



*FY 2020 IPPS New Technology Add-on Town Hall Meeting
December 4, 2018*

Good morning. My name is Robert Falb and I am the Director, U.S. Public Policy and Advocacy for The Alliance for Regenerative Medicine (ARM). Thank you for the opportunity to make the following comments today.

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. Our membership includes an international community of small and large companies, non-profit research institutions, patient organizations, and other sector stakeholders dedicated to realizing the promise of regenerative medicine for patients around the world. The Alliance 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 the end of Q3 2018, ARM estimates that there are more than 890 regenerative medicine and advanced therapy developers worldwide sponsoring 1003 clinical trials across dozens of indications, including oncology, cardiovascular, central nervous system, musculoskeletal, metabolic disorders, ophthalmological disorders, and more. These therapies have the potential to revolutionize treatment for some society's most devastating diseases.

First, a quick primer on the various technologies that comprise this sector.

- Cell therapy is the administration of viable, non-genetically modified cells into a patient's body to grow, replace, or repair damaged tissue for the treatment of a disease. The administered cells can be allogeneic, meaning the patient receives cells from a donor, or autologous, meaning the patient receives cells from his or her own body. 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 disk disease, and more.
- 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 his or her body, then introducing the modified cells to deliver a therapeutic effect. ARM members are currently developing gene therapy and genome-editing approaches to treat inherited disorders such as beta-thalassemia, sickle cell disease, Huntington's disease, and inherited retinal diseases, as well as blood cancers such as leukemia and lymphoma.

ARM Recommendations:

Last Year, ARM urged CMS not to apply their substantial clinical improvement criteria to regenerative medicine therapies because these criteria are likely outside the original Congressional intent and are duplicative of the FDA approval rules related to expedited approval programs such as Breakthrough Therapy designation. Specifically, the FDA defines "Breakthrough therapy" as a therapy "that may demonstrate substantial improvement over existing therapies."

In addition, the Regenerative Medicine Advanced Therapy (RMAT) designation is granted to products that are intended to treat, modify, reverse, or cure a serious or life-threatening disease or condition for which clinical evidence shows potential to meet an unmet medical need. The criteria relied upon by the FDA in assigning these designations mirror those for satisfying substantial clinical improvement.

In response, CMS stated that if the technology has a FDA designation that is similar to the substantial clinical improvement standard, the technology should be able to demonstrate that it meets the substantial clinical improvement criterion. The agency further added that it does consider FDA approvals and designations in its evaluation of NTAP applications.

ARM appreciates CMS' statements related to the similarity between data satisfying FDA designations and data satisfying substantial clinical improvement. This clarity will help future applicants understand which types of data can serve as the foundation for the satisfying the substantial clinical improvement criterion. In addition, ARM appreciates that CMS further clarified that it accepts a wide range of data, including peer reviewed articles, study results, letters from major associations, or any evidence that would support the conclusion of substantial clinical improvement. ARM understands this to mean that CMS appreciates and considers the patient experience and point of view in its determination of substantial clinical improvement, which it hopes the agency will confirm in upcoming rulemaking.

In addition, ARM urges CMS to change the payment rate for certain classes of NTAP recipients such as those mentioned above. ARM is concerned that without changes to the current reimbursement methodology for CAR-T therapies and other regenerative and cell therapies that ARM represents, the NTAP will not satisfy the congressional intent of the NTAP program, which is to adequately reflect the estimated average cost of such service or technology. Only with a more appropriate reimbursement methodology that will improve access in the inpatient setting will beneficiaries be able to truly experience the substantial clinical improvement and clinical benefit that these therapies provide.

Conclusion:

In conclusion, ARM believes that the field of regenerative medicine has the potential to heal people and bend the health cost curve toward lower long-term costs and higher quality outcomes. This trend is already evidenced by several approved and marketed regenerative medicine products that are demonstrating significant clinical value. Specifically, these therapies could reduce hospital care, the need for physician, clinical nursing, or professional services, and home healthcare, thereby substantially reducing overall healthcare expenses. ARM is confident that meaningful improvements in clinical outcomes and cost reduction can be accomplished through regenerative and cell and gene medicine technologies.

Thank you.