After the approval

The news cycle has churned with controversy after FDA gave eteplirsen the green light.

Sept. 9, 2016

Exondys gets FDA nod

Sarpeta's eteplirsen, now known as Exondys, is conditionally approved to treat people whose Duchenne muscular dystrophy is caused by a mutation in exon 51. Later that day, the biotech firm says the drug will cost on average **$300,000 per year.**

Sept. 28, 2016

Insurers weigh in

Cigna says it will provide broad coverage of Exondys.

Oct. 4, 2016

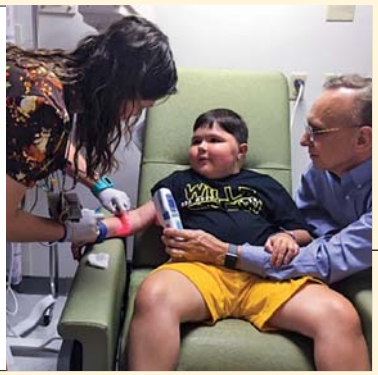
Combination therapy sought

Sarepta pays $40 million for the European rights to Summit Therapeutics' utrophin modulators. The deal follows a smaller agreement to conduct preclinical studies of exon-skipping drugs in combination with Catabasis Pharmaceuticals' NF-kB inhibitors.

Oct. 6, 2016

First patient dosed

A boy at University of Florida Health Shands Children's Hospital receives the first commercially-available dose of Exondys.



Oct. 14, 2016

Sarepta's golden ticket

FDA issues Sarepta a priority review voucher, which can be used to shorten the agency's review of a new drug application by four months. PRVs are transferrable and have been sold for as much as $350 million.

Oct. 7, 2016

Anthem controversy

Anthem, one of the country's largest insurers, says it will not cover Exondys. The company later reportedly agrees to cover the drug for a child whose family challenged the policy.

Oct. 19, 2016

FDA hubbub

John Jenkins, director of FDA's Office of New Drugs, tells the audience at rare disease summit that the path taken by Sarepta is "not a good model for other development programs." He devotes several slides to its deficiencies.

Oct. 20, 2016

JAMA judgment

In an editorial in the *Journal of the American Medical Association,* two Harvard professors, one of whom was on the eteplirsen advisory committee panel, call out eteplirsen's problematic data, question how patient voices should be integrated into the review process, and caution that the eteplirsen approval could have wider implications at FDA.

"The drug has provided a worrisome model for the next generation of molecularly targeted therapies: demonstrate a slight difference in a laboratory test, activate the patient community, win approval, and charge high prices, while relying on limited regulatory follow-up." —Aaron Kesselheim and Jerry Avon of Harvard Medical School

Nov. 4, 2016

Surrogate endpoints questioned

More documents related to FDA's review of eteplirsen are released, including emails between officials debating the reliability of the limited data on the drug.

"Perhaps granting accelerated approval to drugs that show a mere scintilla of an effect on a surrogate endpoint represents a stroke of brilliance—one that will stimulate investment in the development of drugs for these disorders. But in my opinion, this approach should receive broader public (and FDA) input before being implemented." —CDER director Ellis Unger in memo to FDA commissioner Robert Califf

Oct. 26, 2016

Conditional coverage

Insurer Humana says it will cover Exondys, but with narrow parameters. Most notably, it says boys must be able to walk to receive the drug.

Sources: companies, FDA, *JAMA*