VIA ELECTRONIC DELIVERY

January 22, 2024

Senator Bill Cassidy, M.D.
U.S. Senate Committee on Health, Education, Labor, and Pensions

Via email to: GeneTherapyCoverage@help.senate.gov

Re: RFI on Improving and Protecting Access to Gene Therapies

Dear Senator Cassidy,

The Biotechnology Innovation Organization (BIO) appreciates the opportunity to comment on the U.S. Senate Committee on Health, Education, Labor, and Pensions' (Committee's) Request for Information on Improving and Protecting Access to Gene Therapies (the RFI). BIO strongly supports efforts to help improve patient access to, and the affordability of, the amazing medical breakthroughs that our member companies are developing, and we pledge to work constructively with Congress to achieve this goal.

BIO is the world's largest trade association representing biotechnology companies, academic institutions, state biotechnology centers, and related organizations across the United States and in more than 30 other nations. BIO's members develop medical products and technologies to treat patients afflicted with serious diseases, to delay the onset of these diseases, or prevent them in the first place. In that way, our members' novel therapeutics, vaccines, and diagnostics not only have improved health outcomes, but also have reduced healthcare expenditures due to fewer physician office visits, hospitalizations, and surgical interventions. BIO membership includes biologics and vaccine manufacturers and developers who have worked closely with stakeholders across the spectrum, including the public health and advocacy communities, to support policies that help ensure access to innovative and life-saving medicines and vaccines for all individuals.

BIO's member companies work to discover innovative, transformative therapies, including cell and gene therapies (CGTs), that provide a significant, durable benefit and value for patient health outcomes, delivery of care, and overall health care spending. These novel therapies are aimed at serious and rare diseases where patients often have limited treatment options. Taken together, our companies offer hope for cures and treatments where there was none, help reduce health care costs, and ensure a better quality of life.

Introduction

BIO applauds your leadership in bringing attention to the significant challenges that patients with rare diseases face in gaining access to innovative therapies, including potentially curative treatments such as CGTs. As noted in your RFI, a relatively small number of these therapies are available today, but the pipeline of CGTs is robust and the number of FDA approved CGTs is expected to grow dramatically over the next decade. Companies are actively developing innovative payment models to address the initial high costs, long-term cost uncertainties, and direct and indirect cost offsets associated with CGTs in both public and private health care coverage. Further, as per the cycle of biopharmaceutical innovation, many treatments will eventually lose exclusivity, becoming significantly more affordable over time. CGTs – and other innovative treatments in the drug development pipeline – offer tremendous hope for the

millions of Americans who are faced with a rare disease diagnosis and who have no treatment options today.

Our response below will holistically address the questions in the RFI, primarily focusing on the questions: "Which Treatments Should Be Included;" "What is the Current Practice for Patients with Ultra-Rare Diseases or Disorders;" "What Is the Future of Access for These Therapies;" "How Should Federal or State Governments Promote Access to New Models;" and "How Should Lawmakers Seek to Evaluate and Accomplish These Policy Goals?" Our entire response is intended to showcase how lawmakers can promote access to transformative therapies. First, we highlight our principles for promoting patient access to CGTs for patients faced with diagnosis of a rare disease and offer considerations for the Committee regarding the scope of this RFI both for the types of therapies that are important to patients with a rare disease and the universe of patients facing a rare disease diagnosis. Next, we establish the need for solutions to be patient-driven and paid for on a holistic assessment of value. After, we discuss increasing access through market solutions and reform of today's legacy payment systems. Finally, we lay out our general principles for payment reform to facilitate better access to innovative treatments.

BIO's Policy Principles for Expanding Access to Cell and Gene Therapies and other Innovative Treatments Critical to Rare Disease Patients

- **Patient Centeredness Is Key:** Any legislative action should prioritize the health and wellbeing of patients and support patient access to needed therapies. It is important lawmakers continue to engage in robust stakeholder dialogue to inform the accuracy and appropriateness of different payment arrangements.

- **There Is Likely No Single Solution**: Not all payment arrangements are appropriate for all therapies, emphasizing the need for innovation to determine suitable arrangements for different therapeutic approaches and patient profiles. Fostering innovation will enable the market to explore the unique attributes of each therapy and its compatibility with various coverage models and innovative payment approaches.

- **Recognize Value in Form and Time:** Pricing assumptions often overlook the societal value and long-term savings associated with these therapies that surpass the initial cost of the therapies. It is critical that policymakers carefully evaluate both medical and non-medical long-term savings when evaluating payment arrangements for transformative therapies.

- **Modern Payment Systems for Modern Treatments:** To foster the adoption of innovative therapies, policymakers should remove access barriers on patients and reform legacy payment systems in a way that ensures continued innovation.

I. What Are Cell and Gene Therapies? How Do Other Types of Innovative Therapies Address Unmet Needs in Rare Diseases?

The following section is intended to address the question "Which Treatments Should be Included," specifically regarding questions 1 and 2 in the RFI.

BIO agrees with the Committee regarding the need to prioritize and maximize patient access to novel therapies, particularly CGTs. Cell therapies and gene therapies are two distinct

treatment types with their own unique characteristics and sophisticated manufacturing process. In some cases, CGTs are tailored based on a patient's genetic makeup, and in such circumstances, therefore, are unique. Accordingly, CGTs undergo specialized manufacturing and delivery processes that are rapidly developed to suit the individual needs of each patient. This in turn creates unique challenges around time sensitivity, sophisticated logistics, delivery systems that can accommodate genetic testing, distinctive quality controls, and other considerations that impact patient access to these therapies.

Cell therapy refers to the use of whole cells to treat disease. This can include replacing or repairing tissue and/or cells damaged by disease or attacking cancer cells. The cells can originate from the patient (autologous source) or from a donor (allogeneic source). Cells can be derived from stem cells, such as bone marrow, reprogrammed mature cells, such as induced pluripotent stem cells (iPSC), and differentiated cells produced from stem cells in a lab. Cell therapy may be used as part of a therapy or treatment for a variety of diseases and conditions such as cancer, sickle cell disease (SCD), beta thalassemia, or HIV.

Gene therapy is a type of medicine designed to treat a genetic disease by adding the functioning gene or genes into a specific cell (e.g., liver cells, bone marrow cells), which allows the patient's body to return to good health. Gene therapy can also be used to reduce the activity of a harmful gene. Gene therapy can happen ex vivo (outside the body) or in vivo (inside the body). Each delivery method has benefits and limitations, and preference of method depends on the disease being treated. Currently there are many gene therapies that are FDA approved[1] and that are being developed to treat multiple diseases, including hemophilia, inherited retinal diseases, myeloma, phenylketonuria (PKU), and Huntington's disease.

