



American Society for  
Transplantation and Cellular Therapy

**SUBMITTED VIA EMAIL**

January 22, 2024

The Honorable Bill Cassidy, MD  
Ranking Member  
U.S. Senate Committee on Health,  
Education, Labor, and Pensions  
455 Dirksen Senate Office Building  
Washington, DC 20510

Dear Ranking Member Cassidy:

The American Society for Transplantation and Cellular Therapy (ASTCT) appreciates the opportunity to submit comments on the Request for Information (RFI) posted on December 5, 2023 regarding the importance of gene and cellular therapies. As a professional membership association of physicians and healthcare providers in stem cell transplant, we have firsthand knowledge of the challenges of integrating new and potentially curative therapies for our patients. We understand the delicate balance between access to therapies and price limitations and we hope that we can continue to provide the best care for patients based on our clinical expertise rather than financial decisions. We are encouraged to see Congress taking an interest in shaping the solutions for better access to care for patients with ultra-rare diseases and encouraging innovation in this field of medicine.

The ASTCT is a professional membership association of more than 3,700 physicians, scientists, and other health care professionals promoting blood and marrow transplantation and cellular therapy through research, education, scholarly publication, and clinical standards. Our Society's clinical teams have been instrumental in developing and implementing clinical care standards and advancing cellular therapy science, including participation in trials that led to current Food and Drug Administration (FDA) approvals for chimeric antigen receptor T-cell (CAR-T) therapy and hematopoietic stem cell-based gene therapies for genetic immune system and blood disorders.

We are pleased to present our answers (in bold below) to the questions put forth by your committee. ASTCT's Government Relations Committee composed the following:

1. How should lawmakers define an "ultra-rare" disease or disorder cell or gene therapies should be eligible for inclusion in new coverage or contracting requirements for those patients with an ultra-rare disease or disorder? What definitions should lawmakers consider?



American Society for  
Transplantation and Cellular Therapy

**NIH and FDA define a rare disease as one that affects fewer than 200,000 in the US - about 1 in 1650. Extremely rare diseases (sometimes referred to as “ultra-rare”) have a prevalence of <1 per 50,000. (Science. 2022 Aug 19; 25(8): 104698.)**

3. How do patient populations currently access and pay for these therapies?

**Sometimes public foundations help patients pay - through foundation grants. Sometimes patients have to travel long distances to get specific treatment for rare conditions (like transplant for glycogen storage diseases) and the foundations help support them. However, this is not true for *every* patient needing these therapies.**

7. What, if any, are the utilization management tools (e.g. step therapy, prior authorization) that patients are typically subject to when paying for and accessing these therapies? If not the patient, what individual or entity typically works through the process of obtaining approvals?

**Provider organizations generally drive the prior-authorization request for a high-cost therapeutic for patients that meet clinical indications and express interest in receiving the therapy. The standard approach is to compile supporting documentation such as peer-reviewed clinical research articles, patient specific clinical records, and a customized letter to the payer (written by the prescribing clinician) to support receiving prior-authorization from the payer to render the service. Many times, high-risk and high-cost drugs require an additional conversation with the payer’s utilization management clinical liaison (often called a Medical Director) to talk about the patient’s case. High-risk, high-cost, and highly personalized therapies require a resource intensive prior-authorization workflow for most cases.**

11. What does coverage for these therapies typically look like? What does the landscape look like for coverage of these therapies?

**Right now access is poor because there is lack of transparency around coverage from Medicaid, an example being for sickle cell gene therapy. Without knowledge of the coverage, patients are not receiving the treatments without further insight on what Medicaid’s policies will cover for the therapy.**

34. How does a physician or health system initiate the process of prescribing a patient with an ultra-rare disease or disorder one of these therapies?

**The process is initiated after the patient is clinically assessed for fitness to proceed, has expressed interest in proceeding, and financial clearance (including but not limited to prior-authorization and contracting, as needed) is obtained. Once these three critical components have been addressed, the provider and their team can place a manufacturer order directly with the manufacturer to begin the ordering process.**

**Financial clearance is an important requirement and lack of it holds up patient care. In addition, some access to care is dependent on institutional support - having the right programmatic resources. Providers are thinking through how to set up their gene therapy programs, which may require more staff such as navigators/social workers. Not all institutions have these resources available to allocate to a specific gene therapy program.**

35. Do physicians or health systems bear any financial risk as part of prescribing a patient with an ultra-rare disease or disorder these therapies? If so, as part of what program or what type of contract?

**Yes, many health systems will not allow physicians to prescribe these therapies until fully vetting the process and ensuring payment, which is a lengthy and in depth process, due to how expensive these treatments can be.**

36. What is the typical communication between the physician, health system, and manufacturer as a part of prescribing a patient with an ultra-rare disease or disorder these therapies?

**Cellular gene therapies are made by modifying a collection of a patient's own cells. As such, communication between physicians, health systems, and manufacturers is critical. To initiate an order for a patient's personalized therapy, an order must be placed in a manufacturer-specific portal. Once an order is placed, a manufacture date is assigned and the patient's cell collection can be scheduled. Interim care is organized around the collection date. At the completion of collection, the cells are shipped to the manufacturer to begin the modification process. Close communication with the manufacturer is required to confirm that the collection procedure was completed as planned and that the courier service has picked the cells up for transport as planned.**

**Once the manufacturer receives the cell product and the process begins, it is critical for the manufacturer to provide updates on the progression of the product as it moves through the process. Prior to receipt of the product, the manufacturer and health system coordinate the delivery of the cells and the health system verifies**

**receipt and condition upon arrival. These are “living drugs” and are treated as such throughout the 6 week to 6 month manufacturing and release process.**

39. What is the appropriate role of the federal government in ensuring access to these therapies in the commercial market? How can any steps taken on the federal level ensure expanded access while not hurting innovation in this area?

