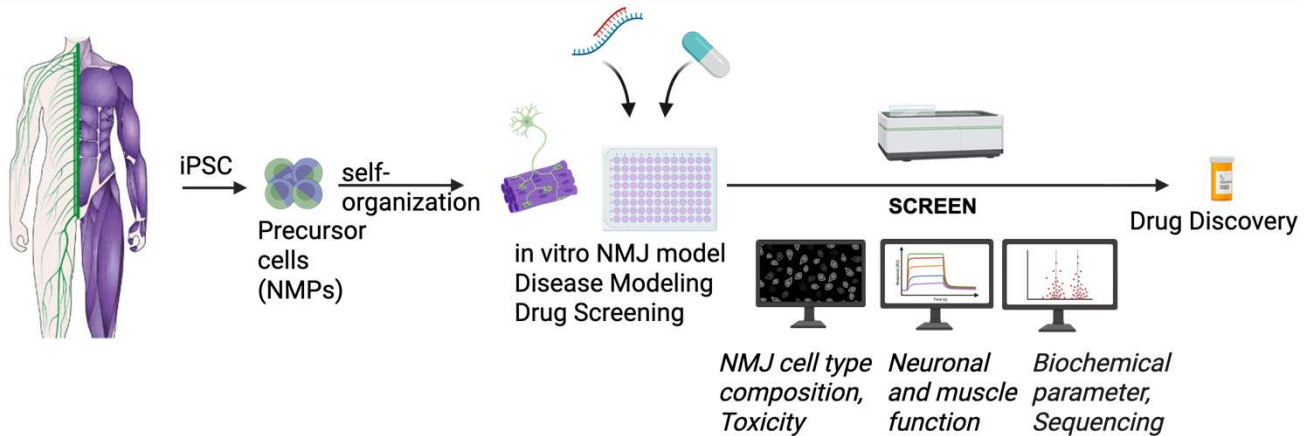


- › Neuromuscular diseases are highly prevalent (~1 in 3,500) and have no cure
- › Current preclinical models fail to capture human specificity and complexity
- › This results in costly false-positive drug candidates, late-stage failures, and high R&D costs

**NMJCare** is a patented 2.5D iPSC-based physiologically relevant human neuromuscular system that combines the scalability of 2D with the physiological relevance of 3D models for high-throughput disease-relevant drug screening

## TECHNOLOGY



**Figure 1: NMJCare Drug Discovery Approach**

### Human neuromuscular high-throughput platform for drug discovery

- › Multicellular neuromuscular junction model (NMJ) generating neurons, muscle, and Schwann cells in one system
- › Functionally active, capturing nerve-induced muscle contraction and disease-specific impairments (e.g. Spinal Muscular Atrophy, Duchenne Muscular Dystrophy, Myasthenia Gravis, Amyotrophic Lateral Sclerosis)
- › Scalable, pharma-compatible 2.5D cultures enabling long-term, functional and morphological readouts

### Development status

- › TRL3-4
- › Faithfully models Spinal Muscular Atrophy (SMA) with clear functional and structural deficits
- › First proof of concept achieved in SMA, demonstrated therapeutic rescue in pilot drug-response screens

### Applications:

- › Preclinical drug discovery and screening
- › Patient-derived personalized screening and disease modeling
- › Mechanism of action, toxicity and efficacy testing

## INTELLECTUAL PROPERTY

Self-organizing neuro-muscular junction cell culture

*Prov. Applications (06/2023): US, SG, CA*

## PARTNER WITH US

We are seeking

- › Co-development opportunities

## RESEARCH EXPERTISE

- › This ERC-supported platform was developed by the group of Prof. Dr. Mina Gouti, a key opinion leader in advanced human neuromuscular systems
- › Her lab specializes in stem cell modeling of neuromuscular development and disease
- › Dr. Ines Lahmann is a technology expert with extensive experience in the muscle system



PROF. MINA GOUTI



DR. INES LAHMANN