

Delivering Therapy for Patients Currently Without Effective Treatment Options

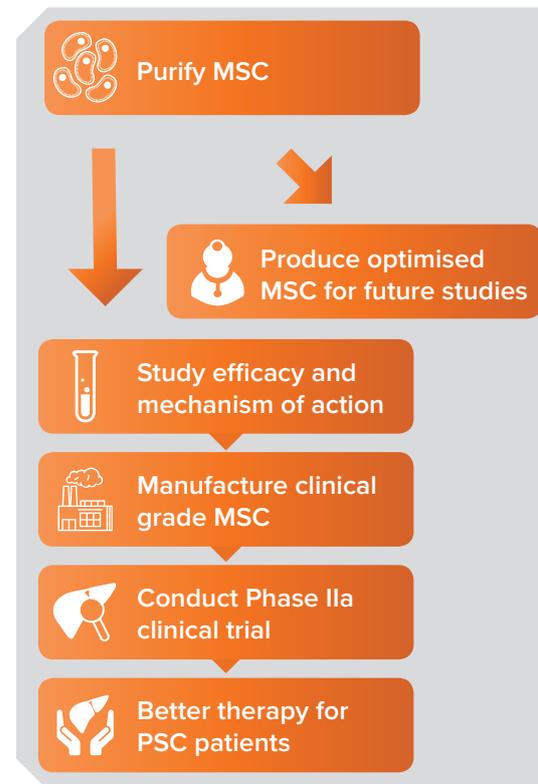
It is estimated that 29 million people in the EU have chronic liver disease, and it is the fifth most common cause of death. Primary sclerosing cholangitis (PSC) is a type of liver disease that is rare and poorly understood. Like most liver diseases, PSC involves inflammation that leads to liver damage. **There is**

currently no curative treatment for PSC, and damage from the disease means that patients often need liver transplants. **MERLIN will look at new ways to treat liver disease with stem cells**, specifically focusing on PSC as a model disease.

How will MERLIN help PSC patients?

MERLIN will start by looking at the effectiveness of mesenchymal stem cells (MSC) against inflammatory liver disease in pre-clinical laboratory models. Based on the outcomes of the pre-clinical studies, a clinical trial will be carried out during the project. The clinical trial will look at the effect of MSC therapy on inflammation in patients with PSC. **This trial will set the stage**

for future work to bring a new, MSC-based therapy for PSC to the clinic. MERLIN will specifically focus on PSC, but will also generate new knowledge that is more widely applicable to stem cell therapy and liver disease in general. In this way, the findings of the trial will inform regenerative medicine strategies for PSC and liver disease, as well as other inflammatory diseases.



How will MERLIN help the European therapeutics industry?

MERLIN will support the development of **high purity stem cell therapies, compliant with emerging regulatory standards.** There are a number of problems with current MSC cell manufacturing approaches that are slowing the approval of stem cell therapies for clinical use in patients. To date, commercially manufactured MSC are low purity and little is known about their distribution in the body (bio-distribution) or how they work once they are administered to patients (mechanism of action). Progress to larger-scale clinical trials and to

mainstream clinical use will require MSC of greater purity than currently available, alongside evidence for the mechanism of action and bio-distribution of stem cells, which is currently lacking.

To overcome these obstacles, **the project will advance European stem cell manufacturing capabilities, develop novel technologies** to better understand the distribution and potency of stem cells once administered and **further our understanding** of how stem cells control inflammatory diseases. These activities will further European

competitiveness in the area of stem cell manufacture and keep European stem cell researchers at the leading edge of regenerative medicine.

MERLIN researchers will tackle these challenges using expertise and proprietary technology including novel cell isolation platforms, GMP-compliant cell production and bio-distribution imaging. This unique combination of expertise positions the research results for **rapid translation to advanced clinical trials.**

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¹ The European Liver Patients Association 2005
² "EU Statistical Year Book" 2007