"Currently, we have 8 medications for multiple sclerosis, but none of them reverse MS. The hope is that mesenchymal stem cells can reverse and repair the damage MS creates in the body. We're taking a careful approach to test for expected and unexpected side effects as we look to see if MSC have the abilities that we think they do."

Jeffrey A. Cohen, MD
Principle Investigator
Cleveland Clinic

**What is MS?** The term "multiple sclerosis" refers to the multiple areas of damage occurring in the body as the result of an abnormal immune response to the central nervous system. The body’s overactive immune cells damage the protective myelin sheath that covers nerve cells, and later stages of MS are marked by damage to and loss of neurons. In healthy individuals, the body has mechanisms to repair this damage; however, in MS, the body cannot keep up with the damage that is continually occurring.

**What are mesenchymal stem cells?** Mesenchymal stem cells (MSCs) are primitive cells in the bone marrow that have a wide range of effects that decrease the activity of immune cells while encouraging tissue repair, both of which may be beneficial in MS. In addition, in numerous laboratory studies, MSCs were able to migrate from the blood into areas of inflammation or injury in the nervous system and reduce damage by developing into cells resembling neurons (nerve cells) and glia (support cells) and, probably more importantly, by creating a tissue environment that encourages intrinsic repair mechanisms.

**Are MSCs safe? What do we know about them?** MSCs have been tested as a treatment for a variety of conditions, including graft-versus-host disease (GvHD), coronary artery disease, non-healing bone fractures, peripheral arterial disease, acute stroke, and spinal cord injury. In previous human studies, MSC transplantation has been well-tolerated and safe, and in some cases, dramatic benefit was reported. However, overall experience with MSC transplantation in humans is limited.

**What is the goal of this Phase 1 study?** The primary objective of this pilot study is to access the feasibility, safety and tolerability of MSC transplantation to repair damage caused by MS. We will be closely monitoring participants to see how their MS responds to see if this approach is well-tolerated, safe and with few side effects.

**How many study participants will be involved in Phase 1?** There will be 24 total participants enrolled for the six-month study, including 12 with relapsing-remitting MS and 12 with secondary progressive/progressive relapsing MS. The study population will include only participants who are either on FDA-approved treatment for their MS, have failed an approved treatment, or have refused treatment with an approved drug.

**If this trial meets its goals, what’s next?** If this trial demonstrates that MSC transplantation is safe in MS patients, further studies will be developed to more definitively assess the efficacy and benefits of this therapy in MS. Future trials may also test other kinds of stem cells. For example, we could explore the effectiveness of cells taken from other, less invasive parts of the body, like fat deposits, as well as donor MSC transplantation (allogeneic). It might also open the door to future studies of MSCs in other neurological conditions.