# Regenerative Medicine and Cell Therapies:
## An Overview of Clinical Studies in the United States

Alliance for Cell Therapy Now
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## Regenerative Medicine and Cell Therapies (RMCTs) Show Promise for Patients with Serious and Life-Threatening Conditions

There are nearly 900 active clinical trials in the United States that are exploring the use of regenerative medicine and cell therapies (RMCTs) for patients with serious and life-threatening conditions. Cancer is the most prevalent condition being studied, representing 48 percent of all active clinical studies, followed by musculoskeletal conditions (11 percent), neurological conditions (6 percent), cardiovascular conditions (6 percent), COVID-19 (5 percent), and eye-related conditions (4 percent).

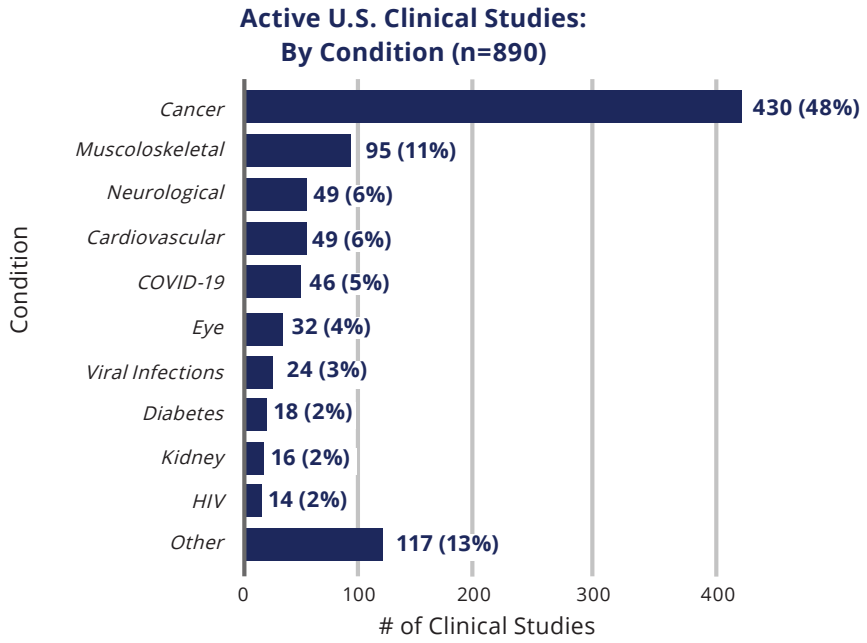
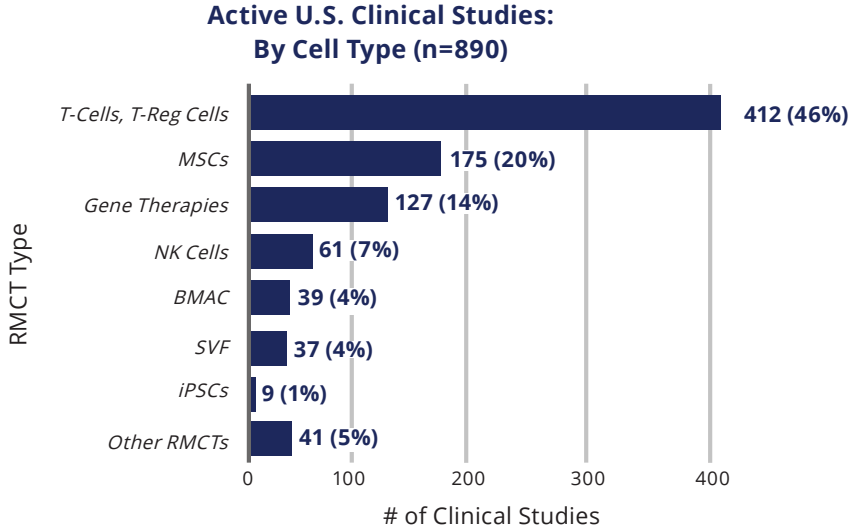



*Figure 1. Number of Active Clinical Studies Exploring Use of RMCTs in U.S., by Condition*

## Clinical Studies are Exploring the Use of Many Types of RMCTs

There are many different types of RMCTs. The majority of studies being conducted today explore the use of T-cells and T-Reg cells (46 percent), primarily for cancer. Many clinical studies are also exploring the use of mesenchymal stromal cells (MSCs) (20 percent), gene therapies (14 percent), and natural killer (NK) cells (7 percent) for various conditions. Studies exploring the use of induced pluripotent stem cells (iPSCs) , bone marrow aspirate concentrate (BMAC), and stromal vascular fraction (SVF) are also being conducted.




