Priceless cure for baby cost $2.1 million, but insurer paid and now she's expected to live

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Four-month-old Ainsley Cardente smiles, enjoys "helicopter" rides from her mom and protests when lunch is late. Perfectly ordinary, all of it. And her life is expected to remain that way thanks to a $2.1 million drug

recently infused into her little body by doctors from Johns Hopkins Hospital.

Ainsley was born with a rare genetic disorder called spinal muscular atrophy, or SMA, that kills most babies by age 2. Rarely, there are less severe versions like the one that afflicts Ainsley that lead to a slower but still catastrophic decline.

"She'll never be in a wheelchair; she'll never feel like a sick person," said Kate Cardente, Ainsley's mother, a physician assistant from the Annapolis suburb of Arnold. "She'll never remember it."

But Ainsley almost didn't get the medicine known as Zolgensma. On the market since May, it's the nation's most expensive prescription drug and the Cardentes' insurer initially refused to cover the cost. So Cardente and her husband, David, fought to get it approved. A letter from Ainsley's doctor at Johns Hopkins Hospital, a top SMA expert, helped win coverage.

"We have a lot to be thankful for this year," Cardente said at a Johns Hopkins outpatient center in Dundalk as a line pumped the drug into Ainsley's foot over about an hour.

But the emotional and potential financial burden is likely familiar to many families without the ability to pay out of pocket for necessary drugs. As more gene therapies and other innovative—and ultra-expensive—treatments are approved in coming years, such challenges are likely to become more common even as they engender hope.

"Ainsley is going to be normal, and, oh my God, that is so great," said Dr. Tom Crawford, the Hopkins pediatric neurologist who treats Ainsley and every baby with SMA in the region. "This is a new world, but society is going to have to figure out how to pay."

Congress is grappling now with how to bring down the cost of pharmaceuticals, a principal issue for the late U.S. Rep. Elijah Cummings. Drug companies say some of the sky-high costs are due to research and development of novel, life-saving drugs aimed at small numbers of patients. But other older, widely used drugs also have jumped in cost.

In a report last April, Cummings, the Baltimore Democrat who chaired the House Oversight and Reform Committee, highlighted the significant difference in the cost of the diabetes drug insulin in the United States compared with other countries. He said that led some patients to ration their life-saving medication.

Spinal muscular atrophy is a neuromuscular disorder that leads to progressive muscle weakness, paralysis and most often death. It's caused by the absence of a gene that produces a critical protein needed to preserve motor neurons. Without it, those neurons rapidly die.

AveXis, the Illinois-based biotech company that developed Zolgensma, notes that its cost is lower than the lifetime cost of the other main treatment for the disease, which can exceed $4 million over a decade. Biogen's Spinraza was approved in 2016 by the U.S. Food and Drug Administration but requires regular and costly spinal injections to stem deterioration.

Zolgensma, approved for children under age 2, works by replacing the missing or nonworking gene permanently with one dose.

"AveXis recognizes the burden and emotional toll of SMA and the urgency" for treatment before neurons die, the company said in a statement to The Baltimore Sun.

"Our goal is to support access for patients who need this one-time gene

therapy, and we're pleased each time a child is approved for treatment with Zolgensma," the statement said. "As a single, one-time intravenous infusion designed to provide long-term benefit, Zolgensma's price reflects the long-term value it brings to SMA patients, families and the healthcare system."

The company said 90% of commercial insurance patients and 30% of government-funded Medicaid patients are now covered. Including patients in clinical trials, about 100 children around the country have been treated so far. About 450 to 500 infants in the United States are affected by SMA a year.

The medicine's stunning price got something of an endorsement recently by the independent watchdog group Institute for Clinical and Economic Review. The group's analysis determined its cost was "actually fairly aligned with how well the treatment both extends and improves patients' lives," said David Whitrap, a spokesman for the institute.

He said Novartis, which acquired AveXis, initially suggested it would price Zolgensma at $4 million but then offered it for the $2.1 million.

"This is a potential cure for an always-fatal childhood disease, and therefore it is precisely the kind of swing-for-the-fences innovation that the U.S. health system should incentivize and reward," Whitrap said.

He warned, however, the price means patients and their insurers will have to stop paying "far too much for all the other drugs that do far too little for patients." He also said the market should not wrongly translate its support for Zolgensma's cost as endorsement of the price for all gene therapies.

That's the "absolute wrong takeaway" considering 20 new such therapies are expected in the next five years, he said. Each should be evaluated

individually.

Ainsley's insurer initially said she was not sick enough. She is one of the rare SMA cases with four copies of a backup gene that lessens the disease's severity. Babies with fewer copies generally die quickly, while those with more copies deteriorate more slowly. Ainsley likely would have been in a wheelchair in grade school.

