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Phone: +1 646 783 7100 | Fax: +1 646 783 7161 | customerservice@law360.com

# Nonprofits Urge HHS To Take Over Sarepta's Exondys Patents

By **Dani Kass**

*Law360 (April 5, 2018, 8:03 PM EDT)* -- A group of nonprofits on Thursday urged the U.S. Department of Health and Human Services to take over five patents covering Sarepta Therapeutics Inc.'s controversially approved and expensive muscular dystrophy drug Exondys 51, saying federal funding wasn't disclosed in the patent applications.

Under the Bayh-Dole Act, inventors are required to disclose when a patent was developed with any government funding, and Knowledge Ecology International has been on a mission to have that enforced. The group has filed multiple petitions targeting patents that cover expensive drugs, hoping to get the government to take over the patents or to negotiate lower prices using that threat.

"It's messy, but feasible, and if the government wants to lower the price, this is something they can do, right now," KEI Director James Love said in a statement. "If the government fails to take title to the patent, it is essentially endorsing the $750,000 per year price of the drug, and the predictable restrictions on access from such a high price."

In Thursday's petition, KEI is joined by Health GAP, Patients for Affordable Drugs, People of Faith for Access to Medicines, Social Security Works and Universities Allied for Essential Medicines.

The five patents cover Duchenne muscular dystrophy treatment eteplirsen, sold as Exondys 51. DMD is a rare neurodegenerative disease that largely targets young boys, weakening their muscles and leaving most in wheelchairs before dying. When the U.S. Food and Drug Administrationapproved Exondys 51 in September 2016, no other treatments were on the market. Since then, the FDA has also approved PTC Therapeutics Inc.'s Emflaza.

Exondys 51 was supposed to cost about $300,000, but the petition argues some patients are actually paying $750,000 to $1.5 million a year for treatment, quoting an article from the New York Times. Given that the treatment has orphan drug exclusivity that doesn't expire until September 2023, the nonprofits say the government needs to address this as a public health issue.

"Given the orphan drug exclusivity for Exondys 51, the stronger measures are necessary for HHS to have leverage to lower the excessive and access-restricting price," the nonprofits said.

According to the petition, the National Institutes of Health gave the University of Western Australia eight grants that contributed to the patents that now cover Exondys 51. The NIH also gave $1.5 million

to the drug's principal investigator between 2004 and 2012, the petition states.

Additionally, the inventors got funds from the Department of Health and Human Services and Internal Revenue Service's Qualifying Therapeutic Discovery Project; Orphan Drug Tax Credits and an FDA priority review voucher. The petition said Sarepta sold that voucher to Gilead Sciences Inc. for $125 million. Other funding came from charities and European and Australian governments.

The FDA had approved Exondys 51 after a particularly small clinical trial with about a dozen patients and results that didn't convince an FDA advisory committee that approval was appropriate. Then-FDA Commissioner Robert M. Califf was forced to intervene to get the drug cleared.

As part of the approval, Sarepta was required to conduct a new clinical trial to confirm its benefits. The trial is expected to conclude in late 2020, and if it fails to show benefits, the FDA could withdraw approval.

Sarepta didn't immediately respond to a request for comment Thursday.

--Additional reporting by Jeff Overley. Editing by Alyssa Miller.