

### **Mutant Human Embryonic Stem Cells (Yissum)**

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# Human embryonic stem cell lines derived from Pre-Implantation Genetic Diagnosis (PGD) embryos

**Categories** 

Mutant Human Embryonic Stem Cells , drug screening, genetic disorders

Stem Cell Research Laboratory, Medical Genetics Institute, Shaare Zedek Medical Center

### **Objective**

• The Stem Cell Research Laboratory is a part of the Medical Genetics Institute in SZMC, which is the largest PGD performing center in the country and one of the leading PGD centers worldwide. The laboratory provides mutant HESC lines. Additionally, the lab investigates basic biological questions related to specific genetic disorders using mutant human embryonic stem cell lines as a model system.

## **Potential Applications**

- Drug screening- HESCs can potentially differentiate into all cell types in large numbers, so that the targeted tissues will be accessible for large-scale screening
- Toxicology assessments of new drug candidates
- Development of new therapeutic protocols, including gene therapy-based treatments
- Improving the currently available treatments by identifying new drug targets
- Invaluable resource to explore unresolved questions related to the biology of particular disorders in human.

#### **Unique capabilities**

- Specialised staff
- Appropriate equipment, for growing human embryos in culture and for the derivation and propagation of human embryonic stem cell lines from 5-7 day old IVF derived human embryos.

#### **Advantages**

- A unique access to early stage genetically diseased human embryos
- A board collection of genetically inherited diseases with a wide range of naturally inherited mutations.
- An unlimited cell source for impaired cells in culture
- Favourable over currently available human-based cellular models: do not require establishing cultures from biopsies of patients, unrestricted life span, can differentiate into all cell types

#### Research Interests

- Understanding the pathology of particularly large trinucleotide repeat expansions, namely fragile X syndrome (FRAXA) and Myotonic Dystrophy type 1 (DM1)
- The mechanisms by which HESCs regulate telomere length



### **Available Resources**

- Potential to derive HESC lines from healthy surplus IVF embryos (IRB 17/11)
- Potential to obtain somatic cells from patients for the establishment of disease specific induced pluripotent stem (iPS) cells (initial stages of approval by SZMC's ethic committee).

## **Laboratory Contact**

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