Study design and plan for the recently approved COVID-19 study with ProTrans

NextCell Pharma AB ("NextCell" or "the Company") announced last week that a clinical trial with the stem cell therapy product ProTrans for the treatment of patients with COVID-19 has been approved. A total of nine patients will be treated within this phase 1b study, called "ProTrans 19+". The company provides an update prior to the initiation of the study.

- **ProTrans 19+ will treat COVID-19 patients with NextCell's lead candidate ProTrans. The study will be performed at the University Hospital in Örebro in collaboration with the Department of Clinical Trials and Karolinska Trial Alliance.**
- **The study is a phase 1b study with the primary endpoint to assess safety. Other primary goals are to ensure the correct dosage and efficacy for acute treatment of ARDS and pneumonia in COVID-19.**
- **Mesenchymal stem cells (MSCs) have a documented effect in pneumonia and early study data show that COVID symptoms have decreased after MSC treatment.**
- **Given a satisfactory study outcome, NextCell's hope is to start a phase II study on the basis of the data, with the aim of confirming any effect in a broader study.**

COVID-19, an acute disease

The number of patients affected by COVID-19 has increased sharply during the autumn as well as the number of patients in need of intensive care. The virus infection can lead to serious respiratory complications, which in some cases develop into acute respiratory distress syndrome (ARDS) which requires respiratory treatment or, in very severe cases, oxygenation outside the body with a method called ECMO. Clinically, COVID-19 can be divided into three phases: 1) early, mild symptoms of infection; 2) effect on the lungs with (2a) or without (2b) reduced oxygen in the blood; and 3) general hyperinflammation.

Mesenchymal stem cells have a documented effect in pneumonia

MSCs are multipotent progenitor cells with immunomodulatory, pro-angiogenic and anti-fibrotic properties. MSC has a strong safety profile and has shown potential efficacy in patients with acute ARDS and viral pneumonia. For COVID-19, current data are limited to a case report of 3 doses of MSC from umbilical cord tissue, and a placebo-controlled pilot study of a single IV infusion of 1 x 106 MSC / kg to 7 COVID-19 patients, both from China. No adverse events were documented and within 2-4 days after MSC administration, COVID symptoms subsided.

ProTrans as a treatment for COVID-19

ProTrans consists of MSCs from the umbilical cord, selected according to a patent-pending selection algorithm based on the previously mentioned immunomodulatory and regenerative properties. For example, MSC:s can reduce the risk of scar tissue and increase the formation of new blood vessels. The treatment is given as an infusion into the armpit. The stem cells pass and then, a large part of the stem cells, linger in the small blood vessels in the lungs, which is also an organ that is severely affected by the COVID-19 virus For this reason, lung diseases may be the most optimal disease group for treatment with ProTrans. In NextCell's clinical trials, ProTrans has shown no serious side effects and the therapy can be considered safe. This applies to all doses evaluated in the type-1 diabetes trials, ProTrans-1 and ProTrans-2. As previously presented, ProTrans treatment showed statistically significant positive data in both medium- and high-dose.

NextCells COVID-19 study, ProTrans 19+

The current study, ProTrans-19+ is a phase 1b study, following the same study design as ProTrans-1, with safety as a primary endpoint. In addition, the goal of this dose escalation study is to evaluate the appropriate dose and effect. Initially, three patients are treated with low dose ProTrans, then three patients with medium dose and finally three patients with high dose. ProTrans is a potential treatment for autoimmune diseases and inflammatory conditions. ProTrans 19 'differs from ProTrans-1 and ProTrans-2 in that the treated patients are hospitalized and have been severely affected by the viral infection, with low oxygenation despite oxygen treatment. At this stage, ProTrans will be deployed to counteract general hyperinflammation through overactive immune system.

Motive

Based on the ProTrans-19+ study results, NextCell is expected to be able to confirm the safety of MSC as a treatment for patients who are seriously ill and find a efficacious dose before a phase II study. There is currently no other treatment that has shown efficacy for this patient group that has the highest mortality rate associated with COVID-19.

Other Information about the study

Principle Investigator for the trial is Professor Josefin Sundh, University Hospital Örebro. The clinical trial protocol is written by Professor Dominique Farge, St. Louis Hospital in France, and Doctor Lindsay Davies, CSO NextCell Pharma. The steering committee is Professor Farge (chair), Professor Edvard Smith (NextCell Pharma) and Doctor Tomasz Oldak (PBKM). In this open phase 1 study, three different The study title is: “Treatment of Respiratory Complications Associated with COVID-19 Infection Using Wharton’s Jelly - Umbilical Cord Mesenchymal Stromal Cells (ProTrans®): Open Phase IB Clinical Trial”. At first, the safety of the treatment will be evaluated. In addition, the effect of ProTrans will be evaluated by tracking the patient's clinical status on days 7, 15 and 30, assessed with a "7-point ordinal scale" (1. Not hospitalized, no restrictions on activities. 2. Not in hospital, limitation of activities 3. Hospitalized, not in need of extra oxygen 4. Hospitalized, in need of extra oxygen 5. Hospital, in non-invasive ventilation or high flow oxygen system 6. Hospital, with invasive mechanical ventilation or ECMO 7. Deceased).

For more information about NextCell Pharma AB, please contact:

Mathias Svahn, CEO
Sofia Fredrikson, CFO
Tel: 08-735 5595
E-mail: info@nextcellpharma.com
Hemsida: www.nextcellpharma.com

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

About NextCell Pharma AB

NextCell is a Phase II cell therapy company with the lead candidate ProTrans™, for the treatment of type-1 diabetes. Focus is to take ProTrans™ to market approval via a phase III study. Furthermore, NextCell operates Cellaviva, Scandinavia's largest stem cell bank for family-saving of stem cells from umbilical cord blood and umbilical cord tissue with permission from IVO. FNCA Sweden AB is appointed Certified Adviser, +46(0)8-528 00 399, info@fnca.se.