CGTs face different financing and reimbursement challenges depending on their administration in inpatient, outpatient hospital, or physician office settings. For instance, in the outpatient setting, CGTs are subject to federally mandated discounts through the 340B Drug Discount Program (340B Program). The expanded scope of the 340B program has made it more difficult for the biotechnology industry to pursue new medical advancements including the development of CGTs and has created other unintended consequences including higher list prices for health care purchasers. In a 2000 study, the Government Accountability Office (GAO) warned about these unintended consequences when it concluded that expanding government discounts on drugs could lead to higher prices for other patient populations, including the privately insured.[2] Accordingly, the 340B Program poses considerable challenges for the financing and reimbursement CGTs. Unlike other drugs or treatments, CGTs require specialized providers and health care facilities that are capable of administering such personalized medicines. Therefore, it may not be appropriate for all covered entities to receive 340B discounts on all certain drugs, such as CGTs.

In 2022, we commissioned analysis from Avalere Health that assessed what we refer to as the "transformative therapies" pipeline and related patient access challenges by payor market.[3] This analysis identified over 200 therapies in the drug development pipeline for conditions that currently have limited or no treatment options and that may present significant challenges related to payment and access that vary by payer market and setting of care, such as

[1] Approved Cellular and Gene Therapy Products. FDA. December 2023.
[2] U.S. Government Accountability Office, Report to Congress, "Expanding Access to Federal Prices Could Cause Other Price Changes," August 2000, available at: http://www.gao.gov/archive/2000/he00118.pdf.
[3] Stengel, Kylie et al. Pipeline Transformative Therapies May Require Payment Model Innovation. Avalere. May 2023.

inadequate reimbursement in certain settings due to bundled payments. The vast majority of the transformative therapies in Avalere's analysis are CGTs. But given the substantial pace of innovation, we caution against making policy around restrictive or limiting terminology that would exclude patient populations that could otherwise benefit from improved access to necessary treatments through innovative coverage and payment models but might not be strictly classified as a cell or gene therapy.

Further, bringing attention to unmet medical needs necessitates a focus not only on ultra-rare diseases, but all rare as well as non-rare diseases. CGTs treat not only rare diseases but are also in development for non-rare diseases. Indeed, our analysis from Avalere found that while the majority (80%) of the therapies in the pipeline are for rare diseases, another 12% are aimed at patient populations that are small but non-rare (e.g, patient populations in the hundreds of thousands) and approximately 8% target larger patient populations (e.g., potentially millions of patients). While rare diseases often lack significant attention and resources due to their small patient populations, vulnerable patient populations face increased risk for a multitude of non-rare diseases. To that end, we strongly recommend that the Committee **not** focus solely on "ultra rare" diseases for which no common, accepted definition exists today), nor do we believe that the Committee should attempt to define "ultra rare."

Rather, BIO encourages legislators to consider the remarkable potential of transformative therapies as a whole and their ability to:

- Address significant unmet patient need, improve quality of life for patients, and provide a meaningful improvement over the current standard of care, including for patients who face health disparities;
- Offer significant benefits to the broader ecosystem, including societal benefits and the potential for lowering overall health care costs particularly in the long-term, but also present challenges to the current reimbursement system;
- Cross a broad spectrum of innovation and modalities, including CGTs; and
- Hold the promise of curing, preventing, or halting the progression of disease – fundamentally changing how diseases will be treated in the future.
- Necessitate unique diagnostic, administrative, and treatment requirements such as the need for genetic testing, unique manufacturing needs, and highly specific means to administer therapies.

II. Ensuring Patient-Driven Policies

The following section is intended to address the question "What is the Current Practice for Patients," and "What is the Future of Access for These Therapies" specifically regarding questions 3, 5, 7-10, 39, 44, 46-48, 50, 51, and 53. For answers to the remaining questions in these sections, see our responses in III, IV, and V.

Above all, any solutions to enhance access to novel, innovative therapies put forward by Congress should place the health and wellbeing of patients first to ensure that patients quickly receive the clinically-appropriate care necessary for their condition. Patient-centered solutions prioritize patient access and empower patients to improve their health and enhance their lives with the most appropriate and effective course of treatment for each patient's given condition. To this end, it is critical that patients are involved and provide input into policymaking. Patient feedback is crucial for policies to truly reflect the diverse set of patient experiences, concerns, and needs. Effective policymaking must consider feedback from patients, their families, and representatives to align with the desired outcomes, risks, and other considerations that impact

patients' daily lives. Physicians with expertise in rare disease treatments, as well as those who specialize in the needs of underserved populations, should also be consulted to inform coverage policies to ensure alignment with clinical practices. BIO looks forward to partnering with patients, patient organizations, and Congress to develop patient-centric policies that encourage the development of future innovation while supporting patient access to needed medications.

Improving patient access to transformative therapies, including CGTs, requires examining insurance coverage policies that impose excessive cost-sharing burden and other access barriers on patients. Patients have continued to struggle with cost-sharing burdens controlled by some health plans and to face difficulty getting coverage for the medicines they need. Too often, the most vulnerable patients with chronic or complex illnesses face discriminatory insurance policies that demand higher out-of-pocket costs or deny or limit coverage, hindering the patient's ability to access the most promising treatment plan. It is therefore essential that physicians determine medical necessity and prescribing of rare disease treatment, not insurers. Physician specialists are experts in rare disease treatment, spending years in training, research, and clinical practice. In addition, high out-of-pocket costs can have a significant impact on medication adherence and patient health. Patients are also increasingly being subject to higher cost sharing; in 2022, for covered workers at large firms with specialty drug coverage, the average copayment amount was $110 and the average coinsurance rate was 26%.[4] As patients are burdened by additional out-of-pocket costs, health plans receive millions of dollars in manufacturer rebates from prescription drug purchases that frequently are not used to reduce those out of pocket liabilities.

To reduce patient cost sharing burden, manufacturer patient assistance programs offer financial assistance to individuals who are unable to afford their medications. As the healthcare insurance environment continues to change and more costs are shifted onto the patient, these programs have become even more important to provide access and ensure patient adherence to proven therapies. Unfortunately, insurers have been adopting practices that put barriers between patients and the assistance they need by barring patient assistance from counting toward deductibles and out-of-pocket maximums. This practice- known as accumulator adjustment programs- makes it more likely that patients may choose to forgo needed treatments when confronted with a burdensome copayment or coinsurance amount. It is critical that legislators act to improve affordability by ensuring that insurers make manufacturer patient assistance programs count toward patient OOP costs and that they share rebates and discounts insurers directly with patients.

