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Gene Therapy Legislation Codifies Best Price(s)

Institute for Gene Therapy Hosts Capitol Hill Briefing, Works with CMMI on CGT Pilot

Relevant Companies

On February 21 the Institute for Gene Therapies (IGT) hosted a Capitol Hill briefing on the potential of gene therapies in treating rare and ultra-rare diseases as well as their policy asks for the new Congress. Panelists included IGT Chair Erik Paulsen (former House member, R-MN) as moderator, Dr. Sudha Sharma from National Genome Center at Howard, Nate Plasman, a patient advocate for Duchenne muscular dystrophy, and John Feore, the IGT Policy Director

⋙ Our Take & Next Up

The Capitol Hill Cell & Gene Therapy briefing was the first of three that will be hosted by IGT. Future meetings will likely include discussions on reimbursement options.

CMMI is conducting stakeholder meetings on cell & gene therapies to help shape their Medicaid model. We wait to see what the model will look like in 2024/2025. By then, the number of FDA approved treatments is also expected to significantly increase. Cell and gene therapies still face significant hurdles in development and struggle to address safety and long-term efficacy.

- **To quality for gene therapies, genetic testing is often required, presenting an initial barrier.** Additionally, manufacturers are struggling to provide long-term durability data due to the small number of patients in their clinical trials and difficulty in maintaining long-term data collection methods.
- **Clinical trials have also struggled to advance due to serious safety concerns from treatments.** Most recently, Graphite Bio ended development of its sickle cell gene therapy due to serious adverse events in the first patient dosed.
- **There are also human factors that impact the success of treatments** - which complicate gene therapies. e.g., Luxturna (Roche/NVS) has CMS coverage through a local coverage determination (LCD), but many older adult patients do not qualify for treatment due to the possible lack of viable retinal cells.

Key Points

Cell & gene therapies have garnered attention in Washington this year for not only curative traits but also concern overpricing/reimbursement by the government. Gene therapies are curative treatments that target the genetic causes of a disease by introducing genetic material through a viral vector, while cell therapies involve the transfer of live, intact cells to help lessen or cure a disease ...

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