Research Using Stem Cells

A core element of the Alzheimer's Association's mission is to eliminate Alzheimer's disease through the advancement of research. To this end, the Association is committed to discovering the causes of Alzheimer's, prevention strategies, methods for better and earlier diagnosis, more effective treatments and ultimately, a cure for this fatal disease.

Stem cells are undifferentiated cells that can be coaxed into becoming specific cell types, such as neurons (brain cells). Although there are many ways in which stem cells have the potential to be useful for Alzheimer's research, **currently there is no evidence supporting stem cells as a safe and effective treatment for Alzheimer's disease**.

Stem Cell Clinical Trials and Therapy

The U.S. Food and Drug Administration (FDA) oversees clinical trials and determines whether an experimental treatment is safe for public use. The FDA has approved only one stem cell product, which is for certain disorders affecting the body's blood-forming system. **There is no FDA-approved stem cell therapy for Alzheimer's disease**. And, in fact, there have been no completed stem cell clinical studies to date in Alzheimer's or other dementias to warrant FDA consideration or approval as a treatment.

There are now more than 500 sites in the United States that offer what they are calling stem cell-based therapies, some of which claim to treat Alzheimer's disease and/or dementia. On its website, the FDA states that it "is concerned that the hope that patients have for cures not yet available may leave them vulnerable to unscrupulous providers of stem cell treatments that are illegal and potentially harmful."

The FDA cautions consumers to make sure that any stem cell treatment they are considering has been approved by FDA (none have been approved for Alzheimer's or dementia) or is being studied under a clinical investigation that has been submitted to and allowed to proceed by the FDA. "This also applies if the stem cells are your own," the FDA adds. "Even if the cells are yours, there are safety risks, including risks introduced when the cells are manipulated after removal."

The Alzheimer's Association is providing this unbiased information to facilitate a better understanding of the current status of stem cell research for Alzheimer's and to help keep you and your loved ones safe in this period of rapid scientific advances.

The Potential Roles of Stem Cells in Alzheimer's Research

Alzheimer's disease research is advancing quickly, including research on potential therapies to prevent, slow or stop the disease. The Alzheimer's Association supports and encourages any legitimate scientific avenue that offers the potential to advance

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the goal of eliminating Alzheimer's, including stem cell research. We oppose any restriction or limitation on research, provided that appropriate scientific review and ethical and oversight guidelines and compliance are in place.

As the body develops, most cells become very specialized—or differentiated—and many lose the ability to replenish themselves through cell division. A stem cell, simply put, is a cell that retains the ability to divide and is relatively undifferentiated, meaning that it can still give rise to another type of cell (such as a neuron or a red blood cell).

There are different types of stem cells. Some of the principal types are listed below.

- Embryonic stem cells are derived from embryonic tissue. The majority of human embryonic stem cells are derived from embryos that come from eggs that have been fertilized in vitro (not from embryos fertilized in a woman's body). Embryonic stem cells (either animal or human) can divide many times, giving rise to millions of stem cells from just a single starting cell. In addition, embryonic stem cells can be directed to differentiate into a wide variety of specialized cells. For some medical conditions, these specialized cells may be able to be used to replace cells or cellular factors (such as proteins) that are lost or damaged as a result of disease or that may be therapeutic. In other cases, researchers may be able to use the specialized cells to learn more about the disease process, opening new possibilities for prevention or treatment.

- Adult stem cells are stem cells that live among the differentiated cells of adult tissues or organs. Adult stem cells are usually able to differentiate into one or more of the specialized cells of the tissue or organ in which they reside, but not into other cell types. That is, their capacity for differentiation is limited. Not all tissues and organs have adult stem cells. In addition, numerous specialized cell types are only produced during early development and last a lifetime. Many neurons fall in this category.

- Induced pluripotent stem cells (iPSCs) are adult cells that have been reprogrammed to become more like embryonic stem cells by directing them to express developmental genes. While this causes the adult cells to "de-differentiate" and allows them to give rise to other types of cells, it is not known whether embryonic stem cells and induced pluripotent stem cells differ in clinically significant ways.

The advent of stem cell technology suggests that we may ultimately be able to use stem cells to develop a cure for Alzheimer's disease—and it provides several pathways for this critical research:

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- Therapy development: Scientists envision that treatments that are currently being developed to reduce the brain cell death in Alzheimer's—thus creating a healthier environment within the brain—may be used in conjunction with future stem cell-based therapies to not only stop the disease but also possibly restore once-lost functioning.

- Research into the causes of Alzheimer's: Despite the creation of valuable animal models of Alzheimer's from which we have learned a great deal, it is only humans that fully develop the classic features of Alzheimer's disease. The ability of human embryonic stem cells or iPSCs to form any type of human cell makes them attractive candidates for scientists to use to create alternative and complementary models of the healthy or diseased human brain for testing ideas, theories, therapies, etc.

- It is now possible to create stem cell lines from the skin cells of individual Alzheimer's patients and induce them to become neurons or other brain cells. Through examining these cells we may be able to identify patient-specific processes that cause or contribute to the development of Alzheimer's and identify genes that can delay or prevent the disease. This may also enable us to create entirely new, targeted therapies.

- New drugs can be tested for safety and effectiveness on stem cells or on specialized cells derived from stem cells. As described above, scientists can now use stem cells to create neurons and other brain cells, and these cells are being used to determine whether potential new medications can alter the disease processes that are associated with Alzheimer's. Other cell lines, such as cancer cells, have a long history of being used to screen new medications in this way.

Alzheimer's poses unique challenges because it affects many types of brain cells in multiple brain regions. At this time, it is unclear if stem cells can form all these different cell types. We also don't yet know if the brain cells created from stem cells, or other cellular factors (such as proteins) derived from them, could effectively and safely repair the extensive network of cell-to-cell connections that are damaged when brain cells die in Alzheimer's. The discoveries to date point to the intense need for more and continued Alzheimer's research in every area, driving us closer to the Association's goal of a world without Alzheimer's disease.

Current Status of Stem Cell Research

There is no evidence supporting stem cell therapy for Alzheimer's disease at this time. The Alzheimer's Association will monitor the progress of stem cell research and update this document as needed.

— Alzheimer's Association Medical and Scientific Advisory Council, reviewed April 2024

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