Researchers initially thought those with four gene copies might never develop symptoms and be treated unnecessarily, but Crawford said they now believe that is a "vanishing concern" and the risks of delaying treatment are too high.

Crawford smiled as he watched a staff of nurses and others tend to Ainsley last month. They loaded a large syringe with the drug, which had been delivered by special courier from a manufacturing plant near Chicago in a small box, which was later presented to the Cardentes with a bow.

Ainsley sat on her mother's lap in a Hopkins-issued onesie unmoved by the momentousness of the day.

Five to eight SMA cases are identified in Maryland a year, and two-thirds of the children would die without treatment. Over the last quarter century, Crawford says he attended dozens of those deaths from what was known as "the most hopeless disease in pediatrics."

On this day, however, he marveled at no longer being "a hospice doctor." Beyond a small amount of treatable liver damage, he expects Ainsley to do normal things like go to school and prom and have a career and a family.

She was the second baby for whom Crawford secured approval to treat

with Zolgensma, and he is unsure of the battles ahead. He still doesn't know why Ainsley's rejection was reversed in December by the insurer, a federal insurance program used by the U.S. Department of Defense, where David Cardente is an auditor.

CareFirst BlueCross BlueShield administers the Federal Employee Program for the government and made the initial decision to decline coverage, as well as the decision to reverse. CareFirst BlueCross BlueShield officials declined to comment for this article.

In addition to the case made by the doctor, the Cardentes turned to others, including the offices of Maryland Attorney General Brian Frosh, U.S. Sen. Chris Van Hollen of Maryland, Maryland Health Secretary Robert Neall and The Baltimore Sun.

Frosh's office offers free consumer services to those with billing and other disputes with health care providers. Sometimes consumers can't get into a clinical trial or they are denied a transplant. Increasingly, people have disputes over drug coverage.

The Health Education and Advocacy Unit in Frosh's office works with state insurance regulators and can refer matters for criminal or civil reviews. Agents handle about 2,000 complaints a year, including about 700 appeals. Among those denied treatment, the office claims a 50% rate of reversals, amounting to millions of dollars in covered costs each year.

Kim Cammarata, the unit's director, said residents have rights in state law and through the federal Affordable Care Act that include insurer appeals and outside reviews.

"No doubt, it can be hard to navigate the system," she said. "Some issues may seem mundane, but every time you're dealing with a consumer it's

important. It's their drug or procedure. In the Cardentes' case, it saved the baby's life."

There are several measures in Congress to address the cost of drugs that have various levels of support. One would allow Medicare, the government health program for seniors, to negotiate drug prices. One from Van Hollen, called the "We Paid Act," would limit prices of drugs that were based on federally funded research.

A study published last year in the *Proceedings of the National Academy of Sciences* found that all 210 FDA-approved drugs from 2010 to 2016 were based on taxpayer-backed discoveries. Such a law might have applied to Zolgensma because the drug company researchers received millions in federal grants as well as charitable donations, according to KEI, a nonprofit that tracks funding.

It's unclear whether any measure might pass Congress and be signed by President Donald Trump, but Van Hollen said it's imperative given how many families face high prescription drug costs.

"Whether it's a daily need like insulin or a specialized treatment like what the Cardente family required, American families should be able to afford the medicines they depend on," the Democrat said.

Dr. Margaret Moon is chief medical officer for the Hopkins Children's Center and on the faculty at the Hopkins Berman Institute of Bioethics. She said there are questions about how society should pay for expensive treatments and health care generally.

"People are freaked out about spending $2 million to save one child's life, but think of all the money spent at the end of a life when the body has shut down," she said. "We allow health care in this country to be outrageously expensive."

Americans have decided as a society they do not want to ration health care, she said. But not everyone can pay, and there aren't always government or charitable programs to cover costs, even when the treatments are life-saving, like chemotherapy, she said.

Physicians and administrators at Hopkins spend a lot of time deciding how to provide as much care as possible to patients without exposing the hospital to too much risk that it won't be reimbursed, Moon said. When Spinraza was approved for SMA, for example, the hospital would ask the drugmaker to supply a first dose to a child as they worked out insurance payments for more. That wasn't possible with the one-dose, $2 million Zolgensma.

Medical advances continually push the hospital to find ways to cover the costs, she said.

"Sometimes we're stuck with unanswerable questions," Moon said.

Ainsley is doing well now, even rolling over from back to front and sometimes front to back. The Cardentes said they'd like to figure out a way to help other families. Kate Cardente said that might mean just telling them how they advocated for Ainsley.

"There really isn't a blueprint for the next family," she said. "I'm just so glad we got our miracle day."