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HEALTH

F.D.A. Approves First Drug to Treat Severe Multiple Sclerosis

By KATIE THOMAS MARCH 28, 2017

The Food and Drug Administration approved on Tuesday the first drug to treat a severe form of multiple sclerosis, offering hope to patients who previously had no other options to combat a relentless disease that leads to paralysis and cognitive decline.

The federal agency also cleared the drug to treat people with the more common, relapsing form of the disease.

“I think that this is a very big deal,” said Dr. Stephen Hauser, the chairman of the neurology department at the University of California, San Francisco, and leader of the steering committee that oversaw the late-stage clinical trials of the drug, ocrelizumab. “The magnitude of the benefits that we’ve seen with ocrelizumab in all forms of M.S. are really quite stunning.”

The drug, which will be sold under the brand name Ocrevus by Genentech, showed the most notable results in patients with relapsing multiple sclerosis, appearing to halt progression of the disease with few serious side effects. In patients with the more severe form, primary progressive multiple sclerosis, the drug only modestly slowed patients’ decline, but medical experts described it as an important first step.

Center for Multiple Sclerosis at Mount Sinai Hospital in New York. “Once we open that door, then we do better and better and better. It’s a very encouraging result.”

Genentech, which is owned by the Swiss pharmaceutical giant Roche, said Tuesday that it would charge a list price of $65,000 a year, which — though expensive — is 25 percent less than an existing drug, Rebif, that was shown to be clinically inferior to Ocrevus in the two clinical trials that led to Ocrevus’s approval.

Rebif, sold in the United States by the German company EMD Serono, carries a list price of about $86,000. In a statement, Genentech noted that the price of drugs to treat multiple sclerosis had risen sharply in recent years.

“We feel that the industry needs to start to reverse this trend, and believe that pricing Ocrevus 25 percent less than the comparator in our trials is an important first step,” the company said.

Although people with the relapsing form of the disease have more than a dozen treatment options, many of the most effective drugs also come with significant side effects, which means that doctors often wait to prescribe them until a patient’s disease has advanced significantly. Ocrevus is viewed as relatively safe: Side effects included reactions at the injection site (the drug is infused every six months), and more upper respiratory infections and cold sores.

As a result, “it represents a new treatment option that has the potential to be used earlier,” said Dr. Peter Chin, the group medical director of neuroscience at Genentech, who was closely involved in developing the drug.

An estimated 400,000 people have multiple sclerosis in the United States, and about 15 percent have the primary progressive form of the disease.

In the trials that studied the relapsing form of the disease, which involved 1,656 patients, those taking Ocrevus saw a 47 percent reduction in their rate of relapses compared with patients who were taking an existing treatment, Rebif. In the clinical trial for people with primary progressive multiple sclerosis, which involved 732

Ocrevus works by depleting a specific type of a patient's B cells, which circulate in the blood and are part of the immune system. While they normally help the body fight off infections, they are believed to malfunction and contribute to central nervous system damage in people with multiple sclerosis.

"I think if the safety holds up, it will become the leading M.S. therapy," said Dr. Steven L. Galetta, the chairman of the department of neurology at NYU Langone Medical Center, who is an expert in multiple sclerosis and who was not involved in the clinical trials. But, Dr. Galetta said, the medical community will be watching to see how the drug performs once it is widely available. The clinical trial showed a slightly heightened rate of tumors in patients with primary progressive multiple sclerosis, which he said needed to be monitored closely. "There can be side effects, but you just didn't have enough patients initially to confirm that signal," he said.

Jerrie Gullick, one of the patients who received Ocrevus in the clinical trial, said the drug had significantly slowed the progression of her primary progressive multiple sclerosis since she began taking it about three and a half years ago.

Ms. Gullick, who is 51, had been declining steadily since she learned she had the disease in 2010. At the time she learned she had multiple sclerosis, Ms. Gullick was an active 45-year-old who walked six miles a day between her home in Park Slope and her office in Downtown Brooklyn, where she worked as the chief financial officer of a technology start-up.

But her condition quickly deteriorated, and the drugs she was given appeared to do little to help her. She lost the ability to walk more than two to three blocks at a time, and she had to move out of her third-floor walk-up to an elevator building. Fatigue led her to leave her job, and she began to notice cognitive decline.

Ms. Gullick said that at first she hoped she was on the placebo because while she noticed her disease slowing, her condition did not improve. "From a patient perspective, what you want is to get back what you've lost," Ms. Gullick said. "What this drug seems to do is to stop what was happening."

day I realized I might have 20 or 30 more years of this," she said. "It's like I was on a bullet train, and I was transferred to a local."

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