Currently, plans and their contracted pharmacy benefit managers (PBMs) also employ complex benefit designs, restrictive tier management, aggressive utilization management, "lasering" (also known as specialty carve outs or discriminatory coverage exclusions) and other tactics that restrict patient access to medications. Through lasering, for example, health plans eliminate coverage for specific specialty drugs and instead require patients to undergo additional inappropriate processes through an alternative funding vendor after their claim is denied. Patients are inappropriately steered to charitable organizations or manufacturer patient assistance programs, which are meant for patients that have no insurance altogether.

Discriminatory coverage exclusions disproportionately target patients with complex and rare conditions and put them at risk by delaying their therapy and interfering with their providers'

[4] Kaiser Family Foundation Employer Health Benefits 2023 Annual Survey.

clinical judgements.[5] Plans also employ accumulator adjustment programs to prevent co-pay assistance from counting towards patients' deductible or out-of-pocket maximums, resulting in higher cost for patients, especially patients with chronic conditions who rely on expensive medications. The Centers for Medicare & Medicaid Services (CMS) itself has expressed concerns regarding this tactic, noting that when "the value of a manufacturer-sponsored assistance was not applied to the patient's deductible…the patient may be forced to stop taking the drug, switch to an alternative offered by the plan, or pay the full bill for the non-formulary drug, none of which are patient-friendly, especially for those patients with rare and life-threatening conditions."[6] BIO urges legislators to protect patients by banning these plan and PBM tactics that impede patient access to treatments patients need.

Over the past decade, there has been a rapid expansion in the number of prescription medications excluded from formularies. In 2022, 1,156 unique prescription medicines were excluded from standard formularies, which represented an increase of a whopping 961 percent since 2014.[7] Agencies are also beginning to acknowledge the pressing need for plan and PBM reform. In its December 2023 Letter to Plans and Pharmacy Benefit Managers, CMS expressed concerns regarding the inappropriate use of utilization management tools, noting that "providers, especially those in rural areas, report that these practices have become increasingly unsustainable and burdensome" in that they "impede access to needed care for people and delay essential treatments, as well as take clinician time away from direct care."[8] Formulary restrictions and utilization management tools are particularly detrimental for patients with conditions treated by CGTs, whereby any delay in treatment may be life-threatening. It is critical that legislators work to advance vital patient protections that will prevent plans and PBMs from implementing harmful coverage restrictions.

Legislators should act to reform appropriate patient safeguards against such plan and PBM tactics. One such safeguard that can be bolstered is the process of external reviews, which allow patients to appeal a coverage decision when the treating physician determines medical necessity of a treatment. While external reviews are required to include a physician "who manages the condition", often times a rare disease is a subspecialty of a specialty. For example, a neurologist treating epilepsy and sleep disorders does not have the same training or clinical expertise as a neuromuscular physician who treats ALS, Duchenne Muscular Dystrophy and Spinal Muscular Atrophy (SMA). Therefore, a neurologist is not appropriate to be the external reviewer on a case for an appeal reviewing a treatment for a neuromuscular disease. This same analogy could be applied to other rare diseases. Accordingly, legislators should ensure that, at a minimum, a rare disease expert with direct experience treating that rare disease should be consulted in external reviews.

III. Fostering a Holistic Approach to Value

[5] Industry Experts Question Alternative Funding Companies That Carve Out Some Specialty Drugs, 'Abuse Charities,' AISHealth, Sept 2022.
[6] Final Rule: Medicaid Program; Establishing Minimum Standards in Medicaid State Drug Utilization Review (DUR) and Supporting Value-Based Purchasing (VBP) for Drugs Covered in Medicaid, Revising Medicaid Drug Rebate and Third Party Liability (TPL) Requirements (CMS-2482-F), Department of Health and Human Services, Centers for Medicare & Medicaid Services.
[7] Xcenda. Skyrocketing Growth in PBM Formulary Exclusions Continues to Raise Concerns About Patient Access. May 2022.
[8] CMS Letter to Plans and Pharmacy Benefit Managers. CMS Fact Sheet. Dec 2023.

The following section is intended to address the question "What is the Future of Access for These Therapies" specifically regarding questions 39, 41-44, 48, and 51. For answers to the remaining questions in these sections, see our responses in II, IV and V.

To provide sustainable access to novel, innovative, or transformative therapies, it will be essential to pay for them based on an holistic consideration of their value to patients, families, and society as a whole.
Transformative therapies, including CGTs, can address very serious diseases with high unmet medical need. They can also serve small patient populations, including rare and orphan diseases, and provide a substantial, durable health benefit. As new innovations in treatment provide new opportunities for patients with high unmet medical needs, existing outdated methods of reimbursement for such treatments have presented challenges for patients, providers, payers, and manufacturers. The future of transformative therapies relies on effective coverage and reimbursement policies that facilitate timely and appropriate access of treatments for each patient's unique condition.

Many transformative therapies are approved as treatments targeting serious, unmet medical needs among highly targeted and often small groups of patients. Patient populations may range from a few dozen individuals for rare diseases such as junctional epidermolysis bullosa (JEB)[9] to a hundred thousand patients such as SCD, depending on the therapy. For these patients, transformative therapies have the potential to offer tremendous quality of life gains, and in some instances, outright cures. The unprecedented benefits to patients include improved overall physical and psychological well-being, improved functional abilities, the ability to return to productive and fulfilling lives, the eliminated or reduced need for chronic therapy, the relieving of caregiver burden, and other clinical and secondary impacts.[10] The value of these transformative therapies span a lifetime and result in cost offsets such as reducing healthcare utilization and productivity gains. For instance, the average direct costs of hemophilia treatment for a patient can be up to $500,000 per year[11] or $5 million in 10 years. Meanwhile, a study in the American Journal of Managed Care found that a gene therapy treatment for hemophilia A would have an estimated one-time cost of $2.5 million. Accordingly, cost-savings would be seen after a mere 4-5 years, not including savings in non-medical costs associated with early retirement, caregivers, underemployment, and other hemophilia-associated costs.[12] [13]

The significant breakthroughs in medical technology could not exist without the intensive research and development (R&D) needed to bring forth treatments. In recent years, innovative transformative therapies, including CGTs, that are under development have entered the market in substantial numbers, highlighting new opportunities to build upon research and spur innovation. However, these exciting advancements do not arise without challenges. The expected cost to bring a new drug to market is estimated to range from less than $1 billion to more than $2 billion.[14] This estimate factors in the high-risk vs. high-fail rate of clinical trials,

