Autoimmune Disorders

Overview

Autoimmune disorders are conditions where the body produces an inappropriate response to healthy tissue and substances normally found in the body, effectively destroying them. The body's immune system is unable to tell the difference between healthy body tissues and antigens such as bacteria, viruses, toxins, cancer cells, blood or tissue from another person. It wrongly identifies the normal tissue as pathogens and attacks its own cells. This leads to the destruction of one or more types of body tissue, abnormal growth of an organ or changes in organ function. These diseases commonly affect the blood vessels, connective tissues, endocrine glands, joints, muscles, red blood cells and skin. Common diseases that are classified into the broad spectrum of autoimmune disorders include multiple sclerosis, type 1 diabetes, lupus, rheumatoid arthritis, uveitis, scleroderma, grave's disease and chronic thyroiditis, among others. There are over 80 types of autoimmune disorders in total.[1]

Around 23.5 million Americans are affected by autoimmune diseases, making it a leading cause of death and disability in the United States. Different diseases are more prevalent in certain races and ethnic groups, and 75 percent of the patients are females.[2,3] What causes the inappropriate immune response in many of these disorders is still unknown, though bacteria and viruses, along with genetic factors, are thought to be involved. These diseases can strike any part of the body with widely varying symptoms, making treatment very difficult. One common form of treatment is immunosuppression, a medication which decreases the immune response in these diseases. However, this treatment also suppresses normal immunity, leaving the body at risk for infection.

Inflammation is associated with autoimmune diseases. While the inflammation can be acute, in many cases, a chronic inflammatory disorder can develop. These disorders result in abnormal inflammation and cause even more destruction of healthy tissue, as well as chronic pain, redness, swelling and immobility.

Autoimmune Disorders and Regenerative Medicine

Developing treatments for autoimmune disorders and inflammation is a significant focus in the regenerative medicine community. The mechanism of action of several of these cell based therapies is still being explored, however, data suggests immunomodulation and suppression can be achieved through cellular interaction between therapeutic cells and the patient's immune system, including anti-inflammatory T-cell responses. Several of these technologies are based on mesenchymal stem and progenitor cell populations derived from a variety of adult

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tissue sources. Several companies are developing regenerative medicine therapies to mitigate the adverse effects of graft-vs-host disease (GvHD), a complication of bone marrow transplantation that kills up to 80% of children affected.

Athersys, Inc. is testing its lead product, MultiStem (allogeneic adult stem cell technology), for prevention or reduction of GvHD in cases of patients undergoing allogeneic hematopoietic stem cell transplants (HSCTs) for the treatment of leukemia and related conditions. Clinical studies yielded positive results in the Phase 1 trial, potentially increasing treatment and prevention options for this autoimmune disease. Additionally, Athersys is conducting a Phase 2 clinical study with partner Pfizer to test the safety and efficacy of MultiStem in individuals suffering from Inflammatory Bowel Disease (IBD).

Autoimmune Disorders: Economic Impact

$100 Billion Annually

NIH estimate for direct healthcare costs to treat autoimmune diseases.[4]

This figure is likely less than the total healthcare costs since isolated inflammatory disorders are not factored into this number.

Celgene is commercializing placenta-derived stem cell therapies for autoimmune and inflammatory diseases. They are currently conducting several clinical studies of their product PDA-001 in autoimmune and inflammatory conditions including Phase 2 studies in both Crohn's disease and rheumatoid arthritis and Phase 1 studies in multiple sclerosis and sarcoidosis.

NeoStem is developing a T-cell therapeutic, Athelos, which works to restore immune balance in GvHD patients by enhancing T-regulatory cell numbers and function. They are currently in a Phase 1 trial.

Osiris's lead product, Prochymal is in Phase 3 studies in the United States for GvHD, and has been approved in Canada. The product demonstrated significant survival benefits in patients with the most severe forms of GvHD. Osiris is also testing Prochymal in Phase 2 studies for Crohn's disease.

Tigenix, a leading European cell therapy company, is making several advancements with their adult stem cell programs targeting autoimmune and inflammatory diseases. Their stem cell platform uses allogeneic adipose tissue derived expanded stem cells (eASCs). Tigenix currently has two products in clinical studies for autoimmune and inflammatory disorders, Cx601 in Phase 3 development for Crohn's disease and Cx611 in Phase 2 studies for rheumatoid arthritis.

[1] MedlinePlus, "Autoimmune Disorders," May 29, 2011, U.S. National Library of Medicine website, www.nlm.nih.gov/medlineplus/ency/article/000816.htm

[2] U.S. Department of Health and Human Services Office on Women's Health, "Autoimmune Diseases Fact Sheet," April 14, 2010, womenshealth.gov website, womenshealth.gov/publications/our-publications/fact-sheet/autoimmune-diseases.cfm

[3] Johns Hopkins Health System, "What is Autoimmunity?: Broad Spectrum of Autoimmune Disease," Johns Hopkins University School of Medicine website, autoimmune.pathology.jhmi.edu/whatis_spectrum.cfm

[4] American Autoimmune Related Diseases Association (AARDA), Inc., "Autoimmune Disease Fact Sheet," AARDA website, www.aarda.org/autoimmune_statistics.php