Drug companies often deny patients access to experimental treatments for rare diseases. Here’s why

Even with experimental treatments on the rise, patients with rare diseases frequently face an unwillingness by drug companies to provide them before clinical studies are completed. Developing drugs for these diseases is an especially fragile process because the patient populations are small and often diverse, having different genetics, symptoms and other characteristics, which makes studying the drugs’ effects difficult.

Drugmakers believe offering a drug before studies are finished could impair its development and jeopardize FDA approval.

Companies base their decisions on whether to provide a therapy through expanded access on a number of factors, said Jess Rabourn, CEO of WideTrial, which helps pharmaceutical companies run compassionate use programs. In general, there should be evidence that patients can tolerate the treatment and an expectation that any benefit outweighs the risk, he said.

“This idea that you have to wait until the research is done is baloney,” he said. “We’re talking about patients who are going to die if they’re told to wait.”

But drugmakers often view it differently, even though evidence suggests that granting early access very rarely disrupts drug approval.

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