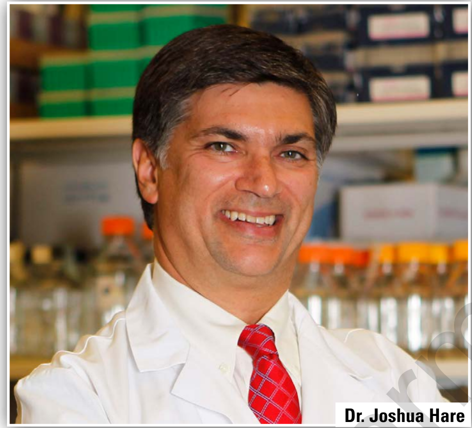
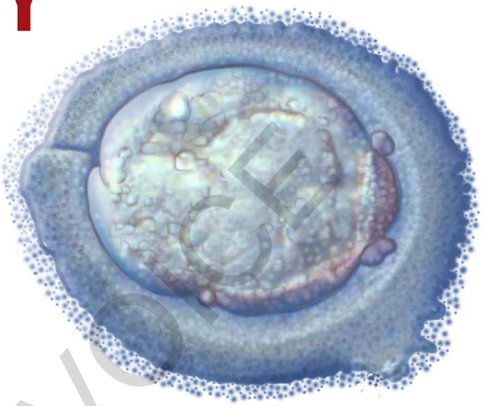
Using Stem Cells TO TREAT FRAILTY

Longeveron's Chief Science Officer and Co-Founder Dr. Joshua Hare talks about the company's cell-based therapies for age-related diseases.



Dr. Joshua Hare



Breakthroughs in stem cell research could potentially lead to new treatment options for chronic diseases and injuries affecting various organ systems. Research is being conducted with stem cells in diabetes, Alzheimer's, heart disease, and stroke, to name just a few areas of therapeutic interest.

One of the more exciting ideas emerging from the field of regenerative medicine is the possibility that stem cells can treat age-related disability and frailty, reduce inflammation, and improve function and quality of life.

Aging frailty is estimated to affect more than 10% of people 65 and older. Aging frailty results in increased fatigue, difficulty going about daily activities, decreased mobility, heightened risk of injury from falling, more frequent visits to the doctor, and a general decrease in health.

An underlying trigger thought to be involved in aging frailty is chronic low-grade inflammation within the body, which can take a severe toll on general health. This includes weakening of the immune system.

Now a company, Longeveron, is researching how stem cells can be used to treat frailty in older people.

"We are pursuing the aging process itself, and very specifically, one aspect of aging that leads to disability and increased vulnerability of patients — frailty," says Joshua Hare, M.D., Longeveron's chief science officer and co-founder. "Aging frailty is a very common condition that affects tens of millions of people and a condition that is most responsible for medical problems in individuals of older age. We recognize that infusions of stem cells might help people with aging frailty, and therefore might make the quality life and functional capacity much better."

Aging frailty is thought to arise because of two things: inflammation and loss of adult stem cells as we age, he says.

"Throughout our lives, our organs replenish themselves," Dr. Hare says. "We're particularly aware that our skin regenerates, our hair grows, but this is also happening with the skeletal muscle system, as well. We're all familiar with the fact that we become weaker as we age, and the reason is that we lose skeletal muscle through a process called sarcopenia. We think sarcopenia arises due to the loss of stem cells. We think we can replenish the stem cells in people with aging frailty and increase muscle and muscle function and reduce inflammation."

The company's research with mesenchymal stem cells shows it may alleviate inflammation and promote endogenous tissue repair. Its Longeveron human Allogeneic Mesenchymal Stem Cells (LMSCs) are produced from stem cells derived from human donor bone marrow and not from a patient's own stem cells.

In September, the company began a Phase IIb clinical trial to evaluate the safety and efficacy of LMSCs in patients with aging frailty syndrome.

In November, the company released results of Phase I and Phase II studies called the CRATUS study. The first phase of the studies, which involved 15 people, showed that stem cell infusions were safe for patients. A second phase, which included 30 people, found that those who received a single stem cell infusion treatment showed improved lung function and an ability to walk farther than the group that received a placebo. Patients also performed better on a series of tests that evaluate, for instance, how fast patients can get up out of a chair and standing coordination. The average age of those participating in the clinical trials was 78, and those who received stem cell infusions intravenously showed physical improvement at three and six months after the therapy.

The studies also showed that Longeveron's stem cells have anti-inflammatory effects, pro-regenerative capabilities, and can decrease scar tissue. The therapy also promotes new vessel growth and improves endothelial function. Allogenic human mesenchymal stem cells have been shown to upregulate anti-inflammatory pathways and restore endothelial function. The company's hypothesis is that by restoring endothelial function and lowering TNF-alpha this could potentially reduce the risk of developing type 2 diabetes mellitus and vascular events.

The company is in the process of applying for the Regenerative Medicine Advanced Therapy Designation from the Food and Drug Administration. This designation was enabled by the 21st Century Cures Act and provides for increased and earlier interactions with the FDA, similar to those available to sponsors of breakthrough-designated therapies. In addition, sponsors with the RMAT designation may be eligible for priority review and accelerated approval. The meetings with sponsors may include discussions of whether accelerated approval would be appropriate based on surrogate or intermediate endpoints.

Longeveron, founded in 2014, is also conducting a Phase I study to test the safety and efficacy of LMSCs for the treatment of subjects with clinically diagnosed Alzheimer's disease. An important component in the progression of Alzheimer's disease is neuroinflammation. Prior studies in mouse models of Alzheimer's disease support this approach.

Longeveron also is conducting research of LMSCs in metabolic syndrome, a group of risk factors that increase a person's chance of developing cardiovascular disease; type 2 diabetes mellitus; serious macrovascular events, including myocardial infarction, stroke, and angina; and death.

The company has raised a total of $28 million through investors as well as grants from the Alzheimer's Association; TEDCO, Maryland's fund that supports stem cell therapy, and the National Institutes of Health. The Maryland Stem Cell Research Fund (MSCRF) was established by the Governor and the Maryland General Assembly through the Maryland Stem Cell Research Act of 2006 to accelerate research using human stem cells and advance medical treatment.

Dr. Hare says in the future, stem cell therapies will be transformative and become the norm.

"Just as antibiotics became a revolutionary, transformative therapy in the 20th century, we're going to see safe and effective cell-based therapies be widely used in the 21st century," he says. "We envision a time when everybody will get cell therapy, in the same way folks take an antibiotic at some point in their lives." PV

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