Biomedical Innovation

Emerging Initiative

Duke-Margolis Cell and Gene Policy Initiative

Leveraging cutting-edge medical science, cell and gene therapies now offer new options for patients with previously untreatable conditions, and are poised to revolutionize the treatment of genetic disorders, cancers, and potentially many chronic diseases. The pipeline of cell and gene therapies is expanding—with thousands of active trials globally, targeting oncology, central nervous system disorders, infectious diseases, sickle cell anemia, and other conditions.

Despite this potential, significant challenges exist to advance these novel treatments from labs to the bedsides of the patients with serious and life-threatening conditions that they are designed to help.

The Need

Access to gene and cell therapies to alleviate suffering and death 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—particularly state Medicaid programs— and private insurers, creating barriers to their adoption.

Because the treatments are relatively new, their long-term benefits and risks are unclear after initial approval, and with further progress in manufacturing and administration, will likely evolve over time. These uncertainties present challenges for payers, health care providers, and patients seeking the best—and in some cases, the only—option for treatment. There is a need for better and more reliable postmarket monitoring and evidence development systems for cell and gene therapies can refine procedures, identify and avoid safety issues, and improve effectiveness.

Also, manufacturing and treatment processes of cell and gene therapies is generally complex, bespoke, and costly. The Food and Drug Administration (FDA) and other global regulatory agencies have implemented new regulatory initiatives and are considering further regulatory reforms to address their long-term safety, efficacy, manufacturing standards, and approval pathways. However, a major gap remains between current manufacturing and the potential for much more efficient and automated manufacturing that regulatory, payment, and coverage policies could help address, and encourage the development and the adoption of more standardized, well-characterized, and efficient approaches that can be applied to multiple conditions.

Leveraging Cutting-Edge Medical Science

Duke
MARGOLIS INSTITUTE for Health Policy

Why This Work Matters

We are at a crucial point with new adoptions of innovative but high-priced therapies, along with interest from policymakers and patients.

Duke-Margolis has the complementary research perspectives and expertise to address the complexities of cell and gene therapy. We have studied these issues for a range of other technologies and have already built a body of work that explores payment models and postmarket evidence generation.

Duke-Margolis has built trusted networks with the stakeholders that need to work together towards issues of access and outcomes. We work closely with the Food and Drug Administration and Centers for Medicare & Medicaid Services and have multi-stakeholder collaborations focused on clinical trial transformation, supply chain, payment models, and real-world evidence generation, along with a new roundtable focused on pricing and competition policy. Cell and gene therapies will bring together all of these issues and will require the involvement and commitment of stakeholders across the health ecosystem to find solutions that address both innovation and access for patients.

Duke-Margolis, through its emphasis on cell and gene therapy policy, will build on our existing work and networks to launch a robust body of policy research. 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.

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.

Finding Solutions

Duke-Margolis is working to expand our cell and gene therapy policy focus in important areas.

- **Through a cell and gene therapy policy roundtable.** In 2024, Duke-Margolis is seeking to launch a new initiative dedicated to enhancing the opportunity and accessibility of cell and gene therapies for patients. This work would bring together policymakers, regulators, payers, manufacturers, patients, and health care systems—and our Duke-Margolis team—to address key policy issues affecting the innovation, development, and costs of cell and gene therapies. The roundtable would meet throughout the year to discuss priorities, identify and inform priority policy research questions, and support our analyses and recommendations in this space.
- **Through a comprehensive policy research plan.** As part of this initiative, Duke-Margolis aims to address key policy research studies that address the access, equity, and value of cell and gene therapies. Research topics are likely to include:
  - Regulatory policies and innovative payment and coverage models to address financial obstacles to cell and gene therapies, such as:
    - insurance risk associated with relatively rare but high-cost genetically treatable conditions, especially for Medicaid plans and employers;
    - one-time payment concentration;
    - uncertainty about long-term safety and durability;
    - manufacturing complexity and high cost of goods sold;
    - high costs of therapy administration and patient management, and
    - lowering these costs through the development of reliable platforms;
  - Supporting infrastructure to measure and improve outcomes that matter most to patients and their families, including:
    - strategies to track and improve the long-term safety and effectiveness of cell and gene therapies;
    - long-term management and care coordination for treated patients;
    - incentives and supports to develop and sustain a routine and robust post market evidence infrastructure; and
    - capabilities to address structural barriers and inequities in access to transformative therapies.

Our research will aim to provide timely support for public initiatives, such as the Center's for Medicare and Medicaid Services' CMS CMMI pilot program to provide a voluntary template and standards for state Medicaid programs to negotiate outcome-based agreements with manufacturers of sickle cell therapies, and facilitate private- and public-private action where needed, e.g., to augment existing gene therapy postmarket registries to improve evidence on the reliability and efficiency of these therapies.

We will surface evidence-based, real-time solutions to help ensure equitable and efficient access to promising cell and gene treatments.