Spinraza, which treats Spinal Muscular Atrophy (SMA), a rare genetic condition in children, costs $750,000 for the initial year of treatment. The Food and Drug Administration approved the first treatment for the condition two days before Christmas in 2016.

Across the country, similar stories are playing out as private insurers and already-squeezed state Medicaid programs wrestle with what, if any, limits to place on patients' access to break-the-bank drugs - weighing the needs of the ill against budge realities.

At the same time, policymakers and physicians increasingly demand to understand why drug manufacturers affix price tags that have risen to once unimaginable highs.

"It looks like a drug that works for a tragic condition that afflicts children and cripples and kills them. That's the good news, "Dr. Jerry Avorn, a professor of medicine at Harvard Medical School, says of Spinraza. But "how in the world did the price of $750,000 a year get chosen?"

Biogen, the maker of Spinraza, defends its price.

"We compared industry norms for other drugs in rare disease. We looked at the efficacy and safety profile of the drug itself," says Dr. Wildon Farwell, an epidemiologist and senior medical director of clinical development at Biogen.

But that logic - comparing a new drug to extremely high-priced drugs already on the market - has only fueled an inflationary cycle, Avorn says, adding: "In my view, that's akin to a kid who get caught bullying another kid and beating him up after school, and says, "Well, all the other kids were beating him up so it's OK.' If it's wrong, it's wrong."

Spinraza isn't a cure for SMA, which affects 10,000 people in the U.S., but clinical tests show it holds promise for some. Scientific discoveries by researchers at the University of Massachusetts Medical School in the early 2000 - partly funded by grants from the National Institutes of Health and donations from patient-advocacy groups - helped pave the way for Spinraza.

The drug was granted "orphan" status, which provides tax credits for research and helped speed the review process. It won approval in five years after the start of clinical trials, based on results of a few small studies. The primary one showed improvement in 40 percent of the infants given the drug. It was tested only on children, most under age 2, though it was approved for pediatric and adult use.

All that means Biogen's research and development costs likely were not unusually high, although the company would not release figures. Five days after getting the FDA's approval to sell the drug in the U.S., Biogen announced the price: $125,000 a dose, or $750,000 for the first year. Fewer doses in following years drop the total annual cost to $375,000. The drug must be taken for life.

The FDA does not know or consider pricing when it grants approval. If just half of U.S. patients get treatment for one year, the tab would be more than $3.7 billion. Spinraza brought in $203 million in the second quarter of this year, more than four times its revenue in the first quarter.

When Biogen unveiled the price tag, one Wall Street analyst at the investment bank Leerink predicted "a storm of criticism" and that insurers would parse "which patients receive access."

In addition to the financial questions, there are other unanswered concerns among families and medical professionals. Will Spinraza work in older children or adults? No children involved in the study were on ventilators at the start of the trial. Is it safe for children on ventilators? Also, because SMA's rate of decline varies, how can doctors, families or insurers measure if the drug is stalling the disease's progression? And, finally, how long will its effect last?

For some fast-track drugs like Spinraza, FDA approvals don't offer this kind of guidance.

Dr. Susan Apkon, who treats dozens of children with SMA as a physiatrist at Seattle Children's Hospital - and who urged Washington state's Medicaid pharmacy board to cover the drug - says there is no easy answer.

"If a drug works, we want to give it to the child or adult, whatever the drug is," says Apkon, who does not receive money directly from Biogen but is a co-investigator in one of the company's ongoing studies.

Still, "there is one pot of money, and we need to figure out how it gets distributed," she says. "The system is broken."

With any costly new drug, it all comes down to "tough choices," says Jack Hoadley, a health policy analyst at Georgetown University's Health Policy Institute.

"Treating one of these patients may mean not treating 1,000 patients with some other, less expensive problem - or saying they have to raise more tax dollars," he says. "Private insurers have the same trade-off. Do we pay for this if it will ultimately raise our premiums?"

Coverage eligibility varies by insurer and, in Medicaid, by state.

Most insurers and Medicaid programs require that patients show some kind of proof of progress - or at least maintenance of function - in order to continue therapy beyond initial doses.

While agreeing that the drug offers some hope to patients, Donna Sullivan, chief pharmacy officer for Washington State Health Care Authority, which oversees Medicaid, says the price tag rankles her.

During a recent meeting with Biogen officials, Sullivan was blunt: "I told them the price was unethical."

In her state, there are about 150 children with SMA. After reviewing the data on Spinraza, Washington Medicaid approved broad coverage rules, including allowing patients on ventilators to get the drug.

But large new spending puts additional pressure on state budgets. When combined with economic downturns, that can lead lawmakers to trim medical provider payments or optional Medicaid services, which include adult dental care, podiatry, chiropractic treatment and other services.
