New biotreatment options pioneered in Ohio require new funding approach

Traditional models for covering health care costs don't work in a world where formerly fatal conditions can be managed or potentially cured with a single dose of cell and gene therapy. Instead of being saddled with continued expensive treatment for severe chronic illness over a lifetime of disability, a growing pipeline of pioneering therapies requires creative new approaches to pay for lifesaving and life-changing treatments.

That was the message for Ohio legislators in a briefing at the Riffe Center Nov. 16 presented by Ohio Life Sciences (formerly BioOhio) and the Little Hercules Foundation, which advocates for patients and families dealing with Duchenne muscular dystrophy.

"This is an exciting time in Ohio for the life sciences industry, said Eddie Pauline, president and CEO of Ohio Life Sciences.

"More life-saving treatments and drugs are being created here from our amazing research institutions. That research is leading to more companies emerging here and staying here in Ohio, employing Ohioans at a high rate of pay. We have companies outside of Ohio investing in this state more than ever," he told lawmakers. Boston-based Sarepta Therapeutics, for example, came to Ohio after purchasing biotechnology developed at Nationwide Children's Hospital in Columbus, Pauline said.

Kelly Maynard, Little Hercules president and founder, said new cell and gene therapies are "transforming many rare and life-limiting conditions into manageable chronic conditions but are also extending and saving lives for patients where there are no other treatment options."

Cell and gene therapy replaces missing or faulty cells or genes with new working copies, often in a single dose. Some therapies use a person's own cancer-fighting or immune cells to treat cancer, for example. Ten such treatments have been approved by the federal Food and Drug Administration and about 2,000 are in clinical development.

Aggie Fritsch, 4, would not be alive today if not for the gene-therapy treatment she received at 8-months-old in 2019, her mother, Haley Fritsch, said in a video shared with legislators. The Pioneer, Ohio, child born with spinal muscular atrophy, or SMA-1, would have already succumbed to her usually fatal genetic disease but for receiving a single dose of what was then the most expensive gene therapy in the world. Instead, "everything she's doing is a miracle," her mother said, including singing, lifting her arms above her head and moving her legs.

Most babies with SMA-1 die before their 2nd birthday due to severe weakness, including difficulty swallowing. These babies require machine ventilation to breathe and a g-tube for nutrition before the fatal disease takes their life. But with the $2.1 million treatment Aggie received at Cincinnati Children's Hospital, Haley Fritsch doesn't doubt her daughter will "live a long, healthy life."

To support continued development of cell and gene therapies, policymakers must embrace new payment models in which one-time remedies that initially seem prohibitively expensive are viewed as

viable, even preferable, to life-long treatments that will eventually exceed the cost of potentially curative cell and gene options, said Dr. Marianne Hamilton Lopez of the Margolis Center for Health Policy at Duke University.

Cell and gene therapies offer the first hope for many families, said Hamilton Lopez of Washington D.C. The challenge is balancing innovative treatments with the reality of cost and access, she said. Costs for 10 currently available therapies range from about $373,000 to $3 million, she said. With some 200 clinical trials now in phase three, FDA approvals are expected to increase over the next few years, she added.

Cell and gene therapies offer durable outcomes and, in some cases, even cures, Hamilton Lopez said. More than half of the therapies now being tested target various cancers, with smaller shares testing treatment for central nervous system disorders, infectious diseases, hematology, genetic disorders, immunology and cardiovascular and musculoskeletal conditions.

Because cell and gene therapies are so new, data about their long-term outcomes is still being evaluated, Hamilton Lopez said. While outcomes are scrutinized, some state Medicaid programs, private insurers and the federal Medicare program are developing alternative value-based purchasing systems to spread coverage for cell and gene therapies over time with required periodic updates on a treatment's effectiveness.

These innovative models make it easier for payers to absorb higher initial costs of cell and gene therapy while holding expenses to reasonable levels. For example, Washington and Louisiana have enacted caps to expand the use of therapies that cure hepatitis C without substantially increasing state Medicaid spending, Hamilton Lopez said. However, states may have limited capabilities to obtain and analyze the data needed to assess the effectiveness of cell and gene therapies over time and may need to work with third-party data aggregators, she said.

Creating new payment systems to support continued development of cell and gene therapies "is a heavy lift to do disease by disease," Maynard said. "It's something we have to keep talking about…. We've just got to keep connecting the dots with all stakeholders," she added. Early indications are that payment for cell and gene therapies have not exceeded 2% of a state's overall Medicaid costs, Maynard said.

"Since Ohio is a place where these innovative treatments are being created, we also need to be a place there they're accessible to Ohioans," Pauline said. "I think it gives Ohio an opportunity to be a leader across the country if we can be a destination for individuals to know that they can live here and have access to these treatments. It will have tremendous impact on our continued economic prosperity if we can figure that out."