Controversial drug approval stirs deep concerns—and hope

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In September, the Food and Drug Administration approved Exondys, a controversial treatment for Duchenne muscular dystrophy based on tenuous data from just 12 patients. The cover story in *Chemical & Engineering News (C&EN)*, the weekly newsmagazine of the American Chemical Society, explores what the decision could mean for future drugs for Duchenne and other rare diseases.

Lisa M. Jarvis, a senior correspondent at C&EN, notes that Duchenne muscular dystrophy is a deadly disease caused by the malfunctioning of a protein called dystrophin. The protein helps protect muscles from the stress of everyday use. Without a functioning version of the protein, muscles break down. People with Duchenne—most of whom are male—are usually wheelchair-bound by early adulthood and die in their late 20s to early 30s.

Some families of the dozen boys involved in the clinical trial leading to the approval of Exondys, or eteplirsen, attest to the drug's effectiveness. But the study had no placebo arm and yielded little to no quantitative evidence that the drug worked. Critics say that in approving the drug, which costs families $300,000 per year, the FDA bowed to pressure from patient advocates and families rather than making a decision based on scientific evidence. They fear the agency has set a potentially harmful precedent, while families hope the move will buy their sons time for pharmaceutical companies to keep looking for other treatments.

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More information: "After eteplirsen" cen.acs.org/articles/94/i45/et … ntemplate-comes.html

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