**The federal government is uniquely suited to protect coverage and access to these life-saving therapies, especially for patients with Medicaid coverage. Due to their operational complexity, financial risk, and requirement for highly skilled and experienced clinical care, these therapies are not provided in all centers. Centers are disproportionately located throughout the country and patients frequently need to leave their state to access treatment. The current Medicaid system is not designed to support access to these types of therapies, and many barriers to care exist. This creates inequitable access to these novel treatments, from the start.**

**Barriers include multi-week out of state request review periods, single case agreement (contract) negotiation between the Medicaid plan and healthcare provider to ensure reasonable payment, lack of provider enrollment reciprocity between states, and lack of consistent travel support for patients seeking care that is otherwise unavailable to them in-state. All of these issues create long delays for patients seeking care, and are often absolute roadblocks. *A federal solution, such as carving out the management of cell and gene therapy, could make it possible for patients to access the care they need, in a way that is efficient and doesn't place undue financial and operational burden on providers and the patients they serve.***

**Additionally, the federal government is uniquely positioned to set the bar for coverage of these expensive yet lifesaving novel therapies by making coverage determinations for Medicare patients. Moreover, the federal government could influence state Medicaid plan decisions for coverage, too, by offering financial or other incentives to states to cover treatment for children or all Medicaid-covered patients.**

40. Should the federal government mandate coverage of these therapies? What markets (e.g. small, large group markets) or plans should be required to cover these therapies?

**The federal government can heavily influence the willingness of Medicaid and private payer plans to cover these therapies by demonstrating the value of these therapies. The ICER report on the value of gene therapies for sickle cell disease is a helpful starting point, as the report factors in a lifetime of costs, both monetary and otherwise, that are accumulated per patient with sickle cell disease and compares it to the lifetime costs of a patient whose sickle cell disease is cured by gene therapy.**

**Many practicing physicians expect that the costs saved, in addition to the lives saved, will be immense by providing cures earlier in life. Studies and financial modeling that demonstrates as much will enable payers to rationalize coverage. The federal government could commission such a study, through either CMMI or GAO or other necessary agency.**

41. What are the anticipated costs or savings to health systems, plans, payers, or patients as a greater number of these therapies become available?

**The anticipated costs include the product itself, plus the hospitalization(s) involved in providing the therapy and the management of any complications. The savings include a lifetime of hospitalizations, medications, and complications that can be avoided by curing the underlying disease. Furthermore, by enabling patients who previously had a life-limiting condition to live a full life, and also by removing a chronic condition that can often limit their full participation in school and work, you are giving a major net positive boost to the society and economy overall. Therefore, not all of these things can be quantified in terms of cost savings.**

42. How should anticipated benefits from these therapies be evaluated against the potential costs of these therapies?

**The costs of these therapies should be understood in the context of the monetary savings of curing a lifelong and life-limiting disorder, and must be viewed in the context of the ethical obligation to right the historic wrong of many decades of underfunding research for curative therapies that would benefit many underprivileged minorities.**

44. How can future payment or coverage models for these therapies be designed in a way that drives down total health costs for the patient?

**Outcomes based agreements with companies/manufacturers helps ensure that companies develop therapies that are maximally successful and safe. This also helps drive down cost by making sure that payers are not reimbursing for expensive treatments that do not provide a cure.**

47. How quickly should these covered therapies be made available to patients?

**Therapies should be made available to patients as soon as possible following FDA approval, when safety and efficacy have been demonstrated. Unnecessary delay in**



American Society for  
Transplantation and Cellular Therapy

**access to these therapies can be life threatening in many circumstances and can push patients towards less-efficacious and more toxic interim therapies.**

**There have been instances of payers excluding gene therapy treatments approved via FDA's Accelerated Approval pathway citing lack of safety and efficacy data. This is an extremely alarming trend for a few reasons. The first, this creates disparate and random access to FDA approved therapies that are for the treatment of serious, life threatening diseases. Second, excluding coverage for drugs approved via Accelerated Approval has the potential to be very damaging to the future investment in and development of innovative therapies for rare diseases. Without coverage for these FDA approved treatments, there will be very little incentive for future development of new drugs for rare diseases. Lastly, without real-world use after a drug is FDA approved, there is no way to study real world outcomes to ensure efficacy and safety expectations are met.**

48. What other considerations should be made around benefit design to ensure access to these therapies (e.g. deductibles, cost-sharing)?

**When designing coverage plans, care should be taken to remember that high deductibles or excessive cost sharing would effectively render these therapies unattainable to large segments of the populations that need them. In addition, patients with these disorders already spend a large amount of money accessing medical care as is.**

49. Should healthcare providers share in the financial risk of prescribing these therapies to patients? Why or why not?

**No, healthcare providers are already working incredibly hard to reformat their operations, provide education, and establish new systems to even be able to provide these therapies at all. Asking providers to share in the financial risk will sharply limit access for patients.**

ASTCT hopes that our answers to some of these questions provides clarity on the topics relating to innovation in therapies and access within the U.S. We strongly support any efforts to address patient access to care and ways to best serve our patients in the most clinically proper manner. Innovation and new therapies have a direct impact on patient access to life-saving therapies and we thank you for your efforts in helping.

If you have any questions or need clarification please contact Alycia Maloney, Director of Government Relations for ASTCT, at [amaloney@astct.org](mailto:amaloney@astct.org).



American Society for  
Transplantation and Cellular Therapy

Sincerely,

A handwritten signature in blue ink, appearing to read "M-A P" followed by a horizontal line.

**Miguel-Angel Perales, MD**

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Chief, Adult Bone Marrow Transplantation Service

Attending Physician and Member

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