Advancing Access to Transformative Medicines for Arizonans

A GENE THERAPY PRIMER

Executive Summary

Innovative and transformative gene therapy treatments are working their way through all stages of the FDA pipeline with the potential to cure diseases and the ability to save lives, providing patients hope for the future.

While innovation can come with seemingly large upfront costs, the long-term benefits that emerging gene therapies offer have the potential to save the health care system future costs and, most importantly, provide those suffering from chronic diseases a better quality of life.

- Gene therapy has the potential to cure diseases, giving patients and caregivers a new lease on life, returning people to the workforce, and creating potential long-term savings to the health care system.
- Gene therapy can reduce costs associated with chronic diseases over the patient's lifetime including symptom treatment, medications, interventions, and hospitalizations, reducing utilization and the associated long-term impacts to the healthcare system.
- Improving access to gene therapy can help reduce healthcare disparities for minority populations.
- By 2025, more than 20 new gene therapies are expected to be approved each year. Given their upfront expense, policymakers likely will need to work with stakeholders to innovate payment options to increase patient access to these lifesaving and life-improving therapies.
- To ensure Arizona remains on the forefront of ensuring patient-centered healthcare, policymakers should regularly review new developments and clinical trial results to give patients quicker access to proven treatments.

Arizona's Patient Focused Public Policy

Arizona has a well-deserved and growing reputation for implementing public policies that focus on patient-centered healthcare and ensuring access to cost-effective care. This reputation has been bolstered in recent years as policymakers have passed a series of bipartisan bills to ensure patient access to cutting edge therapies. Recent examples include:

- Changes in the process of adding new conditions to the state's Newborn Screening Program that moved Arizona from being one of the last states to add new tests to being one of the first

- The most expansive telehealth bill in the country to ensure access to treatment in the comfort of the patient's own home

Each of these reforms addressed a specific patient population with the goal of expanding their access to the best care available. But medical research is not static. Each year, new, exciting treatments come onto the market, thus the need to ensure patient access to those treatments is an ongoing policy pursuit. Ongoing reviews of patient access and roadblocks to treatment is necessary by lawmakers to ensure that public policy keeps pace with scientific developments.

While there are many exciting areas of medical research taking place, perhaps the most exciting area currently coming to fruition is gene therapy. Gene therapies are intended to slow or stop disease progression by addressing the underlying cause of disease at the level of a genetic mutation. The promise of gene therapy and the potentially game-changing effects it could create has been around for decades, and advances occurring each year are quickly moving these treatments from scientific theory to reality.

These new treatments are not just a game changer for science, they are a game changer in the lives of real people who suffer from debilitating and potentially lethal conditions. As these new gene therapies move into the real world of medicine, patients who until have had no options for treatment that can cure their underlying condition can truly begin to dream about having a normal life that is no longer controlled by their illness.

More than 20 new gene therapy approvals are expected annually by 2025. To ensure that Arizona patients will be able to benefit from these medical advances as they are approved, all stakeholders, including payors, patients, policymakers, and pharmaceutical companies, must work together - quickly - to advance a flexible policy ecosystem that reflects the potentially transformative shift these therapies represent.

What is Gene Therapy?

Gene therapy is a new approach to treating or curing disease by addressing the root cause of disease at the genetic level. This process can take a variety of forms including replacing a disease-causing gene with a normal copy of that gene, deactivating a disease-causing gene, or introducing a new gene into a patient.

There are thousands of experimental gene therapies working their way through all stages of the FDA pipeline from pre-clinical research to human trials. If approved, each of these innovative and transformative treatments have the potential to cure diseases that up until now, patients have had few if any options for curative treatment and most options available were merely for symptom management.

While gene therapy trials cover a wide variety of conditions, cancer and rare diseases currently have the most trials underway. These therapies could benefit minority populations that are disproportionately affected by a disease, children suffering from a disease that is likely to take their life at a very young age, and patients suffering from diseases like hemophilia that can cost hundreds of thousands of dollars to treat annually. A deeper dive into these three examples will illustrate the life changing potential of new therapies as well as the benefits of ensuring access to treatment.

cell has defective gene

healthy gene is introduced

cell function is restored

Examples of Emerging Gene Therapies and Populations that Stand to Benefit

Sickle Cell Disease

Sickle Cell Disease (SCD) affects approximately 100,000 people in the United States. SCD is most common among the African American population, affecting 1 out of every 365 births. The rate of SCD among the Hispanic American population is also elevated with a rate of 1 out of every 16,300 births.

SCD causes anomalies in the shape and structure of an individual's red blood cells, creating painful blockages in blood vessels and reducing the flow of oxygen and leading to health challenges, including severe pain, anemia, and potentially life-threatening complications. These potentially fatal complications include elevated risk of child mortality, stroke, kidney damage, liver disease, pulmonary hypertension, and sleep apnea.

