Overview of Cellatoz Therapeutics, Inc.

Cellatoz Therapeutics is a biotech company with differentiated expertise in translational research and process development. Equipped with its proprietary cells and commercialization-ready cGMP grade facility in Korea, Cellatoz specializes in developing advanced innovative cell therapies in regenerative medicine and immuno-oncology fields . The company's IP-protected Musculo-Skeletal Stem Cells (MSSCs), which can differentiate into various components of Muscular skeletal system, target regenerating cartilages and treating complex fractures. The company is also developing treatments for peripheral neuropathies, including Charco-Marie-Tooth disease utilizing Neuronal Regeneration Promoting Cells (NRPCs), which are Schwann cell-like cells differentiated from tonsillar mesenchymal stem cells. Moreover, the company focuses on changing the immune profile of a patient to treat solid tumors such as glioblastoma and recurrent ovarian cancer using immune cells incorporating gene modification and combination therapy with existing drugs. The company collaborates with the major medical schools and hospitals in Korea, including *Seoul National Univ. Hospital*, *Samsung Medical Center, and Ewha Medical Center,* and is building partnerships in North America, Europe and Japan.

Cellatoz's Proprietary Cells

◆ **Musculo-Skeletal Stem Cells (MSSCs)**

MSSCs, created from pluripotent stem cells such as embryonic stem cells (ESCs) and iPSCs, are capable of differentiating into diverse types of cells of muscular skeletal system such as bone, tendon, muscle and cartilage by manipulating the microenvironment around the MSSCs. Not only MSSCs themselves, but also the media and methodology used to make them are proprietary. Despite the functional similarities with *human* skeletal stem cells (*h*SSCs) recently identified in the bone marrow by Dr. Longaker's group at Stanford Univ., MSSCs have a different expression pattern of cell surface antigens than that of *h*SSCs. The company intends to collaborate with various partners to realize the full potential of the MSSCs for the benefit of patients.

◆ **Neuronal Regeneration Promoting Cells (NRPCs)**

Differentiated from tonsillar mesenchymal stem cells (t-MSCs), NRPCs are Schwann cell-like cells. By differentiating the t-MSCs, the company has created a completely different type of cells which secrete a variety of neurotrophic factors to induce axon sprouting and remyelination of damaged nerves and possibly becoming parts of the restored myelin sheaths. Such characteristics of NRPCs make them a good candidate for treating health conditions caused by damage to the peripheral nervous system, including Charcot-Marie-Tooth (CMT) type 1A and peripheral nerve injuries (PNI).

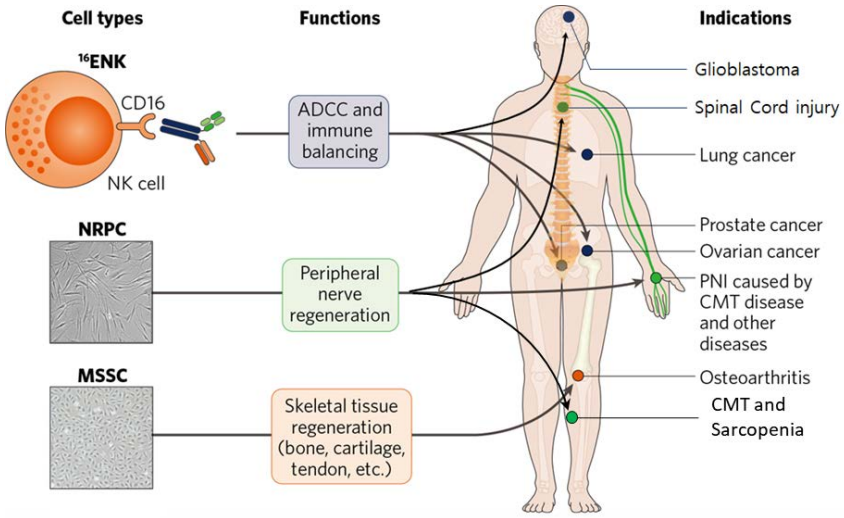

Fig.1. Applications of the proprietary cells at Cellatoz.

