Hospital del Mar Research Institute Barcelona

Press release

Positive results of the clinical trial of a drug to improve cognitive function in Down syndrome

- ***Phase 1/2 of the clinical trial shows that the administration of the AEF0217 molecule developed by the biotech Aelis Farma, is safe and can improve key skills such as communication, social interactions and daily living for people with Down syndrome***
- ***These results reinforce the start of the phase 2 clinical trial, through an international multicenter study with people with Down syndrome, focused on the dose necessary to achieve the treatment goal***

Barcelona, December 17th of 2024. – The **ICOD** (Improving Condition in Down syndrome) project, a pioneering study in addressing the cognitive difficulties associated with **Down syndrome**, has demonstrated the **safety of treatment** with the **molecule AEF0217, developed by the French biotech Aelis Farma,** as well as its **effectiveness in improving cognitive function in these people**. The study was led by the Research Institute of the Hospital del Mar.

This phase of the trial (phase 1/2 of the project), funded by the European Union under the Horizon 2020 R+D programme and Aelis Farma, has been carried out with 29 people with Down syndrome between 18 and 35 years old, with mild or moderate disability. The objective was to test the safety of the administration of the treatment, as well as to explore the potential to improve the cognition and daily functionality of the participants. In a previous phase, its safety in people without this syndrome had already been validated.

To carry out the study, the volunteers were given the molecule AEF0217, developed by Aelis Farma, or placebo, for 28 days. The results, which are now being released, show both the safety of the treatment and the improvement in cognitive functions in people who received it compared to those who received placebo.

Measured with reference scales, it has been found that **AEF0217 significantly improved behavioural skills in the areas of communication, daily living skills and social interactions**. These improvements were also associated with a consistent trend towards greater cognitive flexibility, which is the ability to adapt to new situations or changes.

In addition, studies with electroencephalography, which look at spontaneous brain electrical functioning, show statistically significant changes in brain function after AEF0217 treatment, indicating that people with Down syndrome needed less effort to complete a work memory tasks. These results reinforce the idea that the treatment acts effectively at the brain, cognitive and functioning levels, providing initial promising concordant data on its beneficial potential.

Dr. Rafael de la Torre, who coordinates the ICOD project en conducted the clinical studies at the Hospital del Mar Research Intitute with Dr. Ana Aldea, explains that ***"the promising and impressive results of this study generate real hope of developing a safe and effective treatment for cognitive dysfunctions in people with Down syndrome".*** And he adds that ***"the effectiveness data are particularly striking, addressing crucial domains of adaptation, such as expression and writing skills, as well as daily living skills and social interactions. These effects, obtained after only four weeks of treatment, are the first in the field of Down syndrome and represent an important step forward towards the development of a treatment that could significantly improve the autonomy and adaptation of these people***".

Down Catalunya, the entity representing the Down association in Catalonia and which has collaborated with the Hospital del Mar Research Institute in the project, values ***"these results very positively, due to their impact on improving the quality of life of people with Down syndrome and in general, with intellectual and developmental disabilities"***.

A new therapeutic approach

The molecule developed by Aelis Farma is based on the fact that people with Down syndrome have **hyperactivity of the cannabinoid receptor CB1**, according to several studies, which indicate that its modulation with specific inhibitors drastically improves cognitive performance in animal models. Treatment with AEF0217, the first drug in a new pharmacological class, CB1 Receptor Signaling Specific Inhibitors (CB1-SSi), mimics a natural defense mechanism of the brain to counteract the hyperactivity of this receptor. At the same time, the function of the APOE4 genotype, linked to endocannabinoid physiology and which is the main genetic risk factor for Alzheimer's, is also studied.

The team of Dr. Diego Real de Asua from the Hospital de la Princesa in Madrid has also participated in the work. To carry it out, the collaboration of the participants, their families and family associations has been necessary, who have supported the project and have helped to complete this pioneering clinical trial.

Next stage: international study

The results obtained reinforce the continuity of the project and allow us to move towards the next stage: an **international multicenter phase 2 study** that will begin in the middle of next year. This trial will focus on determining the appropriate dosage of the treatment to maximize its benefits. The objective will be to deepen the improvement of both functionality in daily life and specific cognitive functions in people with Down syndrome.

More information

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