

Response to the Health Future Task Force Treatments Subcommittee Request for Information

The Treatments Subcommittee of the Health Future Task Force in the United States House of Representatives issued a Request for Information (RFI) from stakeholders regarding medical innovation in the United States, which the stated of goal of increasing the availability and development of life-saving treatments for patients, while addressing rising costs. In the RFI, the subcommittee stated that they had four primary goals, the first goal of which was to evaluate innovative payment solutions for expensive curative therapies in Medicare and Medicaid.

The American Society for Transplantation and Cellular Therapy (ASTCT) believes this is an important goal and wishes to submit its response to this request. We are grateful for the opportunity to respond that is afforded by this RFI. Our responses to this primary goal are similar to what we have stated in the past when discussing this topic for Cures 2.0. The ASTCT believes that modernization is required in the Medicare program to ensure patient access for cell and gene therapies. Many of these therapies present innovations over existing treatment options and, in some cases, may be the only treatment option available to patients.

These advanced therapies also come with high costs. The ASTCT does not condone high-priced drugs; however, as a Society of physicians, researchers, and other professionals, we understand the value of these therapies to patients and are concerned the high costs and burdens that patients and providers face will continue to present barriers to access to these therapies, and unsustainable costs to providers to offer them.

Our members must be able to make clinical decisions with the best interest of the patient in mind, not the cost of therapy, and these decisions must be supported by coverage and payment structures that do not impede the development and adoption of innovative therapies. Our recommendations and responses to the questions posed in this RFI are focused on ways that Congress and the HHS Secretary can modernize outdated Medicare reimbursement processes that do not account for the complexity of both the delivery and costs of these new therapies.

Particularly, as part of the RFI's outline of the first goal, the subcommittee inquired of stakeholders and other respondents if the potential site of care delivery and Medicare reimbursement impacts the provision of novel therapies and gave the example of CAR-T therapy. The ASTCT's members are clinicians who administer CAR-T therapy and contribute to clinical research for CAR-T and other types of *ex vivo* hematopoietic stem cell based gene therapies, so we felt it was important to weigh in on this question in particular.

Overall, Medicare reimbursement systems are not designed to handle the costs and complexities that are introduced by cell and gene therapy products. These costs and complexities do not arise merely from the cost of a product alone, but other associated services and procedures.

The ASTCT feels there are short- and long-term changes to Medicare reimbursement that could be made to improve access to these therapies and modernize reimbursement.

First, we believe that **Medicare must recognize and cover costs for cell collection and cell processing for cell and gene therapies, as these costs represent hospital services**, ordered by a physician, provided to patients by clinical staff, and come with the same risks, obligations, and compliance requirements as any other hospital service. Today, these services are not covered or paid and providers bear the costs. These services are not part of the manufacturing process, since the manufacturer exerts no control over the services, nor does the manufacturer provide compensation for these services.

Secondly, we believe that to ensure these therapies have the best chance to be effective for patients, it is important that **prophylactic cell collection for potential later use should be covered and reimbursed**, since some patients may be at risk for developing a disease, or their existing disease may worsen, and earlier collection and cryopreservation of cells may improve outcomes when the therapy is later created utilizing “less damaged” cells.

Medicare inpatient fee-for-service reimbursement currently utilized the New Technology Add-On Payment (NTAP) program to help offset the costs of new technologies. However, the process of applying for NTAP creates challenges for both applicants as well as hospital providers of these new therapies. NTAP applications are evaluated through the IPPS rule-making cycle, which creates issues with FDA approval deadlines.

Specifically, an approval that comes outside of the rule-making cycle deadline (even by days) results in products losing NTAP approval for months or up to a year after launch, which can limit a product’s adoption/use for Medicare patients. **We recommend modernizing the NTAP application process by requiring a twice-per-year evaluation/approval of NTAP and directing the Secretary to increase the NTAP cap to reimburse providers for new technologies in line with the cost to provide them.**

Finally, we believe that long-term changes to Medicare reimbursement is required to ensure that the program can handle the growth in new therapies and so that Medicare beneficiaries will be able to access new innovative curative therapies. Structural overhauls in particularly to the IPPS Medicare payment systems are needed, since cell and gene therapies do not fit under the current MS-DRG rubric of medicine/surgery.

However, modernizing payment systems will involve careful study and testing to ensure that new solutions are equitable, scalable, and functional. Therefore, **we recommend that an advisory group be created and funded to study and recommend structural changes to the existing IPPS Medicare payment methodology for cell and gene therapies in order to modernize this payment system.** Such a process will also facilitate identification of the most appropriate way to move beyond the existing Medicare IPPS rate-setting methodology of charges reduced to cost.

The ASTCT is grateful for the opportunity to respond to this RFI. The ASTCT welcomes the opportunity to discuss these recommendations in more detail or to answer any questions you may have. Please contact Alycia Maloney, ASTCT Director of Government Relations, at amaloney@astct.org for any follow up issues.

Sincerely,

A handwritten signature in blue ink, appearing to read 'S. Davies'.

Stella M Davies, MBBS, PhD, MRCP
President, ASTCT