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CellProthera advances towards its Phase III clinical trials with the development of a new potency assay

- **CellProthera developed the potency assay of ProtheraCytes® that measures the release of paracrine factors which promote the regeneration of tissue after heart attack**
- **This potency assay is a major milestone towards Phase III authorization**

Mulhouse, France, December 13, 2023 – CellProthera, a regenerative cell therapy developer specializing in ischemic diseases, announces the development of a new potency assay to guide clinical development of CD34+ cell-based therapies for use in the pivotal Phase 3 trial for its lead indication, post-acute myocardial infarction. Data from the assay, published in *Scientific Reports*, demonstrates the potency of ProtheraCytes® clinical batches, and may improve development of additional therapies made from CD34+ cells, including CellProthera's new program for ischemic stroke[i].

CD34+ cells are a remarkably versatile cell type, used in a growing number of therapies, including the recently approved gene/gene-edited therapies for sickle cell disease[ii]. CD34+ cells have a therapeutic effect that helps recovery following infarction, and by increasing the number of CD34+ cells administered, CellProthera's therapy can improve the growth of blood vessels following an ischemic event like stroke or heart attack. The development of a potency assay is required by regulatory authorities before commencing the Phase 3 pivotal study.

Through a series of *in vitro* studies, researchers from CellProthera and collaborators at Institut de Recherche en Hématologie et Transplantation determined that all the clinical batches of ProtheraCytes® secrete vascular endothelial growth factor (VEGF), a critical protein involved in the formation of blood vessels. The study found that the concentration of the VEGF, correlated with the number of CD34+ cells expanded in ProtheraCytes® clinical batches, which are made by expanding a population of the patient's own CD34+ cells in an automated GMP manufacturing process. CellProthera will use a potency assay based on this finding in its forthcoming Phase III clinical trials, in patients following myocardial infarction.

"The development of this potency assay signifies a great milestone on our journey to bringing ProtheraCytes® to patients," says Ibon Garitaonandia, Chief Scientific Officer of CellProthera. "Over 15 million people suffer strokes each year, and *a third of a million patients have severely damaged heart tissues following acute myocardial infarction in the US, Europe and Japan alone, but treatments for both are currently limited to alleviating symptoms. A one-time, curative solution would make an incredible difference to these patient groups. We look forward to continuing to move closer to this goal with the announcement of the results of our Phase I/IIb EXCELLENT study next year."*

The paper, 'Development of a potency assay for CD34+ cell-based therapy,' was published in *Scientific Reports*[iii]. It was co-authored by Anne Aries, Céline Zanetti, Rachid Lahlil and Philippe Hénon from the Institute of Research in Hematology and Transplantation and Christine Vignon, Aurélien Goubaud, Arthur Cormier, Anne Diederichs and Ibon Garitaonandia from CellProthera. The findings were also presented at Cell UK 2023 in London.

About CellProthera
CellProthera is a clinical stage regenerative cell therapy developer specializing in ischemic diseases with a leading program in myocardial infarction. CellProthera has developed a unique GMP-compliant cell expansion process as well as a proprietary automation technology for in vitro production of a large quantity of purified, CD34+ stem cells. Its lead therapy, ProtheraCytes, is an autologous cell therapy that has been developed for tissue reperfusion and salvage of damaged cardiac tissue. ProtheraCytes® is registered as an Advanced Therapy Medicinal Product by the European Medicine Agency (EMA). CellProthera's proprietary technology platform comprises an automated expansion device called StemXpand® and its disposable kit StemPack®. CellProthera is headquartered in France.

[i] https://pubmed.ncbi.nlm.nih.gov/38016184/
[ii] https://investors.vrtx.com/news-releases/news-release-details/vertex-and-crispr-therapeutics-announce-us-fda-approval
[iii] https://www.nature.com/articles/s41598-023-47079-8