

RECOMB Gene Therapy Trial: Project Prolonged By 24 Months

[Leiden, 28 June 2023] The Recomb consortium is thrilled to announce the extension of the Recomb RAG-1 SCID gene therapy trial by an additional 24 months, setting a new official end date for December 31, 2024. Thanks to this generous decision by the <u>EU Horizon 2020 programme</u>, this extension allows for continued advancements for this groundbreaking project.

The Recomb project works to develop treatment for one of the more common types of SCID, RAG1-SCID, by performing phase 1 and phase 2 clinical trials using autologous haematopoietic stem cell-based gene therapy.

Originally scheduled to conclude on June 30, 2023, the Recomb project has experienced remarkable progress over the past months, paving the way for enhanced clinical trials and expanded collaboration. Ethical approval was granted in February 2023 to both clinical sites located in Barcelona, Spain, and Wroclaw, Poland, enabling the enrollment of patients into this vital clinical trial. Additionally, the London clinical site in the United Kingdom is currently awaiting imminent ethical approval. The Recomb clinical sites from Germany and Italy both submitted the application at the beginning of 2023 and hope to have ethical approval shortly as well to start recruiting patients. This multicenter and international approach perfectly fits with the aims of European Reference Networks to facilitate access to treatment in rare diseases.

In a dedicated effort to strengthen recruitment for the trial, The Recomb consortium is delighted to announce new collaborations with external clinical sites. The latest partner, the Kayseri Hospital in Turkey, obtained ethical approval in April 2023, further expanding the trial's reach. The consortium is also actively working towards including The Royal Children's Hospital in Melbourne, Australia as an additional clinical site. This aligns perfectly with the goal of improving recruitment efforts and extending the trial's geographical reach.

These recent developments underscore the growing significance of the Recomb project and the potential it holds for transforming the lives of individuals affected by RAG-1 SCID and their families.

"Recomb has used the slogan 'the (stem) cells travel, not the patient', a phrase that now is likely becoming reality given the extension and the approval in Spain, Poland and Turkey to be included as clinical sites, with several other countries to follow soon. Therefore, the fortunate decision of an extension, will give patients from many areas in the world access to this groundbreaking therapy." said Professor Frank Staal, Recomb Coordinator from Leiden University Medical Center.

For more information about the Recomb project and its latest developments, please visit our website or contact us directly.

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