*Some clinical studies can explore the use of multiple RMCT types, therefore percentages can add up to more than 100%*

*Figure 2. Number of U.S. Clinical Studies Exploring Use of RMCTs, by Type*

# MSCs and Gene Therapies Show Promise for Patients in Need

While cancer is the focus of nearly all clinical studies involving T-cells, T-Reg cells, and Natural Killer cells, studies exploring the use of MSCs are focused on musculoskeletal conditions (21 percent), COVID-19 (18 percent), neurological conditions (13 percent), and cardiovascular disease (11 percent).

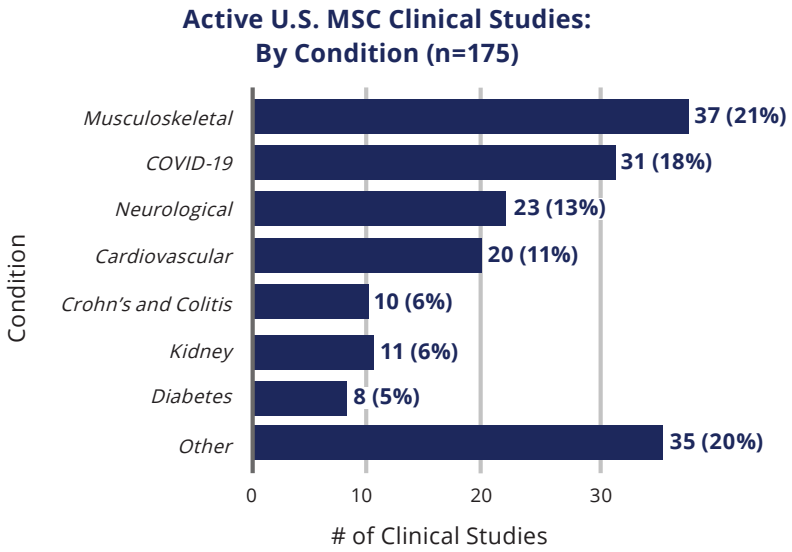



*Figure 3. Number of Active Clinical Studies Exploring Use of MSCs in U.S., by Condition*

Clinical studies exploring the use of gene therapies are focused on cancer (19 percent), eye-related conditions (17 percent), neurological conditions (15 percent), cardiovascular conditions (11 percent), and musculoskeletal conditions (8 percent).

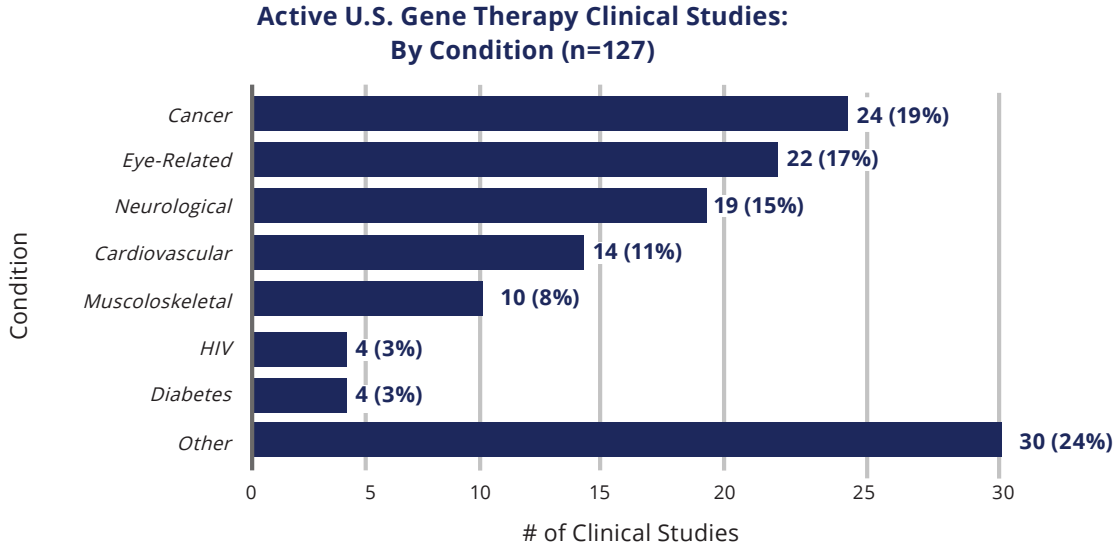



*Figure 4. Number of Active Gene Therapy Clinical Studies in U.S., by Condition*

# Vast Majority of Active U.S. Clinical Trials are Early Phase Trials

Of the 890 active clinical studies exploring the use of RMCTs in the United States, 776 are clinical trials. Ninety-six percent of all active U.S. clinical trials are either Phase 1 or Phase 2 trials. Similarly, 94 percent of the 141 active U.S. clinical trials exploring the use of MSCs are either Phase 1 or Phase 2 trials. While results of early clinical studies are promising, the primary barrier to advancing such therapies is the significant cost of conducting large-scale, randomized clinical trials, which are a precursor to bringing safe and effective therapies to patients.