Treatment for SCD has traditionally been focused on managing symptoms, reducing pain, and addressing complications as they arise. In addition to medications, patients may also be treated with blood transfusions and occasionally with stem cell transplants for young children with the most serious cases. A recent study in the journal of Blood Advances found that for a commercially insured patient with SCD, the estimated lifetime cost to treat the disease with current treatment options is $1.7 million. This figure includes both prescription medication as well as emergency room visits and hospitalizations when symptoms of the disease warrant this level of care.

Duchenne Muscular Dystrophy

Duchenne Muscular Dystrophy (DMD) is a devastating rare disease that affects the muscles of patients, primarily young boys, causing the muscles, including the heart, to weaken and atrophy, resulting in premature death. While the disease affects fewer than 50,000 boys in the United States, for those who suffer from DMD, the median survival age is 22 and the chance of making it to the age of 40 is a mere 13%. Although there have been some advancements in care that have resulted in improved longevity and quality of life, there is currently no cure for DMD and patients eventually lose their ability to walk and lead independent lives.

The annual costs associated with a DMD patient are significant and increase with age as the disease progresses, with an annual economic burden on the United States health care system of more than $1.2 billion. At the family level, costs associated with home and transportation modifications to ensure wheelchair accessibility, equipment and devices, tests and medications, and ongoing supportive care are extensive.

Hemophilia

Hemophilia is a bleeding disorder that prevents a patient's blood from clotting normally. Hemophilia occurs at a much higher rate in men than in women, with Hemophilia A affecting approximately 1 in 5,000 male babies. Individuals with hemophilia experience significant bleeding from a small cut or wound as well as internal bleeding including intracranial bleeding and often develop debilitating joint disease.

Beyond the bleeding abnormalities, hemophilia often creates other challenges for patients. In a study, 94% of children with hemophilia reported that the condition and its complications had, at times, prevented them from attending school or being able to concentrate during class. Adults with hemophilia also suffer challenges as a result of bleeding episodes with 95% of patients reporting that hemophilia had a negative effect on their employment and many reported that complications of hemophilia kept them from working all together.

Putting Costs of New Treatments into Perspective

These new gene therapies, as is true with nearly every new treatment that comes onto the market, are nearly certain to come with a higher price tag than traditional medications. While these costs cannot be ignored, they can and should be put into perspective.

Unlike most medications for chronic disease, gene therapies are potentially one-time treatments that may dramatically reduce health care needs and costs over a patient's lifetime. Therefore, when considering how best to pay for gene therapies, policymakers may need to look beyond the costs incurred in a single year when assessing their value and making decisions about insurance coverage requirements. If a gene therapy can eliminate or significantly reduce the need for other treatments, interventions, and hospitalizations that are part of current disease management strategies, those associated savings should be considered and calculated over a longer time horizon. For example, while there is not yet a gene therapy available for Hemophilia A, there is a gene therapy approved for Hemophilia B. In the United States, the average cost of non-gene therapy treatment for a Hemophilia B patient costs on average between $700,000 and $800,000 per year. The newly available gene therapy has been found to completely eliminate the need for the traditional injections for most patients. Even with a price tag of over $3 million dollars, the treatment can still create cost savings in less than 5 years.

Factors Beyond Costs That Should be Considered in Coverage Decisions

1. **Fostering A Return to Self-Sufficiency and Reducing the Need for Government Assistance**

   For public insurance programs such as Medicaid, the goal should be to provide necessary health services to help all enrollees reach their highest potential, while managing taxpayer costs. A special emphasis should be placed on helping those who are unable to work because of a health condition. It should be a goal to treat that condition as quickly and effectively as possible so the person can join or rejoin the workforce, climb up the economic ladder, and minimize the need for government assistance.

   While most Medicaid beneficiaries work full or part time, the top two reasons beneficiaries do not work are caregiving responsibilities (12% of Medicaid members) and not being able to work because of illness or disability (10%).

   Let's take the example of SCD. As covered earlier, individuals with SCD are high-cost, high-need patients, and that cost is frequently paid by taxpayer funded programs. Nationally, 66% of hospitalizations due to SCD and 58% of emergency room visits related to SCD are paid for by Medicaid. Some of these patients can cost in excess of a quarter million dollars a year for treatment but even for lower-level cases, the average annual Medicaid spending for a member with SCD is in the range of five times higher than an average Medicaid member.

