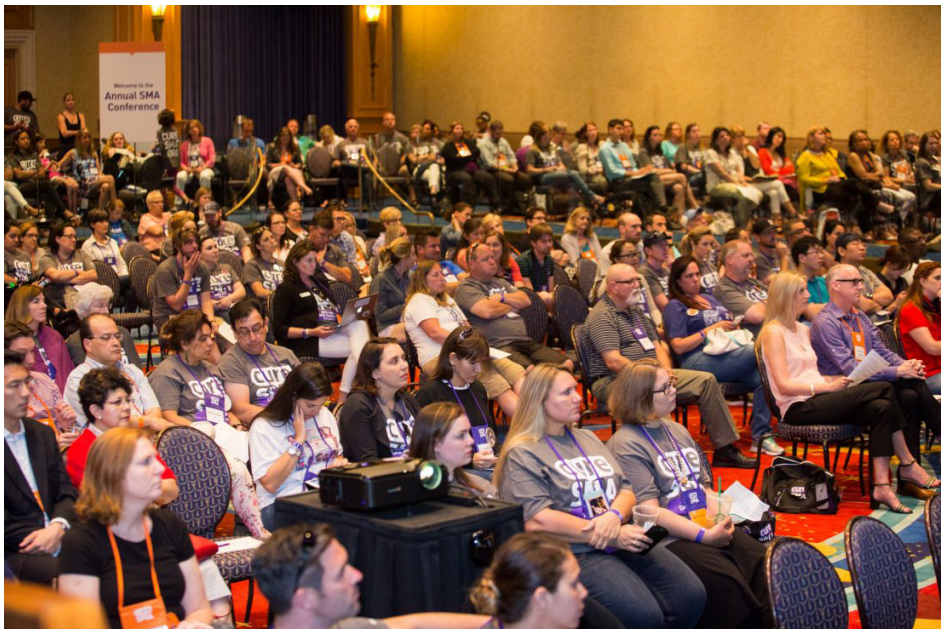
Biogen Sets $750,000 Initial Price For First-Ever Spinal Atrophy Drug



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Xconomy Boston — [*Updated, 12/29/16, see below*] Patients with spinal muscular atrophy got some good news last week **when the FDA approved nusinersen (Spinraza)**, making it the first marketed drug ever for the rare and potentially deadly genetic disease.

But as expected, the drug, from Biogen and Ionis Pharmaceuticals, is a costly one, priced at the high end of the six figure price tags often given to treatments for rare diseases with no alternative treatments.

Biogen (NASDAQ: **BIIB**) this morning disclosed that it has set a list price of $125,000 per injection for its SMA drug nusinersen. The per-injection price translates into $750,000 for the first year of use, when a patient is supposed to receive six injections, and $375,000 each subsequent year for the rest of his or her life.

It's enough to surprise even industry analysts. Brian Abrahams of Jefferies wrote in a research note Wednesday morning that the price comes in "even a bit higher than recently-increasing expectations."

Asked how Biogen landed on the price, Biogen spokesperson Matt Fearer said the company balanced "a number of important factors, including its clinical

value, its impact to patients and the health care system as a whole, and the need for Biogen to fund further research and development to make the next innovation possible."

Nusinersen was approved for patients with all forms of SMA—type 1 through type 4—beating initial expectations that it might only be effective for a small fraction of patients. The FDA also approved the drug months earlier than expected, based on a trial that showed that 40 percent of 82 infants with type 1 SMA—the most severe form of the disease—had improved motor milestones like rolling, crawling, or standing. None of the infants on placebo in the trial achieved such milestones. SMA is caused by a genetic defect that leads to a shortage of SMN protein, which helps keep muscles strong.

The most common side effects in clinical studies were respiratory infections and constipation. Though the FDA included in its announcement a warning about potential kidney damage or dangerously low blood-clotting platelets, Abrahams wrote that the "relatively inarguable value" the drug showed in clinical studies combined with the "broad, clean label" bumped up expectations of its price. Last week, RBC Capital Markets analyst Michael Yee noted that consensus estimates expected an annual price of $225,000 to $250,000 per patient. Biogen has exceeded even those readjusted expectations.

It's important to note that a drug's list price isn't the actual price once insurers and their agents negotiate discount deals and rebates. But those negotiations typically remain secret, so real-world drug prices are rarely publicized.

The average price for a rare disease treatment was $111,820 per patient, per year between 2010 and 2014, according to a 2015 report from **EvaluatePharma**.

Biogen is the latest drug maker to shoot well past those averages, counting on two factors that allow for high prices for rare or "orphan" diseases: A small patient population—there are about 25,000 SMA patients in the U.S.—and no other available treatments.

Patients with type 1 SMA are diagnosed within six months from birth, may never sit or walk, and often die within a few years due to complications. Patients with type 2 through type 4 have a better prognosis, but their lives are still compromised; type 2 patients live longer but might need a wheelchair to get around. A drug that can delay progression of the disease or help patients achieve motor milestones they otherwise wouldn't, as nusinersen is meant to do, could have significant value for these patients.

[*Updated w/comments from Cure SMA*] That's why even despite the high price tag, Kenneth Hobby, president of the nonprofit patient advocacy group Cure SMA—which, it should be noted, draws a small portion of its yearly funding from drug makers—said his group has always supported the business model of pharmaceutical development. That model incentivizes companies to develop innovative drugs, and "has now resulted in a life-saving and family-saving [SMA] drug," he says. In a rare disease like SMA, the "high costs and high risks" of drug development have to be recouped "and more importantly incentivized" up front through higher individual payments spread over a smaller total group of patients, Hobby says.

"The main issue for us is beyond specific pricing, but is rather about broad access," he says, pointing out that the FDA approved nusinersen for "all ages and types" with no restrictions. "We expect that this will lead to broad insurance coverage, combined with patient-assistance programs, to deliver broad access [to nusinersen]."

Biogen spokesperson Fearer said the company has established a program, **SMA 360**, to help patients facing "non-medical barriers" to treatment, such as financial problems or trouble with coverage.

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"We recognize that treatment cost and access are critical concerns for patients, providers, payers and policy makers," Fearer wrote in the e-mail. "We are working to help ensure that no patient will forego treatment because of financial limitations or insurance status."

Access is a big concern for Khrystal Davis, whose five-year old son Hunter has type 1 SMA and recently received three doses of nusinersen for free under a "**compassionate use**" program, in which patients can get access to an investigational drug outside of a clinical trial.

Davis has seen "significant improvement" in Hunter's movements and respiratory function, but now that the FDA has approved nusinersen, Biogen will

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