

ACTIP monograph on stem cells

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Stem cells and treating diseases. Stem cells and their applications offer an enormous potential for the treatment and even the cure of disease, along with enhancing and extending the quality of life.

In research and development, stem cells help us better understand how the human body works and help uncover the underlying causes of serious diseases. In a laboratory setting, stem cells form the basis for creation of “organoids”, three-dimensional cell formations of specific function with diverse differentiation. Organoids show close proximity to real organs and reflect biology much better than differentiated 2D cell cultures. Today, stem-cell derived differentiated cells and organoids allow researchers to screen for new drugs, validate the function of drug candidates, predict drug metabolism and increase the safety of medicines, thereby reducing or replacing animal experiments.

About stem cells. Stem cells are particular cells that have the unique capacity to renew themselves and to give rise to specialized cell types such as heart cells or blood cells. Stem cells are classified as follows:

- **Adult stem cells** are derived from tissues like bone marrow or cord blood. These cells are already successfully used for the treatment of patients with e.g. leukemia. The treatment of further diseases with adult stem cells is currently under evaluation. However, those cells give rise only to limited number of cell types. Hence the need for concomitant research using pluripotent stem cells for certain applications is still remaining.
- **Embryonic (pluripotent) stem cells** are isolated from embryos. These cells are pluripotent, which means that they can develop into any fetal or adult cell type (blood, heart, brain cells, etc.). Mouse embryonic stem cells can develop into a complete organism.
- **Induced pluripotent stem cells (iPS)** are derived from adult cells, such as skin cells, and are being re-programmed to a pluripotent stage, once re-programmed they exhibit similar properties as embryonic stem cells. iPS cells are currently overcoming the need to derive stem cells from embryos.

Stem cells and technology. The use of stem cells in research and drug development shows a significant increase over the past ten years. But still, there is a high need for innovative technologies to standardize the production of qualitative and quantitative amounts of stem cells and their derivatives for both, the use in drug development and for cell therapy.

Expectations and hopes within this area of research are high: stem cells and their applications may eventually enable researchers to find successful treatments for severe diseases for which we can offer few, if any, effective therapies today. Diseases or indications often mentioned in this context include Alzheimer’s, multiple sclerosis, paraplegia, diabetes, Parkinson’s and heart failure.

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