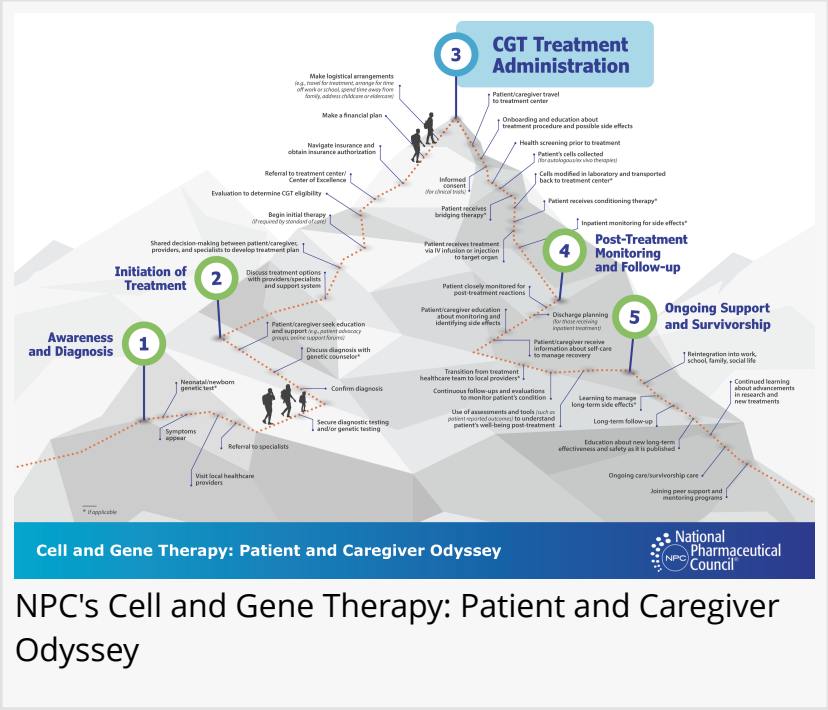
Addressing Challenges on Patients' Path to Cell and Gene Therapy Treatment Is Crucial to Improving Access and Outcomes

A new white paper from the National Pharmaceutical Council analyzes the cell and gene therapy patient and caregiver journey to provide stakeholder solutions.



NPC's Cell and Gene Therapy: Patient and Caregiver Odyssey

WASHINGTON, DC, UNITED STATES, January 29, 2025 /EINPresswire.com/ -- A new white paper from the National Pharmaceutical Council (NPC) analyzes the challenges faced by patients and caregivers pursuing cell and gene therapy and offers recommendations to improve patient outcomes and access to these life-changing treatments. The publication, "Cell and Gene Therapy: Patient and Caregiver Odyssey," was authored by Tyler D. Wagner, PharmD, PhD; Brian Sils, MPP; and Jonathan D. Campbell, PhD, MS.

Cell and gene therapies (CGTs) are transforming personalized medicine by providing targeted treatments for complex and rare conditions, offering hope for improved health and longevity. Although many CGTs are still under development — with 66 new approvals expected by 2032 — the path to treatment is physically and emotionally arduous for patients and caregivers. Diagnoses typically take up to seven years and complex insurance hurdles often delay treatment initiation. Once eligible, patients may face lengthy production times for CGTs, along with intensive monitoring and follow-up — commonly requiring extended hospital stays and travel to specialized facilities. Despite these challenges, CGTs hold promise for significant breakthroughs in treating serious illnesses.

Typically, patients and caregivers face distinct challenges throughout their journey:

 Awareness & Diagnosis: Patients face long delays, misdiagnoses, and barriers (particularly for rare diseases), often taking years to secure a correct diagnosis.

 Initiation of Treatment: Before starting CGT treatment, navigating treatment options, referrals, insurance approvals, and financial planning presents significant challenges.

 CGT Treatment Administration: The process of preparing and administering CGTs is complex. It often requires extended hospital stays and intensive monitoring, which creates significant physical and emotional challenges.

 Post-Treatment Monitoring & Follow-up: Patients undergo long-term monitoring for side effects and efficacy, with follow-up care lasting years.

 Ongoing Support & Survivorship: Survivors manage reintegration into daily life and potential long-term side effects, often aided by peer support.

"There is not enough discussion around the obstacles that patients encounter in just about every stage of their cell and gene treatment journey," said Brian Sils, NPC Research Consultant. "This report helps decision-makers better understand what patients are experiencing throughout this process. We can then have an informed conversation about how to help patients reach their full potential through these transformative therapies."

To address these challenges and improve the patient and caregiver experience, NPC's white paper offers specific stakeholder solutions for each step of the treatment journey:

 Promote Early Diagnosis: Expand newborn screening for genetic conditions, develop and broaden the adoption of new genetic tests, and standardize treatment protocols.

 Introduce New or Innovative Payment Models: To address CGT financing concerns, consider where appropriate annuity-based payments or outcomes-based payments, rebates, or annuities.

 Invest in Infrastructure: Improve infrastructure by expanding manufacturing capacity via platform technologies and implement hub-and-spoke models to link community healthcare practices with specialized treatment centers.

 Offer Coordinated & Comprehensive Patient Support: Stakeholders, including payers, employers, and providers, must build relationships with patients and caregivers and provide ongoing support via financial guidance, decision-making counseling, and travel benefits.

 Track Long-Term Safety & Efficacy Data: Stakeholders should work together to track patient data over time to understand the long-term safety and efficacy of treatment.

"Stakeholders across the healthcare system must work together to address the barriers patients and their caregivers face throughout the entire diagnosis and treatment journey," said Tyler

Wagner, NPC Associate Director of Research. "This is crucial for patients and their caregivers to fully realize the potential of innovative cell and gene therapies."

Read the Research: "Cell and Gene Therapy: Patient and Caregiver Odyssey"

More From NPC: "Improving Access to Gene Therapies: A Holistic View of Current Challenges and Future Policy Solutions in the United States" | Journal of Comparative Effectiveness Research

About the National Pharmaceutical Council
NPC serves patients and society with policy-relevant research on the value of patient access to innovative medicines and the importance of scientific advancement. We envision a world where advances in medicine are accessible to patients, valued by society, and sustainably reimbursed by payers to ensure continued innovation. For more information, visit www.npcnow.org and follow NPC on LinkedIn.

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