◆ **CD16-highly expressing Natural Killer Cells (^{16}ENKs)**

NK cells are known for surveilling and eliminating abnormal cells either with expressing NK activating receptors or with diminished expression of MHC Class I molecules in order to balance the immune system. ^{16}ENKs are being developed as an autologous frozen formulation suitable for repeat dosing, enabling the potential to treat cancer by re-balancing the immune system rather than by just activating specific cells. Using a proprietary high-yield method, homogenized NK cells that express CD16 on their surface are manufactured. The presence of CD16 on its surface which interacts with the Fc region of IgG_1 will lead to antibody-dependent cellular cytotoxicity (ADCC), suggesting that ^{16}ENKs will work synergistically with immuno-oncology drugs.

Last updated on October 1, 2021

Portfolio of Lead Programs

- **CLZ-1001** is a preclinical stage program employing MSSCs derived from embryonic stem cells for the treatment of osteoarthritis of knee. Currently, GLP-toxicity studies including tumorigenicity test are being conducted. An IND filing is expected at the end of 2Q 2022.
- **CLZ-2002** is a preclinical stage program using t-MSC-derived NRPCs for the treatment of Charcot-Marie-Tooth type 1A disease. INDs for Phase 1/2a clinical trial will be filed in the 4th quarter of 2021 in both US and Korea.
- **CLZ-3001** is a preclinical program employing autologous ^{16}ENKs differentiated from human PBMCs for the treatment of glioblastoma. The preclinical study is expected to finish by the 2nd quarter of 2022, and an IND will be filed subsequently.

Tabe.1. Summary of Lead Programs.

Platform Cells	Program Name	Type	Target Indication	2020	2021	2022	2023
NRPC	*CLZ-2002*	Allogeneic[2]	Charcot-Marie-Tooth Type 1A		Preclinical → IND	Phase 1/2a	
MSSC[1]	*CLZ-1001*	Allogeneic[2]	Osteoarthritis of Knee		Preclinical Production / Preclinical → IND	Phase 1	
^{16}ENK	*CLZ-3001*	Autologous[3]	Glioblastoma		Preclinical Production / Preclinical → IND	Phase 1	

1) ES derived MSSCs
2) frozen formulation
3) live and frozen formulation

Dr. Jaeseung Lim,
Co-founder, CEO & CSO

- Ph.D. at Dept. Biochemistry & Molecular Biology, University of Leeds (UK)
- M.S. & B.S. at Dept. Chemistry, Seoul National University (Korea)

- CSO / Senior VP, CHA Biotech Co. Ltd. (Korea)
- Head of R&D / Executive Director, Innocell (currently, GreenCross Cell)
- Senior Research Scientist, Stem Cell Research, National Blood Service (UK)
- One of the global experts in immune cell therapy and successful commercialization of 'Immuncell-LC inj.' product in Korea
- Co-author of '*Second Generation Cell and Gene-based Therapies*', Academic Press (2020)

2017

Cellatoz Therapeutics, Inc. was established on August 14, 2017 with a vision '*to become a pioneer expediting novel but reliable cell therapies instilling new hope for patients with intractable diseases*'

40

As of September 2021, the company has raised a cumulative total amount of US$40 Million from Korean VCs and other institutional investors to build its research laboratory and cGMP grade facility as well as supporting various preclinical programs.

48

As of September 2021, 48 members including research scientists and staffs are working together. Most senior members have expertise in research and development in cell & gene therapies at major companies in Korean biotech industry including *GC Cell, CHA Biotech, Celltrion, LG Chem, & Pfizer.*

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As of August 2021, Cellatoz Therapeutics has developed 4 unique cells as active components in 4 programs, respectively. Three programs are undergoing preclinical studies and the first IND will be filed in the 4th quarter of 2021.

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