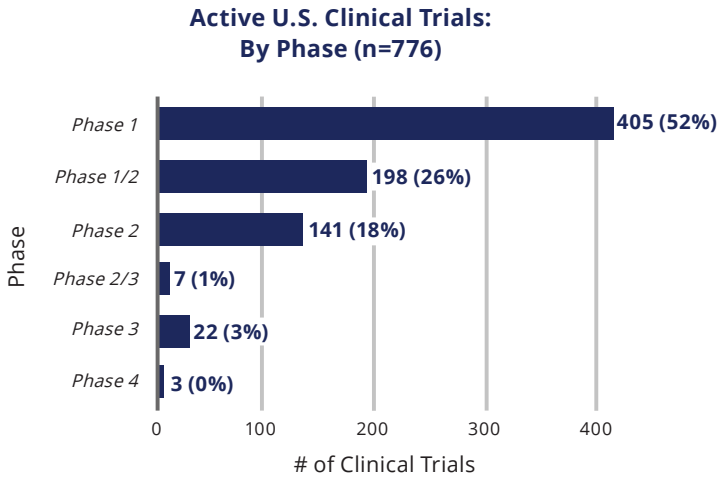



*Figure 5. Number of Active U.S. Clinical Trials, by Phase*

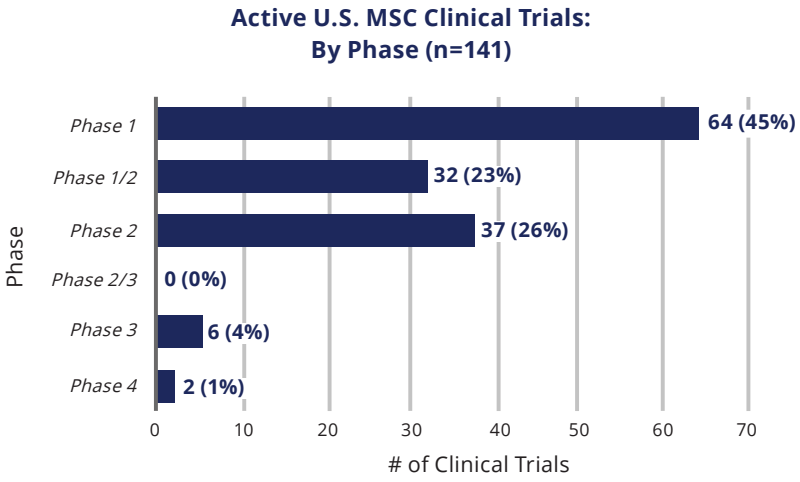



*Figure 6. Number of Active U.S. MSC Clinical Trials, by Phase*

# Federal Government Plays a Limited Funding Role in Clinical Studies for RMCTs

The federal government funds about 18 percent of all active clinical studies exploring the use of regenerative medicine and cell therapies in the United States.

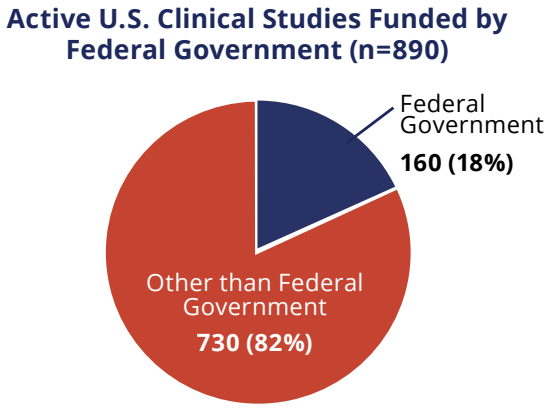



*Figure 7. Federal Funding of Active Clinical Studies in U.S.*

The federal government funds an even smaller percentage—13 percent—of clinical studies that explore the use of MSCs for patients with serious and life-threatening conditions. Without federal support, a majority of the promising results of several studies—particularly those conducted within academic and medical institutions, where most research is performed—will never make their way to patients.