   If a gene therapy is approved that can treat SCD which eliminates the underlying cause of these hospitalizations and emergency room visits, covering that treatment makes financial sense for taxpayers. While costs may be higher in the first year, the on-going costs associated with hospitalizations and ER visits will be dramatically decreased. Moreover, if the patient can be stabilized such that they are able to rejoin the workforce and obtain an income above 133% of the federal poverty level, the taxpayer will see ongoing savings in the Medicaid program as the person moves onto private insurance. The savings for the taxpayers will continue to grow as the individual's self-sufficiency grows and they reduce the need for other assistance programs.

2. Caregiver Impact

When a patient suffers from a debilitating disease, it is not only the patient's ability to work that suffers. Often the primary caregiver, usually a spouse or parent, experiences similar challenges. A recent study reported that in the United States, caregivers lose $522 billion in lost income due to the time they must devote to caring for their loved one.

With hemophilia specifically, 89% of caregivers reported that the demands of caring for a child with hemophilia hurt their ability to work with 31% reporting that they left their job entirely to care for their child. The resulting reduction in family income can push once self-sufficient, middle-class families into poverty and result in more enrollment in public welfare programs. For companies, it can result in more days in which an employee calls out, employees being less productive as worries for their loved one interfere with their job performance, higher utilization of FMLA, and even result in valued employees leaving the workforce altogether.

Coverage of treatments can give not only a patient a new lease on life, but can give the patient's caretaker the same. More than a quarter of DMD caregivers reported reducing their working hours or stopping work completely to care for a relative with DMD. And, on average, caregivers spend more hours per week on caregiving than on a full-time job, averaging more than 50 hours per week. As the patient's condition improves, the caregiver will also be able to return to work. For a company, this improvement at home can result in an employee missing fewer days, higher productivity levels, and higher employee retention. For public programs, the return to work of a caregiver can result in programmatic savings as the individual moves off of government programs

3. Equity and Diversity

Many of the emerging gene therapies are targeted to treat diseases that disproportionately affect minority populations.

As of 2021, 83% of US employers stated that they were implementing diversity, equity, and inclusion (DEI) initiatives. The goal of these DEI programs is to attract and retain top talent and ensure that members of all communities feel welcome in the workplace.

Ensuring coverage of these new and improved treatments for conditions that disproportionately affect minority populations would align with the diversity goals that these companies publicly espouse. Inequity in the ability of all patients to obtain the best health treatments available for themselves or their dependents exacerbates inequity in these communities.

4. Quality of Life for a Patient

Quality of life is an intangible that we simply cannot put a price tag on. We each have only one life to live, and it is an innate human desire to live that life to the fullest extent possible. Patients with serious, chronic diseases face many more challenges and setbacks. Often their only desire is to be free of the disease that limits them. They simply want the ability to live as others do. A young boy with DMD just wants to be able to get out of his wheelchair and play sports with his friends. A child with hemophilia just wants to be able to go to the park without fear that a cut from a fall could put him in the hospital. A woman with SCD just wants to be able to be free of daily pain caused by the disease.