[9] De Luca, Michele and Cossu, Giulio. Cost and Availability of Novel Cell and Gene Therapies. EMBO Reports Vol 24 (2). January 2023.
[10] Ali, Faraz et al. Curative Regenerative Medicines: Preparing Health Care Systems for the Coming Wave. In Vivo. November 2016.
[11] The High Price of Hemophilia. ASH Clinical News. February 2020.
[12] McNulty, Rose. Cost-Savings Model Estimates Reduced Economic Burden with Gene Therapy for Hemophilia A. AJMC. November 2022.
[13] Other studies show similar long-term cost savings. See "A Transformative Therapy Value Model for Rare Blood Diseases". January 2020.
[14] Congressional Budget Office. Research and Development in the Pharmaceutical Industry. April 2021.

with a success rate of only 7.9%.[15] This risk is compounded by the lengthy development process which often takes a decade or more.[16] As such, the cost of treatments reflects a balance between ensuring high value to the patient and overall health care system and manufacturers' R&D costs. Further, many companies engaging in the R&D for these innovative therapies are small biopharmaceutical companies that are not currently turning a profit and must rely on outside investors to maintain their strong pipeline. Policies that jeopardize the interests of the significant investors necessary to continue these R&D programs, such as the proposal to overturn the established definition of "covered outpatient drug" in the recent Medicaid proposed rule, pose a grave risk to the current robust pipeline of transformative medicines in development.

Evaluating the long-term value of transformative therapies is critical to ensure comprehensive and equitable payment arrangements. Instead of adopting a narrow line-item cost containment approach, payment systems should consider the long-term savings for both individual patients, their families, and the broad healthcare system. Often, payment arrangements do not consider the societal burden of living with a rare disease, including but not limited to missed school and work for the patient and caregivers.[17] Further, patients needing access to CGTs, such as CAR-T treatments, must travel to certified treatment centers, which imposes additional travel and lodging costs that are often not covered by commercial insurers and lack coverage under the Medicare and Medicaid programs. It is critical that payers provide timely approval of these requests; for instance, State Medicaid agencies could develop an expedited process to enroll providers in situations when a patient needs to travel to another state for treatment and timely reimburse the out-of-state provider. In addition, a federal safe harbor could be established to provide more certainty for manufacturers to offer travel support programs so patients who need financial support can travel to a treatment site to receive gene therapy.

Accordingly, payment systems must encompass the value of transformative therapies, associated costs with administering therapies, and their wide range of medical and non-medical benefits, including enhanced health outcomes, reduced mortality rates, reductions in the severity of medical conditions leading to increased work productivity, fewer hospital admissions and emergency department use, and a myriad of other cost reductions associated with patient care. When accounting for value, it is apparent that the price of transformative therapies is much less than the lifetime medical and nonmedical costs of living with rare diseases. For example, it is estimated that gene therapies to cure SCD will cost around $1 million[18], while the lifetime medical costs for patients living with SCD averages around $1.7 million[19] (inpatient estimates range from $11,978-$59,851 annually[20]) and non-medical costs averages over $4 million ($63,436 annually[21]).

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[15] Kim, Eungdo, et al. Factors Affecting Success of New Drug Clinical Trials. Ther Innov Regul Sci. 57(4):737-750. May 2023.
[16] Congressional Budget Office (2021). Op. cit.
[17] Kolata, Gina. Sickle Cell Cure Brings Mix of Anxiety and Hope. The New York Times. January 2023.
[18] DeMartino, Patrick et al. A Budget Impact Analysis of Gene Therapy for Sickle Cell Disease: The Medicaid Perspective. March 2021. Jama Network.
[19] Johnson, Kate M, et al. Lifetime Medical Costs Attributable to Sickle Cell Disease Among Nonelderly Individuals with Commercial Insurance. Blood Advances: ASH Publications. January 2023.
[20] Baldwin Z, et al. Medical and Non-medical Costs of Sickle Cell Disease and Treatments from a US Perspective: A Systematic Review and Landscape Analysis. Pharmacoecon Open. July 2022;6(4):469-481.
[21] Baldwin (2022). Ibid.

As new scientific, technological, and medical advances spur the development of cell, gene, and other transformative therapies, policymakers and the media have inaccurately conflated the coverage of new transformative therapies with financial hardship. Speculations regarding the supposed significant cost of transformative therapies are often based on a misguided understanding of the pharmaceutical supply chain and a conflating of undiscounted list prices with net prices. Indeed, cancer treatment is a main focus of current and pipeline cell and gene therapy products. However, the American Cancer Society estimates the total cost of cancer care with today's treatments at $185,759 per person.[22] As research continues, policymakers and the health community should realize that while innovative treatments will have costs, they will also offset costs of older, less effective treatments.

Despite media attention on the price of drugs, transformative therapies have not led to a significant rise in overall drug spending. Over the 10-year period ending 2021, net per capita spending on medicines remained effectively flat, increasing just 0.5% on average, per year.[23] In the past six years, net prices for brand-name drugs have declined; in 2023 alone after adjusting for inflation, net prices decreased by more than 7%.[24] Rhetoric around the high cost of transformative therapies is not only misguided, but also harmful, as it may dissuade low-income patients from attempting treatment even though those patients may qualify for patient assistance programs. Moreover, while new products are being launched and entering the market, others are exiting the health care system. The loss of exclusivity and emergence of generic competition results in substantial savings, exceeding the net spending of newly launched brand medicines during the same period. This built in cost containment creates headroom for future treatments and cures.

IV. Facilitating Market Based Solutions to Advance Patient Access

The following section is intended to address the question "What is the Future of Access for These Therapies" and "How Should Federal and State Governments Promote Access to New Models" specifically regarding questions 39-44, 51, 53, 55-57. For answers to the remaining questions in these sections, see our responses in II, III, and V.

BIO is committed to supporting policies that foster a robust and competitive biopharmaceutical market and facilitate innovation so patients can readily access transformative therapies in a timely and safe manner. As new innovations in treatment arise, it is critical that coverage policies are informed by patient experiences and outcomes. Incorporating patient perspectives and measurable outcomes allows policymakers to assess the real-world benefits and potential risks of treatments and effectively serve patients with unmet medical needs.