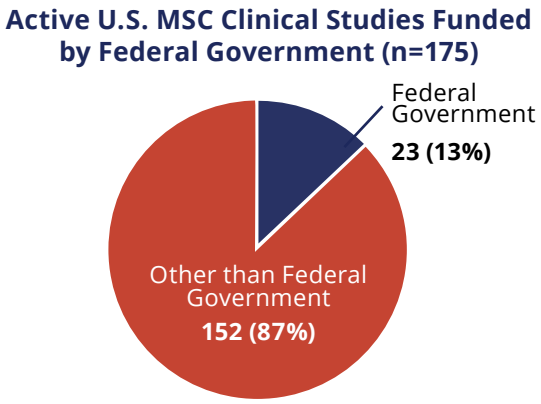
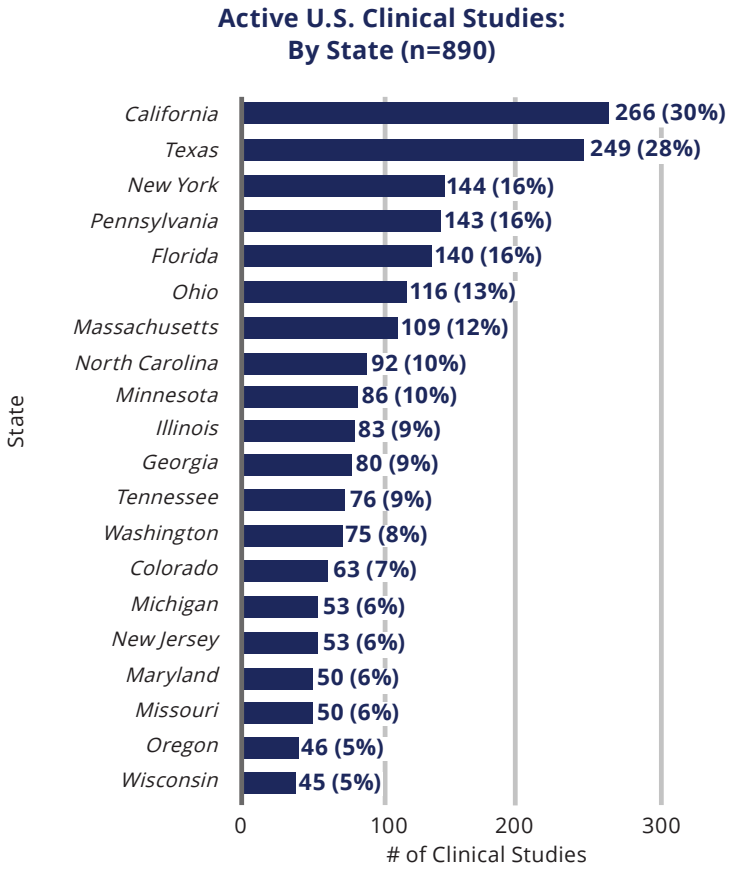



*Figure 8. Federal Funding of Active MSC Clinical Studies in U.S.*

# Most Active Clinical Studies are Conducted in a Small Number of States

Most active clinical studies exploring the use of RMCTs are being conducted in a relatively small number of states, including California (30 percent), Texas (28 percent), New York (16 percent), Pennsylvania (16 percent), Florida (16 percent), Ohio (13 percent), Massachusetts (12 percent), North Carolina (10 percent), and Minnesota (10 percent).

Nine percent of active clinical studies are being conducted in Georgia, Illinois and Tennessee, while 8 percent of active clinical studies are being conducted in the state of Washington.




*Some studies are conducted in multiple locations, therefore percentages add up to more than 100%*

*Figure 9. Number of Active Clinical Studies in U.S., by State*

# Funding is Needed to Advance Promising RMCTs

Hundreds of clinical trials are active in the United States that explore the use of regenerative medicine and cell-based therapies for many conditions, including cancer, neurological conditions, cardiovascular conditions, musculoskeletal conditions, diabetes, and even COVID-19. RMCTs hold great hope for a range of conditions for which—to date—there has been no cure.

Nearly all active clinical trials involving RMCTs in the U.S. are early phase (either Phase 1 or Phase 2) trials. While results of early clinical studies are promising, the primary barrier to advancing such therapies is the significant cost of conducting large-scale, randomized clinical trials, which are a precursor to bringing safe and effective therapies to patients. The financial barriers are particularly problematic for academic and research institutions, as well as small biotechnology companies, who are responsible for the vast majority of clinical trials investigating the use of RMCTs in the United States. Federal funding is urgently needed to bring safe and effective therapies to patients in need.

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**About Alliance for Cell Therapy Now**

Alliance for Cell Therapy Now (Alliance) is an independent, non-profit organization guided by leaders representing academic and medical institutions, industry innovators, and patients, that is working to advance safe and effective regenerative medicine and cell therapies for patients in need. For more information, go to http://allianceforcelltherapynow.org/

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For more information, contact Alliance for Cell Therapy Now via email at communications@allianceforcelltherapynow.org.