**Exploring the Impact of Gene Therapy on Muscle Diseases Using a 3D Model of Human Organs**

**The Challenge:** For families living with Duchenne muscular dystrophy (DMD) and other neuromuscular conditions, gene therapy offers immense hope. Gene therapy aims to treat disease by delivering a working copy of a gene into the body's cells but predicting how well these therapies work in people remains a significant challenge. Often, a treatment that looks successful in cells or animals doesn't work the same way in people, or it might cause unexpected side effects in the human heart or liver. This makes the path to a cure slower and riskier.

**Our Innovation: "Mini Organs" in a Dish-** To solve this, we have created a human-based testing platform in our lab. We start with cells donated by people living with neuromuscular conditions. These cells are reprogrammed into "iPSCs," which are artificially generated stem cells that have the ability to grow into almost any cell type. From these, we can make muscle cells and build 3D "miniorgans-" behave more like human cells than traditional cell models which grow in a flat layer in a dish. These 3D models are even able to contract.

We have successfully grown:

- Mini Muscles: Connected to human nerves to mimic how a body moves.
- Mini Hearts and Mini Livers: To check if the therapy accidentally affects organs it wasn't supposed to target

**Why it Matters for Patients**

This research has the potential to transform how new treatments for DMD are developed.

1. **Increased Safety:** We can spot potential side effects in human-like tissues *before* the therapy is given to a person.
2. **Greater Certainty:** Because we use cells from people with DMD, they capture the specific biology of the condition. This allows us to test whether a therapy is likely to work in real people with the condition, not just in animals.
3. **Faster Access:** By providing richer and more reliable data to regulators, we can increase confidence in a therapy's safety and effectiveness. This may shorten the time it takes for promising gene therapies to reach clinical trials.