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A new era of cell therapies for intractable diseases

Biotech company Cellatoz Therapeutics is developing innovative cell therapies by applying its proprietary cells, known as A-to-Z cells to multiple therapeutic areas. The company is now looking for partners to develop the therapies further.

Cellatoz Therapeutics is leveraging lessons learnt from the first wave of cell therapies to overcome barriers to the treatment of intractable diseases. Equipped with proprietary cells, Cellatoz is ushering in a new era for cell therapies defined by cell-specific markers and the regeneration of damaged cells or tissues. Now, having generated evidence that its approach has potential, Cellatoz is seeking a partner and funding to bring its cell therapies to patients.

Early attempts to use stem cells to treat disease failed to live up to expectations, with the harvesting and activation of primary cells and mesenchymal stem cells (MSCs) yielding therapies with marginal efficacy. The setbacks pointed to a new way forward for the field, leading Cellatoz to set out in 2017 to create reliable, novel, stem cell therapies.

Cellatoz's approach is based on proprietary cells, known as A-to-Z cells, with applications in multiple therapeutic areas. Rather than simply harvesting and activating primary cells and MSCs, Cellatoz is working with different starting materials and differentiating them to create therapies capable of treating intractable diseases.

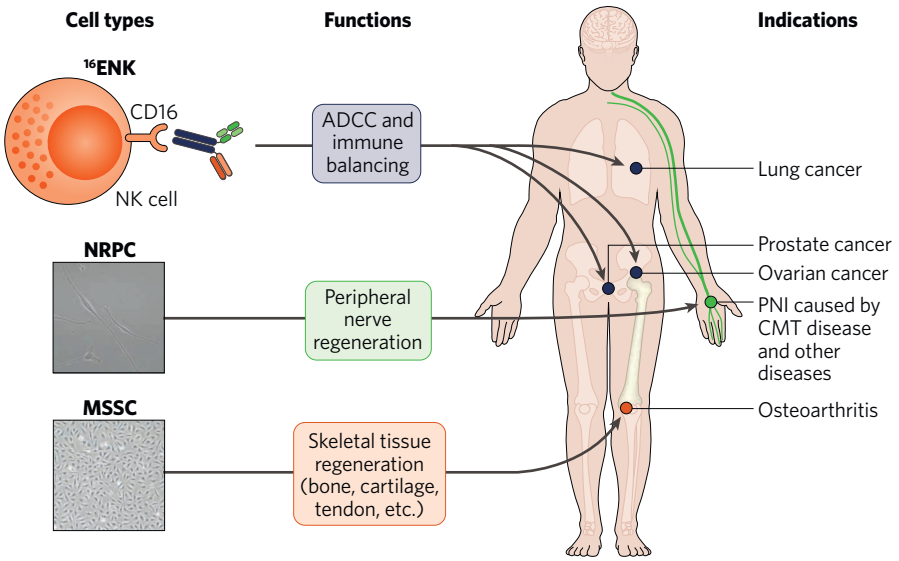

Fig. 1 | Applications of the proprietary A-to-Z cells at Cellatoz. The illustration shows the individual functions of each of the cell types and the diseases that they directly target. ^{16}ENK, CD16-highly expressing NK cell; ADCC, antibody-dependent cellular cytotoxicity; CMT, Charcot–Marie–Tooth; MSSC, musculoskeletal stem cell; NRPC, neuronal regeneration-promoting cell; PNI, peripheral nerve injury.

How A-to-Z cells treat disease

Cellatoz has discussed three applications of its A-to-Z cells to date (Fig. 1). In one program, Cellatoz is using pluripotent stem cells to create musculoskeletal stem cells (MSSCs) capable of differentiating into bone, tendon, muscle and cartilage. As the cells themselves, as well as the methodology and media used to make them, are proprietary, Cellatoz is in the process of establishing a thicket of patents. The protection is more comprehensive than is usual as ordinarily companies use non-proprietary cells.

There is evidence that Cellatoz's proprietary approach to cell therapy could translate into better outcomes. Nonclinical studies showed that a MSSC therapy, CLZ-1001, proliferates and differentiates at the injection site to regenerate bone tissue, thereby enabling recovery from severe injuries. Buoyed by the data, Cellatoz plans to develop CLZ-1001 as a treatment for osteoarthritis of the knee, either as a new drug or in combination with a medical device.

Cellatoz is advancing MSSCs in parallel to work on allogeneic neuronal regeneration-promoting cells (NRPCs). These Schwann-like cells are differentiated from tonsil-derived MSCs. By differentiating the cells, Cellatoz has improved on the efficacy of MSCs that are merely harvested and isolated. NRPCs secrete neurotropic factors to induce axon sprouting and remyelination of damaged nerves.

In light of those characteristics, Cellatoz is applying NRPCs, in the form of CLZ-2002, to the treatment of Charcot–Marie–Tooth (CMT) type 1A disease and other health conditions caused by damage to the peripheral nervous system. CLZ-2002 remyelinated sciatic nerves in an animal model of CMT, driving Cellatoz to start testing the cell therapy in another animal model[1].

Cellatoz is also developing autologous CD16-highly expressing natural killer cells (^{16}ENKs). Using a proprietary high-yield method, Cellatoz manufactures homogenized NK cells that express CD16 on their surface. Cellatoz thinks the presence of CD16 will lead to antibody-dependent cellular cytotoxicity, suggesting that ^{16}ENKs will work synergistically with immuno-oncology drugs.

Work is underway to validate that hypothesis by testing Cellatoz's lead ^{16}ENK, CLZ-3001, in ovarian cancer and other tumor types. As an autologous cell therapy, CLZ-3001 is suitable for repeat dosing, enabling Cellatoz the potential to treat cancer by rebalancing the immune system, rather than by just activating certain cells.

Taking the pipeline forward

Having raised a $10 million series A financing round in 2019, Cellatoz has advanced its lead programs into nonclinical studies with a view to filing investigational new drugs (INDs) in the first half of 2021. The work is taking place at a state-of-the-art research laboratory and good manufacturing practice (GMP) production plant that Cellatoz constructed to house its 35-person team.

The progress of the programs has led Cellatoz to seek support for the next steps. With the MSSC CLZ-1001 targeting osteoarthritis, a major indication, Cellatoz is seeking a partner to support clinical development of that drug candidate.

Cellatoz is taking a different approach to CLZ-2002 and CLZ-3001. As CLZ-2002 targets a rare disease, CMT, Cellatoz is talking to patient advocacy groups and plans to take that cell therapy forward itself using the proceeds of a series B round that it is in the process of raising. Cellatoz also plans to test CLZ-3001 in patients in Korea, its home market, and Japan itself before expanding globally.

Through the clinical trials, Cellatoz stands to validate the hypothesis that its A-to-Z cells could perform better than the first generation of cell therapies based on primary cells and undifferentiated MSCs. In doing so, Cellatoz will lead to the era of cell therapy 2.0, unlocking the therapeutic potential of human cells to tackle major unmet medical needs.

1. Park, S. et al. *Int. J. Mol. Sci.* **19**, E2393 (2018).

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