



CELL THERAPY FOR LUPUS: WHAT YOU SHOULD KNOW

With only a few treatments approved to treat lupus, patients and healthcare providers are excited about the potential of cell therapy and the potentially curative nature of these new treatment approaches. There are various cell therapies approved for the treatment of blood cancers and infections, and there are several approaches being tested for other diseases. While still being studied in people with lupus and other autoimmune diseases, the initial results, while early, are promising, and researchers are further exploring how cell therapy may be beneficial to certain individuals living with lupus.

Understanding cell therapy

Cell therapy uses your own immune cells (autologous cell therapy) or cells from a donor (allogeneic cell therapy) to prevent or treat disease. These cells are engineered (or changed) in a lab and then infused into your body to attack specific immune cells. To treat lupus, cells are altered to target and remove unhealthy B white blood cells (since these cells are believed to play a role in lupus).

There are different types of cell therapies. The treatments use different types of cells to target the immune system and different approaches to produce the adapted cells. The most highly studied in lupus to date is **CAR-T cell therapy** which adds what is called a chimeric antigen receptor (CAR) to a T white blood cell (type of cell that also fights infection and is integral to the immune system), which targets B cells. This is given after receiving medications (chemotherapy) that deplete the immune system (lymphodepletion) and allow the infused modified cells to activate. There may be some benefits observed with cell therapy due to the lymphodepletion too; this is still being studied.

What is the process like to receive cell therapy?

Since these therapies use your own cells or donor cells, the process might feel different than other treatments you have received. Here is an example of what it is like to get autologous (your own cells) CAR-T therapy:

- 1 You may have to stop or decrease your current lupus treatments.
- 2 You may need to stay in a hospital or medical center while the T cells are removed from your blood. This process is called apheresis.
- 3 You may go home for a few days or weeks while the cells are being changed into CAR-T cells.
- 4 Once the CAR-T cells are ready, you may return to the hospital or medical center for a short treatment of chemotherapy or immunosuppressant treatments. This will help make sure that your body is ready to receive the CAR-T therapy.
- 5 After the infusion, you may need to stay at the hospital for a period of time and then close to the medical center for a few weeks. Your healthcare provider will watch you for side effects and signs of infection.

The length of this process may vary depending on the clinical trial you are enrolled in. As cell therapies for lupus are currently in the experimental phase, the only way to receive the treatment is through a clinical trial. Many trials are designed to include individuals who are not responding to other therapies or have more severe disease. Your eligibility will be defined by the inclusion criteria for a particular trial.

While CAR-T therapy represents an intensive treatment, the hope is that it will 'reset' the immune system and diminish lupus so well that the treatment would only be needed once. Trials are ongoing to see how often side effects occur, how many individuals respond and how long the benefits of cell therapy last.



What results have individuals living with lupus experienced with cell therapy this far?

In a recent publication of an ongoing trial, lupus patients improved greatly after cell therapy within a few months.¹ By the later timepoints of assessment in the clinical trial, some participants were symptom-free and treatment-free. The number of individuals that have received cell therapy for autoimmune disease is small, so additional research is needed.



What are some side effects of cell therapy?

Some people with lupus experience side effects after cell therapy. The severity of the majority of side effects has been mild to date. The most notable side effects reported have been:

- **Cytokine release syndrome** - when your immune system becomes highly activated and releases a large amount of biochemicals called cytokines into the bloodstream. Cytokines are normally involved in fighting infections, but when too many are released at once, it can cause the body to react in ways that may make you feel sick.

- **Immune effector cell-associated neurotoxicity syndrome (ICANS)** - when the immune system, after being turned on by the therapy, causes inflammation in the brain and nervous system, leading to confusion, difficulty speaking and in more severe cases, seizures or loss of consciousness.
- **Infections** - If you receive cell therapy, your healthcare team will monitor you closely for any side effects and manage occurrences as needed. It is important to note that cell therapy is early in the evaluation process and researchers/providers are continuously learning from each case, therefore, more information will become available over time. Given that cell therapy is a very complex and involved process, it is essential that you have thorough discussions with your healthcare providers before deciding to participate.



Why are people so excited about cell therapy?

The FDA has approved very few treatments for lupus. For comparison, the FDA has approved 30 treatments for another autoimmune disease, rheumatoid arthritis, but only 3 for lupus. With few treatment options, many people with lupus continue to live with challenging symptoms and damaging flares. The available treatments often have side effects that limit use for some individuals so more treatments options are needed.

New potential treatments, such as cell therapy, offer hope for a future where those living with lupus can thrive without symptoms, ongoing treatments, or the disease itself.

QUESTIONS TO ASK

If joining a clinical trial offering cell therapy is something you want to learn more about, here are some questions to ask your healthcare provider or study team about cell therapy:

- 1 Could participating in a cell therapy trial be a good option for me?
- 2 What side effects may I experience?
- 3 What other medications will I receive as part of the cell therapy treatment?
- 4 How does cell therapy compare to my current treatment?
- 5 If I sign up for a cell therapy clinical trial, will I need to stop my current treatments?
- 6 Will I need to stay in or near a hospital for cell therapy? If so, for how long?
- 7 How much does cell therapy cost? Does the clinical trial cover the cost?
- 8 Are there any activities I cannot do before or after the cell therapy infusion? How much support will I need from a care partner?
- 9 Will cell therapy affect any other diseases that I have?
- 10 Will cell therapy affect my fertility (ability to get pregnant)?

For further information about cell therapy and potential clinical trials, please refer to the Lupus Therapeutics and Lupus Research Alliance websites: lupustherapeutics.org and lupusresearch.org.