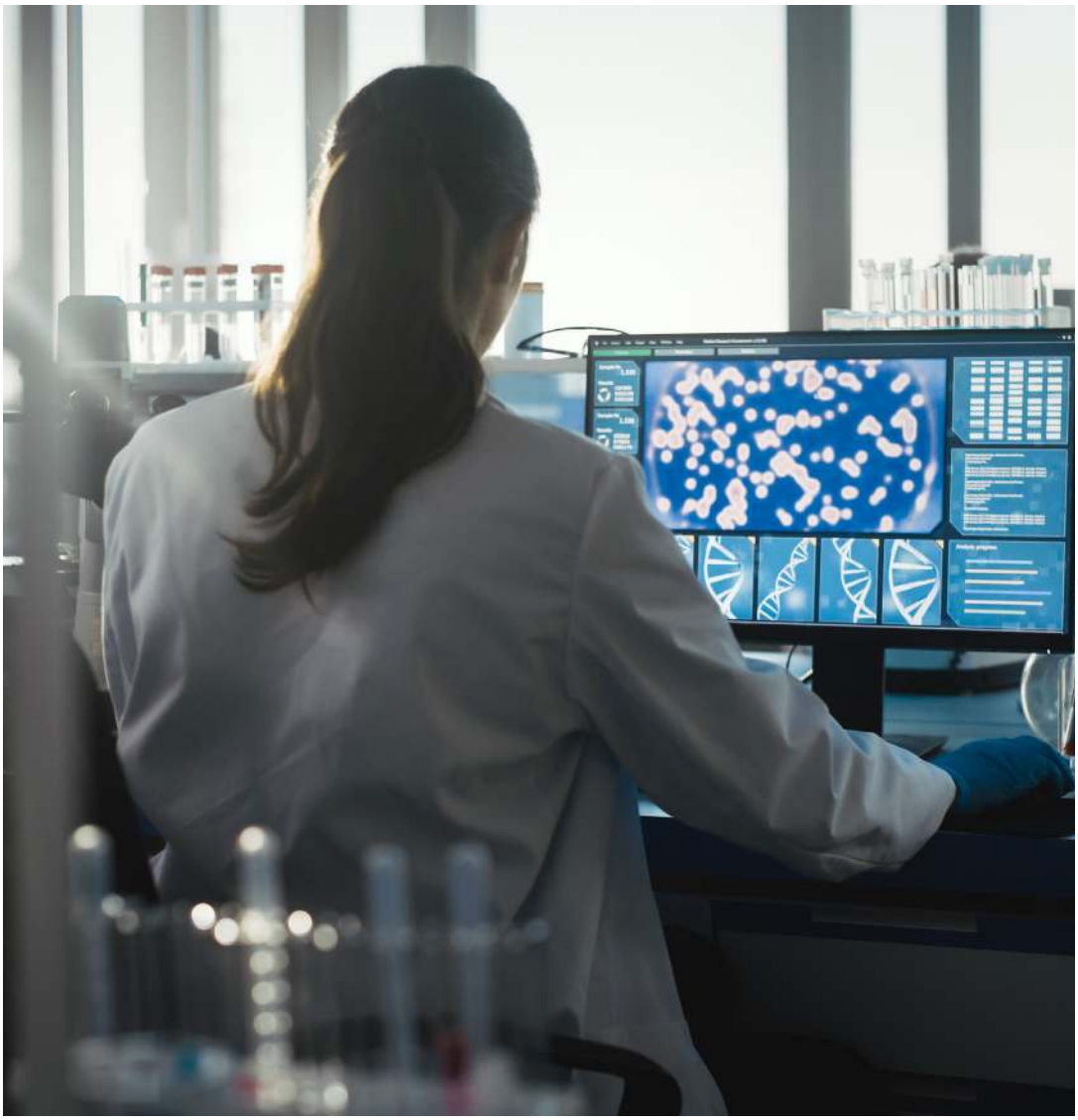
There could be no greater tragedy for these individuals than for a cure to be developed only for access to that cure to be denied. To deny access to such a cure goes against many people's beliefs, ethics, morals, and values.

Since few would disagree with the basic premise that if a cure exists, individuals should be able to access that treatment, the question for policymakers is how best to pay for it.

Innovations in Paying for New Treatments

Gene therapies represent a new treatment paradigm that will require innovative thinking about affordability, payment, and risk for private and public health insurance. With unprecedented treatments achieved after decades of research and billions of dollars in investment to bring the treatments to fruition, many of these treatments are likely to come with a significantly higher price tag. Understandably, patients and their families will want access to these approved treatments but few will be able to afford to pay for them on their own and will ask for these treatments to be covered by insurance. Given the costs, private and public insurers will need to consider how best to incorporate new therapies into their offerings.

New payment models may mitigate the impact of upfront costs and uncertainties in value. Some examples of models being considered by stakeholders include:



- **Mortgage option/pay-over-time models** balance upfront costs with long-term savings. Under a mortgage option, a payor could pay for the treatment monthly or annually over a set term of years, allowing the payor to apply the savings achieved to the cost of the treatment.

- **Outcome or value-based contracts** under which the payor only pays if the treatment is effective or pays proportionately to the level of effectiveness achieved. In these arrangements, payments are made as either individual patients or patient populations meet pre-defined health outcomes over time. This puts the risk on the pharmaceutical company rather than the payor.

- **Subscription models**, like that used by Louisiana for Hepatitis C medications. Under this model, Louisiana paid a set fee to allow state funded health programs to have unlimited access to the medication regardless of the number of people treated with it.

For any new payment model, stakeholders will need to address legal and regulatory complexities that could hinder successful implementation. For example, pay-over-time and outcomes-based models depend on patient tracking and access to patient data over time. Other potential difficulties include accounting challenges that may limit the ability of payors to maximize the benefits of a model, addressing payment negotiations for an individual patient moving across different payors, employers, and health systems, and public health care budgeting methods that may not clearly account for gene therapy-related decreases in care costs over time. For these reasons, lawmakers have a critical role to play in understanding where changes will be needed in policy, as well as the public health care infrastructure.

Conclusion

Emerging gene therapies offer the promise of giving patients and their caregivers a new lease on life, expanding the state's workforce and economic output by returning people to the workforce, and creating potential long-term savings to the healthcare system. But these positive outcomes can only be achieved if patients have meaningful access to these cutting-edge treatments. To ensure that Arizona remains on the forefront of ensuring patient-centered healthcare, policymakers should consider convening stakeholders to understand what policy changes are needed to keep current with these breakthrough innovations in health care.

Arizona Chamber Foundation