First pediatric patients treated with ProTrans in type-1 diabetes phase II study

NextCell Pharma AB ("NextCell") supports the pediatric diabetes clinical trial conducted at Uppsala University Hospital by Principal Investigator Professor Per-Ola Carlsson with the stromal cell treatment, ProTrans. At the end of October 2022, the Data Safety and Monitoring Board recommended that phase II part of this pivotal trial should be initiated. Now the first two adolescents in the older age cohort (12-21 years) have received treatment.

Recruitment of children and adolescents newly diagnosed with type-1 diabetes is underway. Patients who can participate in the study must be between 12 and 21 years of age and should have received their diagnosis in the last 6 months. Two out of a total of 30 patients have now received treatment with either ProTrans or placebo.

"The study is progressing according to plan; I have a fantastic study team. The children, and not least their parents, are knowledgeable and very interested, which is inspiring," says Professor Per-Ola Carlsson.

The first part of ProTrans-Young is a safety study (phase I) where all patients have been treated at Uppsala University Hospital. Three patients aged 12-18 years first received treatment, followed by 3 patients aged 7-11 years. Safety data was reviewed by the Data Safety and Monitoring Board after all 6 patients were evaluated at the 3-month follow-up visit. The Board recommended that the study should continue with the second part, which is a randomized and placebo-controlled (phase II) study including a total of 60 children and adolescents with type-1 diabetes, 30 of whom will be treated with ProTrans and 30 with placebo. Initially, 30 patients aged 12-21 will be treated. We can now report that the first two patients have now received treatment. After treatment and 6-month follow-up of the entire age group, the Data Safety and Monitoring Board will again review the safety data before continuing to treat the remaining 30 patients aged 7-11.

The Principal Investigator is Professor Per-Ola Carlsson, Uppsala University and Uppsala University Hospital with co-investigators Professor Helena Elding Larsson, Skåne University Hospital and Professor Johnny Ludvigsson, Linköping University Hospital. The Data Safety and Monitoring Board consists of chairman Professor Ulf Smith, Sahlgrenska, Professor Mikael Rydén, Karolinska Institutet and Professor Anders Fasth, Queen Silvia's Children's Hospital.

Uppsala University Hospital is the sponsor of the study, which is funded by third-party research grants. NextCell contributes ProTrans and placebo to the study. In addition, the company will support the trial with logistics, documentation and expert competence. No additional monetary compensation will be made.

The full title of the study is: "A Double-blinded, Randomized, Parallel, Placebo-controlled trial of Wharton's Jelly-derived Allogeneic Mesenchymal Stromal Cells to treat Type I Diabetes in Children and Adolescents" (EudraCT 2020-004520-42).

About ProTrans:

ProTrans is NextCell's first drug candidate based on their patent pending selection algorithm. It is an allogeneic cell therapy which means that donated cells, not the patient's own, are used. The cells are extracted from donated umbilical cord tissue and the cells are then expanded to generate large, therapeutic doses of drug product.

ProTrans is defined by the selection algorithm, a method for selecting cells with suitable immunomodulatory effect that is assessed in a panel of different analyzes. The algorithm makes an overall assessment of several functional analyzes to identify optimal donors and cells for the manufacture of cell therapies. NextCell's advanced selection method has a scalable ability and guarantees high reproducibility and efficacy compared to other applications in cell therapy. The careful selection method results in cells of consistently high quality and a strong safety profile with few side effects.

For more information about NextCell Pharma AB, please contact:

Mathias Svahn, CEO
Patrik Fagerholm, CFO
Tel: +46 8-735 5595
E-mail: info@nextcellpharma.com
Hemsida: www.nextcellpharma.com

LinkedIn: https://www.linkedin.com/company/15255207/
Twitter: https://twitter.com/NextCellPharma

Certified Adviser

FNCA Sweden AB is assigned as Certified Adviser, 08-528 00 399, info@fnca.se.

About NextCell Pharma AB

NextCell is a cell therapy company in clinical phase II. The company has developed a proprietary and patented platform technology to produce mesenchymal stromal cells adapted for allogeneic treatment of various autoimmune and immunological diseases. The drug candidate ProTrans is now being tested for the treatment of type-1 diabetes as well as respiratory complications caused by Sars-CoV-2 infection. The focus is to take ProTrans to market approval for type-1 diabetes via a phase III study. ProTrans is evaluated in two clinical COVID-19 studies, in Sweden and Canada. NextCell is working on completing its own GMP facility for the manufacture of ProTrans. The GMP facility is expected to be ready for manufacturing smaller quantities of ProTrans in 2023. NextCell also owns 8.5% of FamicordTX, a start-up company in CAR-T and oncology, and 100% of Cellaviva, Scandinavia's largest stem cell bank for family savings of stem cells from umbilical cord blood and umbilical cord tissue with permission from the Swedish Health and Social Care Inspectorate (IVO).