

First clinical trial of a new drug to improve the cognitive function of people with Down syndrome

- *The Hospital del Mar Medical Research Institute is leading the study that will evaluate whether a new molecule can be used to improve the cognitive performance of people with Down syndrome*
- *The study is part of the ICOD project for the improvement of cognition in Down syndrome, promoted by the European Union, which is funding a project of this type for the first time. The treatment has already passed the trial phase with volunteers without Down syndrome, which sought to certify the safety and tolerability of the medicine*
- *In this new phase, the safety of the treatment in people with Down syndrome will be validated and the first indications of effectiveness will be obtained. It is estimated that results may be available by mid-2023*

Barcelona, December, 2nd 2022. – The Hospital del Mar Medical Research Institute (IMIM-Hospital del Mar) is launching a clinical trial (Phase Ib) to validate the safety and tolerability of a **new treatment to improve the cognitive function in people with Down syndrome and preliminarily evaluate its effectiveness.** The clinical trial is part of the **ICOD project** (Improving Cognition in Down Syndrome) promoted by the European Union within the framework of the [Horizon 2020 R&D funding program](#), with a grant of six million euros, and led by the IMIM-Hospital del Mar. It is the first study that analyzes the feasibility of a new type of approach in this field.

To carry it out, the IMIM-Hospital del Mar team, led by Dr. Rafael de la Torre, coordinator of the ICOD project, will recruit 45 people aged between 18 and 35 with Down syndrome. This phase of the study comes after passing tests with animals and with volunteers without Down syndrome, which have indicated the safety and tolerability of the treatment.

New approach

This treatment is based on modulating the functioning of the brain's endocannabinoid system. According to several studies, people with Down syndrome have hyperactivated the **CB1 cannabinoid** receptor, and its modulation with specific inhibitors dramatically improves cognitive performance in animal models. Based on this principle, the French company Aelis Farma has developed an innovative approach, through a new molecule, called AF0217, the first of its kind, which acts by imitating a natural defense mechanism of the brain to counteract hyperactivity of this receptor.

Dr. Rafael de la Torre has pointed out that with the study that is now underway **"we will not only demonstrate that the product is safe and well tolerated by people with Down syndrome, but we have tools to monitor whether the treatment has positive effects in cognition these people"**. The previous phases of the clinical trial, which have been approved by the Spanish Agency for Medicines and Health Products (AEMPS), have shown that the molecule developed is safe and well tolerated, with no significant side effects. This is **"of particular importance for the acceptance of treatment by the families of people with Down syndrome"**, he adds.

The volunteers who will participate in the study will be divided between those who will take the medicine, an oral solution that is taken with water, and those who will receive a placebo. The study will last 28 days, during which time their response to treatment will be monitored. Before



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starting it and at the end, they will be administered cognitive performance tests and an electroencephalogram to be able to have a first view of how the treatment affects their cognitive abilities.

Once the results of this phase of clinical development are obtained, a new study will be planned, this time with several centers from all over Europe and with a larger number of participants, to confirm the effects of the new molecule on cognitive performance of people with Down syndrome (Phase II study). The intention of the driving team is to launch it in the summer of next year. The final goal is to achieve a treatment that improves the working memory of these people, a key cognitive deficit in this pathology. Working memory refers to the set of processes that allow the storage and temporary manipulation of information for the performance of complex cognitive tasks, such as language comprehension, reading, mathematical skills, learning or the reasoning. It is not ruled out that the mechanism of action of the new treatment could be useful in other intellectual disabilities, such as Fragile X syndrome, or other diseases that cause cognitive impairment such as neurodegenerative diseases.

Concerning the start of the clinical trial, Dr. Joaquín Arribas, research director of the Parc de Salut Mar and director of the IMIM-Hospital del Mar, wanted to highlight that ***"the Hospital del Mar Medical Research Institute is not just one of the pioneering research centers in the State, but rather its commitment to front-line research bears important fruits. In this way, 155 clinical trials were carried out or started in the year 2021 alone, especially in oncology and translational clinical research. The one currently being carried out on people with Down syndrome will focus on an area where there has been no progress in recent years: the quality of life of these people. To all this, we must add the recent announcement of the European Medicines Agency to trust the Hospital del Mar and the IMIM to evaluate medicines using real-life data."***

People who want more information about the ICOD project and the clinical trial can find it on the website <https://www.imim.cat/estudis-clinics/17/assaig-clinic-en-persones-amb-sindrome-de-down-for-the-improvement-of-cognitive-functions>

And about the ICOD project <https://www.icod-project.eu/es/home>

More information

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