Dr. Costa's critical work will extend to our home nation and city," said foundation program officer Claudia Moreira. "By taking this project internationally at this pivotal stage of research, we hope to accelerate and broaden the potential impact of positive findings."

Alana engaged with Costa after he published results of a pilot trial that assessed the effects on brain function of memantine, an FDA-approved drug used to treat signs and symptoms of Alzheimer's disease. That study showed dramatic benefits of memantine on animal models, and was followed by another that demonstrated significant, albeit modest effects in a small cohort of adults who underwent memantine treatment.

This new, larger study, involving 200 participants equally split between the Cleveland and São Paulo sites, seeks to build on findings from the first clinical trial, which ultimately could lead to a much larger, final phase of testing involving at least five sites.

Costa and his team began the U.S.-based part of the trial about two years ago, and to date have recruited about 35 of its 100 participants. Roughly one-third the size of Cleveland's metropolitan area, the Greater São Paulo region has a population nearly 10 times that of Northeast Ohio. The team at Brazil's Hospital Israelita Albert Einstein, led by Ana Claudia Brandão, has just recruited the first 10 participants for the Brazil-based part of the study. The hospital will begin the treatment in October, when the trial medication arrives.

"Beyond the access to additional participants," Costa said, "having the world-class Albert Einstein hospital as our partner is another huge benefit to this important research."

The Hospital Israelita Albert Einstein and Case Western Reserve University also have recently signed a memorandum of understanding to formalize a strategic partnership in the areas of education and research.

The current study consists of 16-week randomized [trials](#), with 16 participants at a time at each site. Half receive the drug memantine, and the rest a placebo. The entire cohort then participates in neuropsychological and clinical evaluations, combined with advanced brain imaging.

"The study of brain development is one of the stalwarts of research at Albert Einstein Hospital," said research leader Luiz Vicente Rizzo. "This specific initiative is very dear to us because of what it may represent in the advancement of science, but also because it is the first joint project with Case Western Reserve University in what we hope will be a long-lasting relationship in the field of health education, research and innovation."

Among the Alana foundation's earlier grants to Costa is a \$1.7 million award—matched with a grant of the same amount to the Massachusetts

Institute of Technology. That support led to Case Western Reserve's announcement this spring that his team had developed a novel noninvasive technique to create stem cells necessary for studying Down syndrome. The cells, obtained from urine samples, can be grown into many cell types that can more reliably test new medications that may enhance the quality of life of people with Down syndrome.

Navigating the intricacies of another country's research review process took much longer than Costa anticipated, which made securing the approval late this spring all the more rewarding.

"When I saw the email confirming the second site," Costa said, "I was jumping up and down."

Provided by Case Western Reserve University

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