Dr. Sakurai's team will create novel therapeutic drugs for intractable muscular diseases such as Miyoshi myopathy and Duchenne muscular dystrophy and investigate muscular disease models. To achieve this goal, they utilize patient-derived iPSCs as a tool for disease modeling and drug screening.

**<Progress>**

**Miyoshi Myopathy**:
- Identified "drug seeds" elevating dysferlin protein levels by high-content and high-throughput drug screening using patient iPSC-derived myotubes.
- Optimization of seed compound is underway to deliver a novel therapeutic drug.

**<Concept>**
- Both iPSCs derived from healthy subjects and patients are differentiated into skeletal muscle cells (myotubes) on 384-well plates.
- A high-throughput drug screening and evaluation system are developed by visualizing pathological changes observed only in patient iPSC-derived myotubes.
- Compounds that improve pathological changes are selected and optimized.

**<Progress>**
- Miyoshi myopathy: Identification of "seed compounds" from Takeda compound library (left panel)