To that end, the FDA is the appropriate federal agency to lead on the review and approval of therapies and their entry into the market as it relates to the safety and efficacy of patient outcomes. The FDA is well-positioned in its rigorous assessment of clinical trial data to determine whether a particular treatment meets the necessary standards for safety and effectiveness. In approving a therapy and determining the indication statement, FDA relies on totality of data, scientific expertise, and risk-benefit profile. Therefore, the population included in the FDA-approved indication is the population that should be the foundation of a

[22] City of Hope, "Cancer Treatment Costs: How to Manage What You'll Pay," July 18, 2023. https://www.cancercenter.com/community/blog/2023/07/managing-cancer-treatment-cost

[23] IQVIA. "Use of Medicines in the U.S.: Spending and Usage Trends and Outlook to 2025." Published May 2021.

[24] Fein, Adam J. "Tales of the Unsurprised: U.S. Brand-Name Drug Prices Fell for an Unprecedented Sixth Consecutive Year (And Will Fall Further in 2024)." Drug Channels. January 2024.

payer's coverage policy and drive authorization. When an age limit or specific disease milestone is included in the FDA-approved indication statement, access to that treatment before that milestone is reached is imperative so a patient does not miss an opportunity for treatment where for many suffering from rare diseases can further irreversibly deteriorate.

BIO cautions against any decision-making by legislators or other agencies that may undermine the FDA's authority to make approval decisions based on safety and efficacy of treatments. For instance, inappropriately applying clinical trial criteria as the coverage criteria and basis for prior authorization undermines FDA's scientific authority. Legislators and other agencies should uphold the FDA's judgement on approval decisions and work within the jurisdictions of their respective expertise. Many rare diseases are complex with very small heterogeneous patient populations. Clinical trials typically will control for this heterogeneity using a homogeneous patient population. Therefore, payers should provide coverage of treatments to the FDA-approved indication statement, without delay, and not apply arbitrary limitations or restrictions.

Granting patients access to cell, gene, and other transformative therapies is dependent on payment systems that reflect the true value of the therapy and related ancillary services. Legacy laws and rules that support traditional payment systems often inhibit innovation and create barriers to the adoption of transformative therapies. While manufacturers have proactively attempted to address the high upfront cost and long-term clinical uncertainties of transformative therapies through innovative payment models, existing policies limit opportunities to engage in flexible payment arrangements. For instance, Medicaid Best Price (MBP) provisions and Average Manufacturer Price (AMP) reporting requirements operate on the legacy construct of volume rather than value. This has had the unintended consequence of inhibiting the adoption of value-based payment arrangements (VBPs) and other innovative payment models across market segments, such as pay-over-time or pay-for-performance arrangements. Medicaid Best Price also has implications for payment arrangements with other payers including private insurers.

Further, the recently proposed Medicaid "stacking" provision[25], if finalized, would also impose hurdles on payment innovation by disincentivizing innovative payment models, particularly voluntary value-based payments and outcomes-based arrangements for transformative therapies, thus reducing reimbursement for hospitals and negatively impacting access for patients. Rather than create new barriers to the adoption of innovative payment arrangements, it is critical that legislators and agencies reform legacy laws and rules that inhibit payment innovations. For example, reforms to the Medicaid Drug Rebate Program in 2020[26] created a more flexible and adaptive framework for the reporting of best price that allows for value-based payment arrangements.

The Medicaid VBPs for Patients (MVP) Act (H.R. 2666) would codify these regulations in statute. In addition to providing for necessary safe-harbors under the Anti-kickback Statute (AKS), the legislation would ensure that best price reported under value-based arrangements through the multiple best price requirements would not be calculated as part of the Average Sales Price (ASP) under Medicaid. These are essential changes that will facilitate the

[25] Medicaid Program; Misclassification of Drugs, Program Administration and Program Integrity Updates Under the Medicaid Drug Rebate Program. CMS Proposed Rule. May 2023.
[26] Medicaid Program; Establishing Minimum Standards in Medicaid State Drug Utilization Review (DUR) and Supporting Value-Based Purchasing (VBP) for Drugs Covered in Medicaid, Revising Medicaid Drug Rebate and Third Party Liability (TPL) Requirements. CMS Final Rule. December 2020.

development of new innovative payment models in Medicaid. We urge legislators and agencies to implement needed changes to legacy laws and rules to foster innovative payment arrangements.

New payment innovations are essential to adapt to the evolving landscape of transformative therapies. As meaningfully noted by Duke University, transformative therapies such as cell and gene therapy "represent a paradigm shift away from reactive, episodic treatment of disease towards a proactive approach to sustaining long-term health."[27] In this new paradigm, traditional payment approaches revolved around volume-based, fee-for-service (FFS) reimbursement have proven to be inadequate in accounting for the long-term health and financial benefits of transformative therapies. Traditional payment approaches have inherent challenges and budgetary pressures that impose undue burdens on patients in the form of high cost-sharing and coverage restrictions. Instead, new payment innovations that can create a patient-focused, adaptive reimbursement framework to support and incentivize long-term health improvements brought by transformative therapies. It is critical that legislators and agencies foster, rather than inhibit, market solutions that drive these payment innovations.

The biopharmaceutical market is well positioned to test various payment innovations based on the unique aspects of each treatment. Payment innovations that may benefit certain transformative therapies include value-based arrangements, indication-based pricing, outcomes-based arrangements, money-back guarantees, product warranties, and other arrangements that account for long-term value, provide efficacy assurances, and align provider incentives with patient health. However, it is critical to note that there is no one-size-fits-all payment solution. The applicability of each payment innovation varies based on the unique characteristics of each therapy. CGTs, for instance, are developed by utilizing a patient's specific cells; in some cases the treatments are unique and are manufactured for a specific recipient. Some rare diseases are fast-progressing while others are slow-progressing, further impacting the duration and administration of treatment. This underscores the necessity of tailoring payment innovations to the clinical and operational factors and other variables associated with each therapy.

Further, BIO is committed to working with policymakers and the Committee to ensure any potential reforms have minimal impact on the programs that already pose significant market distortions in the healthcare innovative market ecosystem, such as the 340B Program, which has already grown far beyond Congressional intent. For example, the previously mentioned "stacking rule" as proposed, would tremendously exacerbate the 340B Program and provide disincentives for the development of value-based purchasing arrangements under the multiple-best price reporting rule or even offering of them in the private commercial market since they are not exempt from the stacking requirement.

V. Reforming Legacy Payment Systems

The following sections are intended to address the question "What is the Future of Access for These Therapies" "How Should Federal and State Governments Promote Access to New Models" and "How Should Lawmakers Seek to Evaluate and Accomplish these Policy Goals," specifically regarding questions 39, 43-44, 47-48, 51, 53-57, and 59. For answers to the remaining questions in these sections, see our responses in II, III, and IV.

[27] Breakthroughs and Barriers Advancing Value-Based Payment for Transformative Therapies. Duke University Margolis Center for Health Policy. May 2019.

