A LIFELINE FOR RARE DISEASES: CREATING A FEDERAL FINANCING POOL TO MAKE HIGH-COST CELL AND GENE THERAPIES ACCESSIBLE

Cell and gene therapies provide hope for patients suffering from rare, debilitating and costly diseases. But patient access to these innovative treatments poses a serious conundrum for the entire health care system. While health insurance is designed to spread risk among a broad swath of the population, the rare nature and costs of these therapies pose significant access and affordability challenges. To mitigate the exorbitant costs, Congress should view these cutting-edge therapies as a public good and create a national risk pool.

A national risk pool is designed to spread the financial risk associated with high-cost drugs across a larger population to ensure that individuals with high drug cost needs can access coverage for those therapies or cures.

To ensure access to novel cell and gene therapies, a national risk pool would:

1. Mandate participation by all payers so the right patients have access at a manageable cost and risk is spread.
2. Finance the cost of treatments through per beneficiary per month fees. Drug companies would be required to participate in outcomes-based contracting to back the drug's efficacy and bring down the costs.
3. Be managed by the federal government or its designee to maintain independence from industry or other special interests.
4. Establish criteria to limit the therapies included in the national risk pool to those therapies treating the rarest conditions and/or having the most expensive price tags.

Background

Cell and gene therapies are a type of precision medicine that tailors treatments to a patient's DNA. As of September 2024, there are 39 approved cell and gene therapies. Once an emerging science, FDA anticipates approving 10 to 20 cell and gene therapies annually beginning in 2025. Of the 10 most expensive drugs in the U.S., seven are cell and gene therapies treating illnesses such as Metachromatic leukodystrophy, Hemophilia B and Duchenne Muscular Dystrophy. While these new treatments have the potential to have a profound impact on patient health and quality of life, including the ability to cure diseases, the costs and growing drug pipeline are unsustainable.

Community health plans serve smaller, more concentrated populations, leading to a limited risk pool. As a result, the financial impact of covering multi-million-dollar therapies disproportionately affects these plans. This can lead to significant premium increases or, in the worst-case scenario, bankruptcy for the plan. With million-dollar therapies continuing to enter the market, the health care system must have an affordable solution. Federal policymakers can balance the need for patient access and ensure the value of these novel therapies through a national risk pool.

In 2023, Casgevy and Lyfgenia were approved to treat sickle cell disease with price tags of $2.2 million and $3.1 million, respectively. With an estimated patient population of 100,000 Americans diagnosed with sickle cell disease, the potential cost of these two therapies could be more than $200 billion annually.

The Alliance of Community Health Plans (ACHP) represents the nation's top-performing non-profit health plans to improve affordability and outcomes in the health care system. ACHP member companies provide high-quality coverage and care to tens of millions of Americans across nearly 40 states and D.C.

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