



October 28, 2019

The Honorable Michael Shirkey  
Senate Majority Leader  
Michigan Senate  
100 North Capitol Avenue  
Lansing, MI 48933

The Honorable Lee Chatfield  
Speaker of the House  
Michigan House of Representatives  
100 North Capitol Avenue  
Lansing, MI 48933

Re: The CON's Proposed New Standard for Immune Effector Cell Therapy (IECT) Services is Outside the Authority and Intent of the CON Commission

Dear Mr. Majority Leader and Mr. Speaker,

The Alliance for Regenerative Medicine (ARM) is writing to express its concern with the new Certificate of Need (CON) Commission requirements in Michigan for CAR T therapy. While ARM understands the intent of the new standards, we are very concerned about the precedent of having the CON Commission create a new standard of eligibility of a site of care that wishes to provide cutting edge cellular immunotherapies. ARM agrees with the CON that the patient's safety comes first and that access to appropriate therapies must occur at the right place and at the right time. ARM believes that the current U.S. Food and Drug Administration (FDA) label requirements and the Risk Evaluation and Mitigation (REMS) for each cellular therapy provides the appropriate safety criteria and standards.

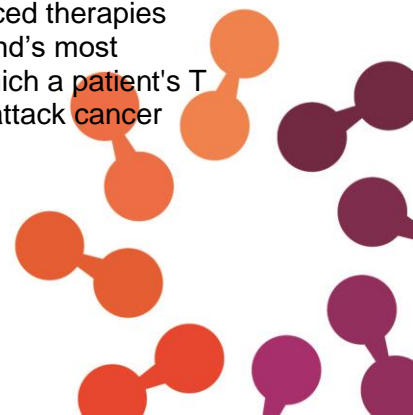
ARM is an international multi-stakeholder advocacy organization that promotes legislative, regulatory, and reimbursement initiatives necessary to facilitate access to life-giving advances in regenerative medicine worldwide. ARM comprises more than 350 leading life sciences companies, research institutions, investors, and patient groups that represent the regenerative medicine and advanced therapies community. ARM takes the lead on the sector's most pressing and significant issues, fostering research, development, investment, and commercialization of transformational treatments and cures for patients worldwide.

The regenerative medicine and advanced therapies sector is the next frontier in the fight against some of humankind's most devastating diseases and disorders. As of year-end 2018, ARM estimates there are 906 regenerative medicine and advanced therapies developers worldwide sponsoring 1,028 clinical trials across dozens of indications, including oncology, cardiovascular, central nervous system, musculoskeletal, metabolic disorders, ophthalmological disorders, and more.<sup>1</sup>

A subset of these clinical trials focuses on the power of chimeric antigen receptor (CAR T) therapies. These therapies are the first in a wave of new and exciting advanced therapies and technologies that are the next frontier in the fight against some of humankind's most devastating diseases and disorders. CAR T therapy is a type of treatment in which a patient's T cells (a type of immune system cell) are changed in the laboratory so they will attack cancer

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<sup>1</sup> <https://alliancerm.org/publication/2018-annual-report/>



cells. T cells are taken from a patient's blood, as it flows through a tube to an apheresis machine, which removes the white blood cells, including the T cells. Then, the gene for a special receptor called a chimeric antigen receptor (CAR) is inserted into the T cells in the laboratory. Millions of the CAR T cells are grown in the laboratory and then given to the patient by infusion. The CAR T cells are able to bind to an antigen on the cancer cells and kill them.<sup>2</sup> ARM is currently tracking the outcomes of the approximately 158 ongoing clinical trials using the CAR T technology in a variety of stages of cancer and cancer types. ARM believes that this new and promising technology provides the possibility that most future treatments for many types of cancer at its many stages will focus on using the power of the patient's immune system to fight their particular disease.

What is critical about all the technologies represented by ARM, including CAR T, immunotherapy, and cell and gene therapy, is that many of the products are transformative – they provide a durable therapeutic benefit or even a cure with a single administration of the therapy. The potential for dramatic clinical benefit is why these innovations are changing medical care and must be considered as part of the solution and not as part of the problem of rising overall drug costs. ARM believes that we are at the beginning of our scientific journey to curing many of these diseases and urges the legislature to work with all stakeholders in order to ensure safe beneficiary access to these classes of therapies.

In addition to CAR T therapies, other powerful technologies under clinical development could be negatively impacted by additional CON requirements in the future. Below we define other types of therapies that can be impacted in the future.

- Cell therapy is broadly defined as the administration of viable, often purified cells into a patient's body to grow, replace, or repair damaged tissue for the treatment of a disease. A variety of different types of cells can be used in cell therapy, including hematopoietic (blood-forming) stem cells, skeletal muscle stem cells, neural stem cells, mesenchymal stem cells (adult stem cells that differentiate into structures as connective tissues, blood, lymphatics, bone, and cartilage), lymphocytes, dendritic cells, and pancreatic islet cells.
- Cell therapies may be autologous, meaning that the patient receives cells from their own body, or they may be allogenic, meaning the patient receives cells from a donor. Allogeneic cell therapies are often referred to as "off-the-shelf" therapies, as they are derived from a donor who is not the patient, enabling advance preparation and available to the patient immediately at the time of need.
- Many cell-based therapies currently being developed utilize induced pluripotent stem cells (iPSCs). Unlike embryonically derived pluripotent stem cells, these are adult cells that have been genetically reprogrammed back into a pluripotent state, capable of becoming one of many types of cells inside a patient's body. This technology may enable the development of an unlimited type of a specific type of human cells needed for therapeutic purposes.
- ARM members are currently developing cell therapy approaches to treat diseases and disorders that include chronic heart failure, Crohn's disease, ALS, ischemic stroke, diabetes, Parkinson's disease, degenerative disc disease, and more.

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<sup>2</sup> <https://www.cancer.gov/publications/dictionaries/cancer-terms/def/CAR-T-cell-therapy>

- Tissue engineering combines scaffolds, cells and biologically active molecules into functional tissues to restore, maintain or improve damaged tissues. Biomaterials are medical devices designed to interact with living systems, providing physical structures and support for engineered tissues. ARM members are currently developing tissue-engineered products and biomaterials to treat cartilage damage and degeneration, wound repair, spinal cord injury, hernia repair, and more.
- Gene therapy seeks to modify, replace, inactivate or introduce genes into a patient's body with the goal of durably treating, preventing or even curing disease. Gene therapy techniques include genetically modifying a patient's cells outside of their body, which are then re-introduced to deliver a therapeutic effect, an approach known as gene-modified cell therapy. ARM members are currently developing gene therapy and genome editing approaches to treat inherited blood disorders beta-thalassemia and sickle cell diseases, blood cancers leukemia and lymphoma, inherited retinal disease, Huntington's disease, and more.

ARM appreciates the opportunity to share its concerns around the precedent that the Michigan CON Commission decisions may have on access to transformative cell and gene therapies today and of the future. Additionally, given current FDA requirements, ARM believes that the additional CON requirement is unnecessary. ARM looks forward to working with you to advance policies that advance access to these innovative therapies and benefit patients. If you have any questions or need any additional information, please do not hesitate to contact me at [rfalb@alliancerm.org](mailto:rfalb@alliancerm.org)

Sincerely,



Robert Falb  
Director, U.S. Policy and Advocacy

cc: Members of the Michigan Senate  
Members of the Michigan House of Representative