A. Medicare Coverage Reforms

Twentieth century legacy Medicare reimbursement structures have struggled to adapt to the unique advancements of new medical treatments and technologies. While innovations in medicine bring about the development of transformative therapies to address serious unmet medical needs, the Medicare program has continued to lag behind medical advancements and has been unable to accommodate and fully sustain the reimbursement levels needed to scale the adoption of transformative therapies. Regrettably, the Medicare program has often skewed towards rigid and standardized approaches, as new medical treatments are assigned into existing payment structures. This reactive approach has led to delays and limitations in providing Medicare beneficiaries with timely access to cutting-edge therapies. Given the potential for transformative therapies to deliver substantial clinical benefit and care improvements for overall patient health, it is imperative that the Medicare program evolve with a broader view of the evolving landscape and appropriate payments to set patients and healthcare providers up for long-term success. As an important step, payment and coverage innovations introduced by the Center for Medicare and Medicaid Innovation (CMMI) have the potential to bring about these necessary reforms, provided that they:

- Prioritize a patient-centered approach;
- Recruit patients, providers, suppliers, etc. on a voluntary basis; and
- Commence on a bona fide scientific research basis and are not utilized politically as a back door to impose restrictive pricing or coverage policies.

By adhering to these principles, Medicare payment innovations can lay the groundwork to support sustainable and predictable provider reimbursement and assure timely patient access to transformative therapies. BIO looks forward to working with lawmakers to apply lessons learned from CMMI models and legacy Medicare payment systems to assure that future transformative therapies can be quickly and efficiently incorporated into a new flexible framework that fosters innovation within the Medicare program.

Legacy Medicare payment systems such as the Inpatient Prospective Payment System (IPPS) were introduced in the 1980s to establish predetermined payment rates for specific services.[28] Diagnosis Related Groups (DRGs) and later Medicare Severity Diagnosis Related Groups (MS-DRG) were created within the IPPS to standardize the reimbursement process for Medicare by grouping similar diagnoses and medical procedures. These payment systems were designed to optimize high-volume, low-variation services to encourage efficiency and provide financial predictability for providers.

Since the inception of the prospective payment systems, however, medical treatments and technologies have advanced considerably. New transformative therapies, as mentioned previously, have greater clinical and cost variability and meet the needs of smaller patient populations—indeed, as discussed above, CGTs are often unique to each patient. While legacy payment systems are still well suited for medical services that have a large patient population and low cost variation per patient, they have been unable to sustainably fund new medical innovation and transformative therapies. As medical innovation continues to progress, it is critical that payment systems adapt to meet the evolving needs of the healthcare landscape. Lawmakers must make necessary reforms to ensure that payment systems appropriately

[28] Chilingerian, Jon A. "Origins of DRGs in the United States: A Technical, Political, and Cultural Story." January 2008.

reflect advances in technology and provide adequate coverage across sites of care for unique patient populations needing access to transformative therapies.

As lawmakers assess alternative reimbursement approaches, BIO also remains concerned about ongoing efforts to expand bundling arrangements within Medicare fee-for-service (FFS) payment systems to cover transformative therapies. BIO strongly cautions against bundling transformative therapies with existing treatment options due to risks of insufficient provider payment, which will subsequently create barriers to patient access. Fixed payment bundles are derived from the most applicable episode of care, while transformative therapies diverge significantly from existing treatment options by addressing distinct unmet medical needs and requiring higher total costs. Therefore, bundling transformative therapies with existing treatment options significantly undervalues transformative therapies and creates a financial disincentive for providers to embrace or prioritize innovative treatments. Facing the risk of insufficient payment, providers may instead prioritize more established treatments within the bundle, even when the established treatment may not be as effective or clinically beneficial for the patient. It is critical that lawmakers address the perverse incentives of bundled payment arrangements within the Medicare FFS setting to ensure that transformative therapies are sufficiently reimbursed.

Still, BIO recognizes and appreciates CMS' current and ongoing efforts to develop payment solutions to address medical innovation through instruments such as the new technology add-on payments (NTAPs). NTAP designation enables additional payments for new technologies to reflect added costs and mitigate financial losses for providers. To receive an NTAP designation, the new technology must be inadequately reimbursed under the existing system and demonstrate significant clinical improvements compared to currently available treatments.[29] While NTAPs represent a positive step in granting appropriate payments for transformative therapies, there are notable gaps in its application. Only approximately 30% of NTAP applications are approved,[30] and NTAP awards are only granted for limited duration of two or three years.[31] Because of its ad hoc nature, NTAPs are unable to fully provide the long-term financial viability and stability that providers need to sustain patient access to transformative therapies. This issue is further compounded by the difficulty of receiving NTAP approvals in a timely manner after FDA approval. Under the current timeline for NTAP, there is roughly a one-and-a-half-year lag between the initial deadline for an NTAP application and the NTAP's effective date.[32] The limited duration of NTAP payments and lack of timely approvals create barriers to patient access for newly approved therapies. Reforms are needed in order to advance a Medicare reimbursement framework that is timely, sufficient over long durations, and flexibly adapts with the pace of approvals for new transformative therapies.

B. Medicaid Coverage Reforms

State Medicaid programs face unique challenges in the coverage of transformative therapies due to their finite resources that are dictated by annual or biennial budget cycles. The confines of the state's budgetary requirements and limits may not easily absorb the many costs faced

[29] "How a New Technology Add-on Payment (NTAP) Works." Avalere. August 2023.
[30] Truglio, A and Livoti, C. "Analysis of Success Rates for the Center for Medicare and Medicaid's New Technology Add-on Payment Program." Value in Health Vol 21(1). May 2018.
[31] CMS, "New Medical Services and New Technologies" December 14, 2023. https://www.cms.gov/medicare/payment/prospective-payment-systems/acute-inpatient-pps/new-medical-services-and-new-technologies
[32] Avalere (2023). Op. Cit.

by patients with rare or ultra-rare chronic conditions. To address the pressure put on Medicaid programs, states must support innovation in both contracting and coverage that focus on providing value in care, treatment, and services. However, while CMS and states indicate they want innovation in policy and reimbursement, their latest policy actions seem to suggest otherwise.

The Medicaid Drug Rebate Program (MDRP) is pivotal in enabling patients to obtain access to all FDA-approved medicines, especially transformative therapies, at the state level. The Medicaid rebate provisions of the Social Security Act (SSA) represent a carefully balanced compromise made by Congress to ensure the Government has access to the lowest available price for covered outpatient drugs while also ensuring patients have access to all FDA-approved medicines that have a signed Medicaid rebate agreement. Since 1990, this program has allowed manufacturers to enter into agreements with state Medicaid programs to give vulnerable patients access to transformative therapeutics and to help brunt the fiscal impacts on state budgets.

