

Equitable and Efficient Access to Cell and Gene Therapies

Background

Cell and gene therapies are beginning to provide new treatment options for patients with previously untreatable conditions. These novel therapies have already begun to transform the treatment of a growing range of single-gene genetic disorders, cancers, and other chronic conditions by delaying disease progression and providing cures. The pipeline of cell and gene therapies is expanding quickly—with thousands of active trials globally, targeting oncology, central nervous system disorders, infectious diseases, sickle cell anemia, and other conditions.

The patients who could benefit from gene and cell therapies are diverse. From those aged 65 and over and covered by Medicare, to low-income populations, including children, pregnant women, adults, and individuals with disabilities covered by state-run Medicaid programs, from our most vulnerable citizens living near the poverty level who are covered by both Medicare and Medicaid, to those covered by commercial insurance, millions of Americans could seek gene and cell therapies as a treatment option.

Access to the potential of gene and cell therapies to alleviate suffering and death however is challenged by payment and delivery models that have been designed primarily for chronically-administered treatments and paying for “downstream” complications, not for potentially one-time therapies that substantially alter or eliminate the patient’s underlying health risk. In particular, the high upfront price of these treatments can significantly impact the budgets of public and private insurers, creating barriers to their adoption. For example, many genetic conditions disproportionately affect patient populations within Medicaid, including sickle cell, hemophilia or other congenital genetic conditions, and muscular dystrophy. State Medicaid programs face particularly acute challenges regarding coverage of high-cost treatments while balancing stretched state budgets, given federal requirements to cover approved products and the limited tools available now to mitigate short-term financial impacts, encourage more effective and efficient therapies, and assure more equitable access. The Center for Medicare and Medicaid Innovation is implementing a voluntary federal-state collaborative model to address some of these challenges, and policymakers and stakeholders have proposed a range of other possible solutions, but clear, effective, and feasible models have not yet emerged to support a routine and reliable role of gene and cell therapies in substantially improving or effectively “curing” serious and life-threatening illnesses.

Duke-Margolis’ expertise and work related to innovative product regulation, manufacturing and supply chains, collaborative strategies to improve the development of real-world evidence, and value-based payment models for medical products and person-centered care provides a foundation for addressing the range of issues that affect access and affordability of these potentially curative therapeutics.

Why this Work Matters

Policymakers, providers, payers, and patients have significant concerns regarding this pipeline of innovative and potentially curative, but high-cost, therapies. Although cell and gene therapies hold great promise for treating a variety of diseases, today’s payment system could limit the number of patients who could benefit. Many cell and gene therapies are one-time treatments with high upfront costs that

will present significant financing challenges for payers—particularly state Medicaid programs—as more of these therapies are approved.

In addition, because the treatments are relatively new with intended lasting effects, the long-term benefits and risks of side effects or complications remain unclear, and will likely evolve significantly over time. These uncertainties combined with potentially substantial opportunities for improvement present challenges for private and public payers, health care providers, and patients with serious and life-threatening conditions seeking the best treatment options.

Duke-Margolis research and stakeholder engagement highlight numerous policy opportunities to enhance the the accessibility and impact of cell and gene therapies for patients, including:

- **Manufacturing & Supply Chain:** manufacturing and treatment processes of cell and gene therapies are often complex, tailored, and costly, with opportunities to encourage the development and adoption of more standardized, well-characterized, and efficient approaches.
- **FDA Regulation/Oversight:** the novel approaches introduced by cell and gene therapies have led to new regulatory initiatives and potential further regulatory reforms to address their long-term safety, efficacy, manufacturing standards, and approval pathways.
- **Pricing, Payment Models, Coverage:** cell and gene therapies carry high upfront prices for affected patients, creating several financial challenges for payment approaches designed for shorter-term or chronic treatments, including adverse selection risk, timing of payments creating a need for reliable longitudinal real-world data and analyses to improve treatments and increase confidence about their use and outcome benefits, and longitudinal patient support and follow-up.
- **Care Models:** current cell and gene therapies are complex to administer and often require extensive care before, during, and after administration, leading to high costs and the need for further innovation in efficient, patient-centered care models.
- **Real World Data and Evidence:** the long-term safety, effectiveness, and durability of cell and gene therapies are not well known at the time of market approval, .

Finding Solutions

Building on **our existing work in this area and through a combination of convening, expert analysis and policy development, and evidence development**, Duke-Margolis is launching a multistakeholder cell and gene therapy policy initiative to address these numerous challenges. By initiating a range of research activities that focus on key policy issues affecting the innovation, manufacture, regulation, patient outcomes, equitable access, and costs of cell and gene therapies in the United States and globally, we will surface evidence-based, real-time solutions to help ensure equitable and efficient access to these promising treatments.

Advance Our Work

Duke-Margolis has identified this portfolio as one of its emerging focus areas for 2024-2027. We are seeking funding to support new student initiatives and scholarships focused on these biomedical innovations, to expand Duke's community of health policy researchers and faculty, and to establish a

Duke Health Policy Action Fund that will provide start-up funds to support innovative health policy scholarship and solutions for the most pressing challenges in access and affordability of innovative therapeutics.

For more information on this work, please contact Marianne Hamilton Lopez, Duke-Margolis Senior Research Director, Biomedical Innovation, marianne.hamilton.lopez@duke.edu. For ways to support Duke-Margolis and the Gene and Cell Therapy portfolio, contact Morgan Pope, Director of Interdisciplinary Development, Duke University, morgan.pope@duke.edu.