MedicalXpress

Drug for spinal muscular atrophy prompts ethical dilemmas, bioethicists say

December 11 2017

When the Food and Drug Administration approved the first drug for people with spinal muscular atrophy a year ago, clinicians finally had hope for improving the lives of patients with the rare debilitating muscular disease. But the extraordinary cost of the drug and complicated logistics of delivering it present barriers for many patients, according to experts in bioethics at the Stanford University School of Medicine.

They teamed up with colleagues at several other institutions to discuss concerns related to the medication in an article that will be published Dec. 11 in *JAMA Pediatrics*.

Chief among those concerns is that the $125,000-per-dose cost of the drug, nusinersen, could restrict long-term patient access to it and the ability of clinics to provide it.

"I don't think anyone looks at the evidence that we've seen so far and thinks that it's a bad idea to use the medication as an option for patients. But the cost really ends up being a significantly limiting factor," said Alyssa Burgart, MD, MA, medical director of clinical ethics at Lucile Packard Children's Hospital Stanford and assistant professor of anesthesiology, perioperative and pain medicine at the School of Medicine.

Other concerns, she said, include the lack of guidelines about fair allocation of the drug, uncertainty about its lasting benefits and the risks of treatment.

Burgart is the article's lead author. Chris Feudtner, MD, PhD, of the University of Pennsylvania and Children's Hospital of Philadelphia, is the senior author.

'A huge step'

Burgart said it's an exciting time for treating spinal muscular atrophy, or SMA, and that the drug is a "game-changer" for families. "It's not a cure," she said. "But it's a huge step, and you hate for the price tag to be the reason that a family has to be devastated all over again, as if the devastation of a diagnosis isn't enough."

Spinal muscular atrophy is a rare genetic disease that interferes with the body's ability to make the survival motor neuron protein, without which patients lose muscle control and strength, and eventually the ability to move, swallow or breathe. The most common type of the disease is SMA-1, which is diagnosed in babies between birth and 6 months old.

Nusinersen, which is injected into the spine and works by temporarily enabling SMA patients to make more of the survival motor neuron protein, is one of the most expensive drugs on the market. Six injections are required in the first year, for a cost of $750,000, and three are required in subsequent years, at a cost of $375,000 a year. Insurance companies cover some patients, but the criteria aren't uniform. It's also unclear how long insurance companies will cover a particular patient.

Burgart said clinicians have encountered cases in which insurance companies cover the medication only for the younger of two siblings because the older child has more disabilities and so doesn't meet their criteria for covering the progressive disorder. "I don't know what it's like to be that parent and to have the joy at the opportunity to potentially modify one child's life, and not have the opportunity for your slightly older child. It's a very cruel time, I think," she said.

Before the FDA approved nusinersen, which is marketed by Biogen and Ionis Pharmaceuticals as Spinraza, the only care options were palliative care or supportive care, including physical therapy, respiratory help or assistive devices. Most babies diagnosed with SMA-1 die before their second birthday, but patients with SMA types that show up later in life live longer.

Early treatment crucial

Early treatment of SMA is crucial for halting muscle degeneration, especially in infants, Burgart said. A blind national clinical trial of nusinersen involving 121 SMA-1 patients found that babies receiving the drug showed significant motor function improvement when compared with babies in the trial who didn't get the drug. The results were significant enough to prompt the FDA to halt the trial early, in August 2016, and expand access to all SMA-1 patients. But early approval left medical centers scrambling to establish treatment programs, which became more urgent when the FDA approved the drug for all SMA patients in December 2016, Burgart said.

Even before final FDA approval, neuromuscular and biomedical ethics teams at Stanford, including pediatric neurologist and SMA expert John Day, MD, PhD, were already collaborating with colleagues around the country to grapple with the practical and ethical challenges of providing treatment.

"It really was thinking about ethical issues as they were coming up: 'How can we provide frameworks for what the issues are—and what are the fair and ethical ways to address them?'" said one of the article's co-authors, Holly Tabor, PhD, associate professor of medicine and assistant director of the Stanford Center for Biomedical Ethics.

For example, what would be required to set up SMA treatment programs

with high standards of care? How many patients could each clinic accept? How many programs were needed? How would clinics manage waiting lists? Would they have benchmarks, such as disease progression levels, for deciding who gets treatment? Is patient improvement the only measure of success, or is it also maintaining existing ability levels?

Bodies fail, but minds work

"For some of these patients, if all they have left is, say, the ability to move one finger or to move their assistive device or they still have the ability to raise an eyebrow as a way to communicate, how can I say that maintaining that is not just as worthy as treating a baby to prevent another disability?" Burgart said.

That's especially difficult because SMA patients don't lose cognitive function, she said. Their bodies are failing them, but their minds still work.

Finally, the cost of treatment looms large not only for patients but also for institutions providing care, with institutional mission playing into every decision.

"If you are interested in making money, you can cherry pick your patients and only take patients who have specific types of insurance, where you'll get a great reimbursement back and can make a lot of money," Burgart said. "But if your mission is to provide the best possible care to your patients, then making money is nowhere near the No. 1 goal. However, if you just treat everyone, irrespective of these issues, and don't think ahead, you can bankrupt your hospital, and then you can't provide any care to anyone. And that's certainly not in the mission of a pediatric hospital."

David Magnus, PhD, director of the Stanford Center for Biomedical

Ethics and professor of medicine and of biomedical ethics, said those concerns resonate beyond treating SMA patients, who represent a relatively small group of people, because competition is fierce for resources to treat patients with any number of illnesses for which new and more expensive treatments are emerging. That forces hospitals to make difficult priority decisions.

'Resources are finite'

"Those issues play out in the hospital every day because resources are finite. Beds are scarce, staff is scarce, equipment, surgical space and research resources are all scarce," said Magnus, a co-author of the article. "What are you going to decide not to do?"

There is some financial relief for patients through a Biogen program called SMA360, which helps patients navigate the treatment process and covers costs for some patients. Relief could also come as new treatments—and resulting market competition for nusinersen—emerge, Burgart said. Several SMA treatment trials are in progress, but the community is especially tracking a single-dose gene therapy that, if approved, would have its own cost and treatment implications.

In their article, the authors stressed the need for clear communication among stakeholders about all the issues they considered, especially so patients are well-informed about risks that might not be known yet. For now, they wrote, researchers and clinicians will continue to share any new data about nusinersen use in SMA patients to inform future decisions, including how broadly treatment should be pursued.

"If it's too liberal, you treat patients who won't benefit, and all they incur is more risk. And if you make it too narrow, you never figure out that you can actually accomplish more," Burgart said. "I think the only solution is to treat as broadly as you can, continue to gather data and

really continue to look at it in a detailed and thoughtful way that helps patients the most."

Provided by Stanford University Medical Center

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