

# Lawmakers question pricing of drug for genetic disease

February 13 2017, by Kevin Freking

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Marathon Pharmaceuticals' pricing of a drug to treat genetic muscle deterioration in about 15,000 Americans, mostly boys, is raising concerns in Congress where lawmakers repeatedly have challenged drug companies.

Sen. Bernie Sanders, I-Vt., and Rep. Elijah Cummings, D-Md., complained on Monday that Marathon Pharmaceuticals plans to charge \$89,000 a year for a drug that's widely available abroad for about \$1,000 a year.

The two [lawmakers](#) said they believe Marathon is abusing a program that grants companies seven years of market exclusivity to encourage research into new treatments for rare diseases. They said the program was not intended to provide companies lucrative exclusivity rights for drugs that have been available for decades.

"We urge you to significantly lower your price for this drug before it goes on the market next month," the lawmakers wrote.

The U.S. Food and Drug Administration approved the drug last week to treat patients with Duchenne Muscular Dystrophy. While the steroid is commonly used globally, the FDA's action represents the first approval of Deflazacort for use in the U.S. The drug will be marketed under the brand name Emflaza.

The two lawmakers say that "exorbitantly" pricing potentially life-saving

medications hurts patient access and drives up prices for the entire health care sector. They are investigating how Marathon set its pricing and want to know the company's total expenses related to development and approval of the drug. They're also seeking the company's revenue and profit estimates.

The lawmakers requested that the company provide emails between company executives and any insurance companies, pharmacy benefit managers or government payers, including Medicare and state Medicaid programs.

The company said it would respond to the letter later Monday. In its response last week to the FDA's approval, the Illinois-based company said it was committed to ensuring that U.S. patients with prescriptions have access to the drug and that the company would provide the most robust patient support programs "allowed by law to qualifying patients."

Lawmakers challenged Mylan NV last year for its EpiPen pricing, which has climbed more than 500 percent since 2007. CVS is now selling a rival, generic version of the EpiPen at about a sixth of the price of the life-saving allergy treatment.

Duchenne Muscular Dystrophy is caused by an absence of a protein that helps keep muscle cells intact. People with the disease progressively lose the ability to perform activities independently and often require use of a wheelchair by their early teens. Patients typically succumb to the disease in their 20s or 30s, but disease severity and life expectancy vary. In a clinical study of 196 male patients, those taking the [drug](#) had improvements in strength across a number of muscles compared to those taking a placebo.

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