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Plasticell leads gene therapy manufacturing consortium

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UK's innovation agency provides funding to develop advanced technologies for the manufacturing of autologous ex vivo gene therapies.

Plasticell, a developer of stem cell technologies and therapies, has announced it is leading a consortium of pioneering gene therapy groups at UCL's Institute of Child Health (ICH) and Great Ormond Street Hospital (GOSH) to develop advanced technologies for the manufacturing of *ex vivo* gene therapies. The initial focus is on rare inherited disorders of the immune system including Chronic Granulomatous Disease.

"There are a number of highly promising gene therapy targets currently under investigation globally but in order to commercialise these potential cures, the industry urgently needs to find better ways of manufacturing therapeutic products. It is a really exciting project as we test innovative solutions for some of the most advanced medical technologies in collaboration with leading groups in the field" commented Dr. Yen Choo, founder and Executive Chairman of Plasticell.

A number of autologous *ex vivo* gene therapies – in which faulty cells isolated from a patient's peripheral blood are corrected by a virus carrying a functional copy of a therapeutic gene – are now either approved or in the late clinical development stage for a number of rare diseases. However, the high cost of these therapies may prevent delivery of life-saving treatments to patients, the majority of whom are children.

Plasticell will utilise its combinatorial screening technology, CombiCult® to develop novel methods for enhancing lentiviral gene delivery to hematopoietic stem cells, and expanding the numbers of corrected cells *ex vivo*. The collaboration builds on Plasticell's work with Professor Adrian Thrasher from UCL ICH, who has led clinical development of gene therapies for Severe Combined Immunodeficiencies (SCIDs), Wiscott-Aldrich Syndrome (WAS) and Chronic Granulomatous Disease. GMP-compliant manufacturing of the therapeutic products will be overseen by Great Ormond Street Hospital, where these and other gene therapies have been produced and delivered clinically.

The scientific consortium, led by Plasticell, will fund this project partly through a competitive, non-dilutive grant of £740,000, obtained from Innovate UK as part of its ‘Innovation in Health and Life Sciences’ competition.

“We are delighted to extend our collaboration with Plasticell. Gene Therapies are showing real benefit in patients.This is an excellent opportunity to improve the manufacturing technology as we move towards drug licensing”commented Professor Adrien Thrasher, Fellow at the UCL Great Ormond Street Institute of Child Health.

“We have previously used CombiCult® to develop cell culture media that expand hematopoietic stem cells and, separately, methods that enhance lentiviral delivery to target cell types. This new collaboration provides us with the opportunity to perform advanced screens using peripheral blood-derived target stem cells and clinical-grade lentivirus to develop an efficient and cost-effective platform for autologous *ex vivo* gene therapy applications” added Dr Choo.