

Press release
November 29, 2023

DREAMS : **a new Horizon Europe project brings hope to rare disease patients**

The objective of this 5-year project is to discover treatments for 5 rare neuromuscular disorders through a groundbreaking methodology combining Artificial intelligence (AI), stem cells and pharmacological screening.

In a groundbreaking endeavour set to span five years, the DREAMS consortium is paving the way for a novel approach to treating neuromuscular diseases. This transformative project merges the power of artificial intelligence, pluripotent stem cells, and cutting-edge pharmacological screening techniques to seek out therapeutic solutions for five debilitating conditions affecting more than 400 000 people worldwide.

The challenge of rare disease drug discovery

Rare disease drug discovery is notoriously hard. The high costs, limited understanding of the diseases and lack of investments, are key challenges for drug hunters and patients. 95% of rare diseases still lack a treatment.

To address these challenges, the European Commission has invested nearly €8 million into the DREAMS consortium—Drug REpurposing and Artificial intelligence for Muscular disorders. With this generous backing, the consortium is driven to pioneer an innovative method that combines artificial intelligence, pluripotent stem cells, and pharmacological screening. The objective is to craft treatments for five rare neuromuscular diseases: Duchenne muscular dystrophy, centronuclear myopathy, Emery-Dreifuss muscular dystrophy, Pompe disease, and Danon disease.

A Dual-Pronged Approach

The researchers behind DREAMS have devised a two-fold strategy. Firstly, they will identify promising therapies for the five genetic diseases through AI and advanced pharmacological screening. Secondly, their cutting-edge artificial intelligence capabilities will be harnessed to identify new diseases that could benefit from these treatments.

Dr. Xavier Nissan, Project Coordinator and Research Director at I-Stem enthuses: "*Artificial intelligence is ushering in an era of unprecedented possibilities. Thanks to Kantify's AI technology, we are not only aiming to predict therapeutic targets and drugs but also to identify novel therapeutic applications for our most promising treatments. This endeavour marks uncharted territory, and I am profoundly excited about the potential impact. If successful, this project will open a new horizon of possibilities for rare disease drug discovery.*"

A complementary consortium gathering patients and drug hunters:

The DREAMS consortium unites a highly skilled and complementary consortium of nine European partners, encompassing clinicians, patients, academic researchers, and private stakeholders. These organizations include:

1. I-Stem (France)
2. Kantify (Belgium)
3. The Institute of Myology (France)
4. Center for Neuroscience and Cell Biology - University of Coimbra (Portugal).
5. The Technion - Israel Institute of Technology (Israel).
6. Samsara Therapeutics (UK)
7. Assistance Publique - Hôpitaux de Paris (France).
8. AFM-Telethon (France).
9. Zabala Innovation (Spain)

One person out of twenty in the world is affected by a rare disease. DREAMS will strive to tackle the core challenges of rare disease drug discovery, opening doors to a brighter future for countless patients worldwide.

Learn more about I-Stem : [I-Stem - Homepage \(istem.eu\)](http://istem.eu)

About I-Stem

Founded in 2005 under the impetus of AFM-Telethon and Inserm, I-Stem is an international reference research and development center dedicated to the development of innovative treatments using pluripotent stem cells (ES and iPS) for rare genetic diseases. Composed of 75 employees, its objective is to use these cells as tools to understand genetic diseases and to develop treatments (cell therapy or high-throughput pharmacological screening).

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