

Ensuring Patient Access to Transformative Therapies

Issue Background

New, innovative treatments – “Transformative Therapies” – provide a significant, durable benefit and value for patient health outcomes, delivery of care, and overall healthcare spending. These therapies are aimed at serious and rare diseases where patients often have limited treatment options. Examples of transformative therapies include cellular and gene therapies, which are truly personalized medicines that target treatment to specific patient populations or subsets of patient populations. These innovative drugs and biologics have the potential to transform the treatment of serious and rare disease, but patient access will be at risk until existing reimbursement pathways are updated to reflect the fast pace of innovation. Further, novel payment mechanisms should be considered that reflect the full value of these transformative therapies and the promise they bring to seriously ill patients.

Policy Position

BIO believes it is imperative that the reimbursement system appropriately and adequately reflect the value of these therapies by ensuring timely, appropriate payment under the existing reimbursement structure. Securing adequate reimbursement will facilitate patient access to these transformative therapies in a timely manner, which is critical when considering the current health status of patients for whom these transformative therapies are intended and clinically indicated. The following policy principles should be applied in any approach to reimburse for transformative therapies:

- **Robust reimbursement across sites of care:** Ensuring that physicians and other providers can provide access to the most appropriate course of treatment in the preferred setting for each patient is critical. This can be achieved through reimbursement that fully recognizes the value of these therapies and the associated care and services required for their delivery.
- **Avoiding a one-size-fits-all approach to the coverage of transformative therapies:** Each therapy in the transformative medicines space will have individualized applications and require unique clinical circumstances, even though they may have similarities in mechanism of action or route of administration. Patients will be treated across varying healthcare settings with differing associated care needs by specialized providers with significant experiences and expertise in providing transformative therapies. The reimbursement system should ensure appropriate reimbursement for these therapies and that providers have the ability to provide these groundbreaking therapies through adequate reimbursement for the associated care they deliver. Novel payment approaches should be considered to help facilitate patient access to continued innovations in care and treatment.
- **Incorporating transformative therapies into the reimbursement structure upon their approval, and not dependent upon existing timelines:** The current coverage and reimbursement system is not structured in the most efficient and effective way to ensure timely patient access of these transformative therapies. As more of these therapies come to market, it will be critical for existing coding and reimbursement pathways and timelines to be adequately flexible to provide timely coverage and access of these therapies for patients that need them.

Key Points

- ✓ Transformative therapies address serious diseases with a high unmet need, serve small patient populations, and provide a substantial durable benefit.
- ✓ It is critical that transformative therapies are adequately reimbursed to ensure providers can deliver these treatments to patients in the most appropriate site of care in a timely manner.
- ✓ BIO is committed to working in partnership with CMS and other stakeholders to ensure patient access to continued innovation over the long-term.