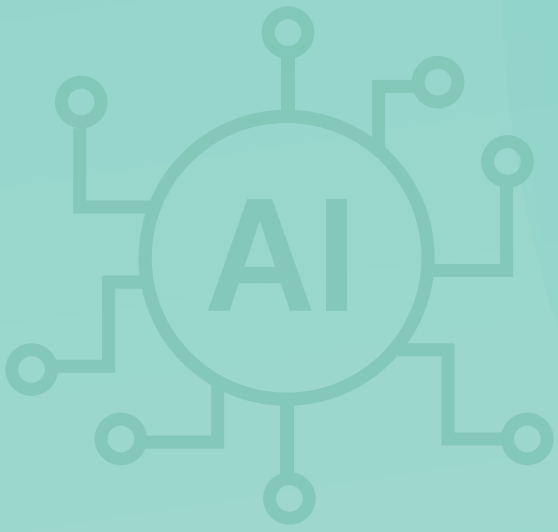
Dreams

# Drug Repurposing with Artificial Intelligence for Muscular disorders



DREAMS aims to discover **therapies for 5 neuromuscular disorders (NMDs)** through a groundbreaking methodology combining **Artificial intelligence** (AI), stem cells and pharmacological screening. As a European Research and Innovation initiative, it will implement novel approaches for target and small molecule discovery to **develop faster and more effective treatments**, improving patients' lives.

The project is driven by an **international consortium** of 9 organizations, featuring renowned researchers and clinicians from across Europe and is led by the CECS/I-Stem Institute, Centre d'Études des Cellules Souches.

## Key pillars

## 01 Cells, Data and Algorithms

Identify common drug targets across multiple rare diseases (RDs) by developing specialized cellular assays. These assays utilize skeletal muscle cells derived from induced **pluripotent stem cells** (iPSCs) to generate relevant data that aids in the understanding of initial disease mechanisms.

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## 02 AI-Powered Drug Discovery

Discover and validate safe and effective drugs for various rare diseases (RDs) by employing advanced Artificial Intelligence (AI) models. This innovative approach facilitates **the discovery of new therapeutic options** and explores extended therapeutic indications, ensuring a broader application of potential drug candidates.

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## 03 Towards Therapy

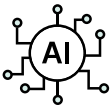
Develop a reusable methodology for conducting clinical trials with small, diverse groups of RD patients. By designing adaptive clinical trial strategies, DREAMS prepares for the **clinical validation of drug candidates** that can effectively target multiple disease indications, thereby enhancing the potential for successful outcomes in rare disease treatment.

## Outcomes

Discover novel **therapies** for various NMDs and related conditions.

Improve insights into the core mechanisms of **rare disease** processes.

Establish new **clinical trial designs** for diverse patient populations in rare diseases.




Develop the first reusable **drug discovery platform** using AI and iPSCs to identify safe and effective treatments for rare diseases.

## Impacts

**Scientific:** Generate new knowledge to the scientific community and provide validated evidence of the combination of the unique forward and reverse approach in specific scenarios of NMDs.

**Societal:** Improve the quality of life of patients suffering from rare NMDs, which encompass hundreds of different diseases that impact patients' movement and locomotion.

**Socio-economic:** Reduce the economic impact of RDs finding cost-effective therapies to treat a subset of orphan NMDs with commonalities.

**Marketability:** Create valuable knowledge and tools to be leveraged for related Initiatives by pre-clinically validating a re-usable approach to cost-effectively find common treatments for multiple RDs.

# Core features of the project

## Selected Rare Diseases

DREAMS focuses on **5 rare neuromuscular disorders** (NMDs) with a known genetic origin, characterized molecular mechanisms, and existing biomarkers. These disorders share common pathophysiological features, including autophagy dysfunction and desmin disorganization.

**CNM:** Dynamin 2 Centronuclearmyopathy
**DMD:** Duchenne muscular dystrophy
**EDMD2:** Emery-Dreifuss muscular dystrophy
**POMPE:** Glycogen-Storage Disease Type II disease
**DANON:** Danon disease

## Advanced Artificial Intelligence Models

The use of a novel AI-powered methodology will enable DREAMS to create a **new paradigm in drug discovery for rare diseases**, where AI will:

- Accelerate the time to target discovery.
- Accelerate the time to molecule discovery.
- Reduce the costs for target and molecule discovery.
- Decrease the risk of failure linked to unwanted off-target effects and toxicity.

DREAMS will establish a unique reusable iPSC/AI powered platform to identify treatments for rare NMDs leveraging the AI/Machine Learning (ML) algorithms of the project partner, Kantify (Sapian plarfom).

## Adaptative Clinical Trials Design

DREAMS wants to design an innovative and adaptive clinical trial protocol with the active participation of both patients and regulatory experts. This initiative will **streamline the process of conducting clinical trials to validated therapies for rare diseases**, which have a limited number of diverse patients, and therefore the classical clinical trial designs are not feasible.

By following this approach, the adaptive model design intends to accelerate the validation of new therapies and treatments, while addressing the distinctive challenges posed by rare diseases.

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## DREAMS IN FIGURES

| 7.8 | 6 | 9 | 5 |
|---|---|---|---|
| Millions of budget | Countries | Partners | Years |

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