Unfortunately, both federal and state governments have attempted to upend the MDRP's statutory provisions through proposed rules and waivers that threaten patient access to transformative therapies. As previously mentioned, CMS' proposed "stacking" rule also attempts to overturn the established definition of "covered outpatient drug" (COD) which will put state payment arrangements of transformative therapies at risk, specifically for those states paying for direct reimbursement of transformative therapies. As we noted in our comments to CMS, BIO has significant concerns regarding the impact this proposal could have on hospital reimbursement and patient access to innovative CGTs (CGTs) that are administered inpatient.

In the majority of states, hospitals received a bundled payment that is intended to cover the cost of the therapy as well as any ancillary services associated with providing care to the patient during the inpatient stay. Since payment to hospitals administering CGTs is often insufficient under the DRG system, states are beginning to pay hospitals separately, outside of the bundled payment for inpatient services, for their acquisition cost of CGTs. In this scenario – when the CGT is "carved out" of the bundle and paid for separately –CMS's longstanding traditional interpretation of COD requires that manufacturers pay a rebate when the drug is administered to a Medicaid patient. Under this arrangement, hospitals are paid adequately, states have the benefit of federal rebates on the utilization, and Medicaid patients in turn benefit from increased access. This also has the effect of opening up the opportunity for VBAs.

However, CMS' proposed change would deem any drug that is administered inpatient and paid for as part of a bundled payment as a "covered outpatient drug" simply by the inclusion of that drug on a claim form. As a result, states would presumably be authorized to seek Medicaid rebates from manufacturers on such drugs by simply identifying the product on the claim form, and without directly reimbursing providers for the cost of the drug. Thus, BIO strongly disagrees with CMS' new interpretation that such action would qualify as "direct reimbursement" and classify drugs paid for as part of a bundled arrangement as "covered outpatient drugs." In so doing, CMS undermines payment arrangements that serve all stakeholders and creates an opportunity for states to merely add a line-item to an otherwise bundled payment, resulting in significant financial losses for hospitals or, may have the effect of limiting access to CGTs because hospitals will not want to provide such therapies.

In this same rule, CMS also proposes to impose new reporting obligations through a drug price verification survey. CMS indicates that it intends to focus mostly on CGTs with respect to required participation in the survey to justify price points. This specific targeting of CGTs

threatens to harm development of these innovative therapies. Many of the companies that invest in CGT research are small biotechnology companies, many of whom are not even making a profit and rely heavily on investment from outside investors. When regulators and legislators target these therapies before they have even come to market, these investors may sit on the sidelines, resulting in lost therapies and continued unmet patient needs.

Transformative therapies, including CGTs, are often approved through the FDA's accelerated approval process based on the clinical benefits and unmet medical needs that drug addresses. Accelerated approval is reserved for drugs that address serious or life-threatening diseases with limited or no treatment options, and, importantly, are proven safe and effective by the same rigorous evidentiary standards used by the FDA to approve all other therapies.[33] Therapies approved through this accelerated pathway are subject to a demanding standard of review to demonstrate "substantial evidence" of effectiveness.[34] After approval, these therapies are subject to post-approval confirmatory trials or studies to verify and describe the anticipated clinical benefit; failure to verify the benefit will result in withdrawal of FDA approval.[35] Studies have found that certain drugs reviewed under the accelerated approval processes have offered greater medical gains than drugs reviewed through the FDA's traditional, lengthier process.[36]

Despite the safe and efficacious approval of transformative therapies, various states have targeted those therapies approved through the accelerated pathway by delaying or limiting access to those therapies or denying coverage altogether. For instance, some states have delayed access to therapies approved through accelerated approval by not covering them until such therapies undergo review by a Pharmacy & Therapeutics (P&T) committee, which is then subject to a gruelingly slow review process. Some state Medicaid programs have also delayed coverage for physician-administered drugs, including CGTs targeting rare diseases, by claiming that there is not a unique product specific HCPCS J-code (despite the availability of a miscellaneous J-code which is common coding billing practice) or that the drug's NDC does not appear in the CMS rebate file. This delayed access is akin to denied access for patients that have no other treatment options, particularly when their disease causes the patients to irreversibly lose function every day. It is critical that state Medicaid Agencies not erect these unnecessary administrative barriers and facilitate timely patient access to treatment.

Further, some states have also attempted to use the Section 1115 Demonstration Waivers to bypass the MDRP coverage requirements to implement closed formularies that limit coverage to a single drug in a therapeutic class, with the intent of excluding therapies approved through the accelerated approval pathway. Closed formularies directly violate the statutory requirements of the Medicaid Drug Rebate Program. According to statute, if a drug approved under accelerated approval meets the definition of "covered outpatient drug" as found in Section 1927 of the Social Security Act and the Manufacturer has a signed Medicaid National Rebate agreement, the drug must be covered by state Medicaid programs.[37] In addition to being unlawful, closed formularies severely jeopardize the quality of care of the most vulnerable and sickest patients, particularly those with rare and ultra rare diseases.

[33] 21 U.S.C. §356(e)(2).
[34] 21 U.S.C § 355(d)(5).
[35] FDA. Guidance for Industry: Expedited Programs for Serious Conditions – Drugs and Biologics. May 2014.
[36] Chambers, et al., *Drugs Cleared Through the FDA's Expedited Review Offer Greater Gains Than Drugs Approved by Conventional Process*, Health Affairs Vol. 36, No. 8, 2017.
[37] CMS State Release No. 185, June 27, 2018.

BIO has grave concerns that states trying to implement closed formularies will deprive patients of medically necessary transformative therapies, resulting in devastating effects on morbidity or mortality of those patients. These states appear to suggest that state P&T committees can determine the safety and clinical efficacy of a drug in a manner superior to that of the FDA, albeit that the FDA is the worldwide gold standard in the review and efficacy of drugs. These policies were firmly rejected by CMS, indicating that a state cannot simply opt out of §1927 and not provide access to "covered outpatient drugs" for which a manufacturer has a signed National Rebate Agreement.[38] However, given CMS' recent proposals to reinterpret the MDRP statute with the proposed "stacking" rule after more than 30 years of precedent, we have serious concerns that the agency could at any time attempt to reinterpret the ability to waive patient coverage requirements of §1927. Such unnecessary delays in accessing critical transformative therapies can be profoundly detrimental for patients whose need for such therapies is time sensitive. BIO remains deeply concerned that delaying treatments for the most vulnerable Medicaid patients puts these patients at risk of significant adverse health events.

