The Road to the Clinic

Opt!STEM
Optimization of Stem cell Therapy for degenerative Epithelial and Muscle diseases

Basic research

OptiStem is researching the processes that regulate the behaviour of muscle and epithelial (e.g. skin, eye, mouth) stem cells in the healthy body.
We are also examining physiological and immunological issues related to the transplantation of these cells.

1. Where are we headed?

What symptoms and diseases could be addressed, reversed or repaired by stem cell therapy?

2. Planning the route

Which stem cells are right for the disease we want to treat? How much can we find out using laboratory experiments?

Pre-clinical work

OptiStem is analysing what happens to transplanted cells in model systems, studying the immune response and developing microsurgical techniques.

3. Paving the way

What are the best models/ laboratory experiments that can be used to study the disease? Do the cells work in these models? Do we need new models?

4. Checking for road blocks

What does the law say?
Are there any ethical issues to consider? What are the risks we face in the clinic and how do we minimize them? Can we make enough cells of the right type?

Clinical trials

OptiStem will carry out several clinical trials:

Phase I/II trials using mesoangioblasts (a type of blood-vessel-associated stem cell) in different approaches to treatment of Duchenne muscular dystrophy.

A phase I/II trial using epithelial stem cells to reconstitute the oral mucosa of patients with severe injuries.

A clinical study that aims to translate an extensively tested treatment for corneal damage into protocols for widespread use across Europe.

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5. Phase I clinical trials

A first test on a small group of people. Is the procedure safe? Are there any side effects? Can we go on to investigate whether the treatment works?

6. Phase II clinical trials

A trial run with a larger group of patients than at Phase I. Is the treatment effective? Are we still confident the treatment is safe?

7. Phase III clinical trials

Treatment of a large group of patients. Can we confirm effectiveness of the treatment compared to standard treatments?

8. Final approval & widespread distribution

What systems need to be put in place to make the therapy widely available? How will it be funded? Has regulatory approval been given?

Checkpoints

Peer review by experts

Regulatory checks and approvals

Informed consent of patients