BIO believes that states need to look forward to innovative policy solutions that emphasize the value of particular transformative therapies, such as voluntary agreements between biopharmaceutical companies, states, managed care organizations (MCOs), and other stakeholders as necessary. For instance, some states may benefit from risk mitigation strategies that allow Medicaid MCOs and the state to share in costs or savings beyond a certain threshold to protect against excessive losses. Innovative, voluntary negotiations provide important opportunities for states to craft agreements that are tailored to the specific unmet needs of patient demographics in the state.

Since specific arrangements that are suitable in one state may not be suitable for another, it is critical that negotiations remain flexible to allow states the opportunity to address unique challenges of their populations and allocate resources effectively, which will subsequently help improve patient access to necessary transformative therapies. For example, an outcomes-based arrangement may work well for certain patient populations and not others. In some other situations, a negotiated pay-over-time arrangement may be more suitable. But states and manufacturers must have the flexibility to meet the needs of the patient population in the most tailored way possible.

C. Commercial Coverage Reforms

In private health insurance plans, there are no federal standards for the coverage of specific drugs, although individual and small group market issuers are subject to minimum standards of coverage for essential health benefits (EHBs).[39] Given the legal flexibility of large group health issuers to formulate benefit packages, some issuers may exclude coverage of transformative therapies or engage in practices such as lasering or excessive utilization management tactics, as mentioned in previous sections. Self-insured plans have also been known to laser out specific drugs or categories of drugs, subsequently barring those drugs from external review in certain cases. This creates substantial inequities where patients are left both untreated and without due process. Nevertheless, it is important to note that private sector plans closely observe and adopt coverage and reimbursement signals from public programs. Often, the reimbursement frameworks and guardrails set by public programs are

[38] CMS letter to Asst. Secretary Tsai, MassHealth, June 27, 2018.
[39] Ofengeym, Yelena, et al. "Accessing Cell and Gene Therapies: Insights on Coverage, Reimbursement, and Emerging Models." Manatt Health. July 2023.

used by private issuers for their own practices. The significant influence of public programs on the broader population covered by private plans underscores the importance of ensuring accuracy and effectiveness of reimbursement pathways set by public programs. Removing access barriers to transformative therapies in public programs can set influential precedents that impact all insured populations.

Prescription drugs, including transformative therapies, are just one part of a patient's care, regardless of whether the disease treated is chronic, rare, life-threatening, or part of a multi-disease diagnosis. In addition to the cost of transformative therapies, other costs can include specialists, travel costs, specialized diagnostic testing and monitoring, hospitalization while being treated, and more. Further, when specialized treatments become standards of treatment, payers tend to pass the costs of these treatments to the consumer in the form increased premiums and other out of pocket costs.

To that end, reinsurance and other risk mitigation programs have shown great promise as suitable tools in lowering overall premiums for certain commercially insured patients, thus bringing down total out-of-pocket costs for the chronically ill. Further, these programs have made insurance more attainable for some who might not have otherwise been able to afford it. Reinsurance can help address key challenges in the marketplace, such as affordability of premiums and excess volatility/uncertainty.[40] Most people that purchase their insurance through the exchanges receive some form of subsidies from the federal government to be able to afford coverage. However, for many that do not receive federal subsidies the ACA still does not sufficiently address the premium affordability question, which continues to be a problem.[41]

Reinsurance allows commercial plans to transfer a portion of the financial risk to reinsurers, thus mitigating fiscal risks for issuers and allowing issuers to avoid passing the full cost burden to policyholders in the form of significantly higher premiums. Several states have created reinsurance pools to stabilize insurance premiums under the exchanges created by the Affordable Care Act (ACA).[42] Twelve states[43] have attempted a few different models of reinsurance in the individual markets with varying levels of success through the use of a State Innovation Waiver granted by CMS. While not all states have seen an increase in enrollment through the health insurance exchanges, all states that have used a State Innovation Waiver for reinsurance programs have successfully reduced premiums in their individual health insurance markets, thus, lowering out-of-pocket costs for consumers.[44]
Considering the positive impacts demonstrated in states that have utilized reinsurance programs, the broader adoption of reinsurance and other risk mitigation programs across states would contribute to greater affordability, stability, and accessibility of transformative therapies in the commercial market. BIO welcomes the opportunity to discuss more detailed recommendations with lawmakers so more states can implement reinsurance programs and explore other risk mitigation opportunities.

VII. Conclusion

[40] "Next Generation Therapies in Massachusetts: New Solutions for Coverage and Payment," Network for Excellence in Health Innovation (NEHI), 2019.
[41] "Benefits and Limitations of State-Run Individual Market Reinsurance, Issue Briefs, The Commonwealth Fund. November 11, 2020. https://www.commonwealthfund.org/publications/issue-briefs/2020/oct/benefits-limitations-state-run-individual-market-reinsurance Accessed: January 5, 2024
[42] "Next Generation Therapies in Massachusetts: New Solutions for Coverage and Payment," Network for Excellence in Health Innovation (NEHI), 2019.
[43] "Benefits and Limitations of State-Run Individual Market Reinsurance." Issue Briefs, The Commonwealth Fund. November 11, 2020
[44] Commonwealth Fund (2020). Ibid.

The promising pace of innovation in clinical science brings hope to patients facing unmet medical needs. However, it is crucial to acknowledge that the landscape of innovation evolves more rapidly than regulatory frameworks. To harness the full potential of innovation and ensure patients can gain access to cutting-edge therapies, lawmakers must collaborate with stakeholders to enhance patient access to transformative therapies rather than control access to these therapies. As we have discussed in this RFI response, harmful policies and tactics that negatively impact patient access to transformative therapies include the Medicaid Best Price "Stacking" Rule, the expansion of the 340B Program, bundled payment arrangements that undervalue transformative therapies, plan and PBM tactics including restrictive tier management, aggressive utilization management, and "lasering," and state practices causing delays or limitations in accessing transformative therapies.

To counter these harmful policies and tactics, legislators should employ the following key principles : any policy action should prioritize the health and wellbeing of patients; a variety of payment and coverage innovations—not just one--will likely be necessary; value assessments regarding transformative therapies must include societal value and long-term savings; and lawmakers should remove access barriers on patients and reform legacy payment systems. To that end, BIO appreciates the opportunity to respond to this RFI and looks forward to continuing to work with lawmakers to ensure patient access to transformative therapies and promote biopharmaceutical innovation.