

# FMF Patients Battle for Awareness and Affordable Drugs

[Gabe Friedman](#) August 13, 2014 [Thinkstock](#)



It was just a fever and a sore throat that first caught my father by surprise one morning in the early 2000s. But then the fever persisted on and off for weeks. After a while, he could barely climb a staircase due to his exhaustion. It took seeing five doctors — two internists, an allergy specialist and two infectious disease specialists — to get a correct diagnosis: He was suffering from Familial Mediterranean Fever, a rare hereditary disease that is most prevalent among Jewish, Armenian, Arab, Turkish and other Mediterranean populations.

Because FMF is relatively rare and seldom discussed, the delayed diagnosis was not unusual. But really there's little excuse for the wait: As many as 1 in 5 Sephardic Jews carry one copy of a mutation in the MEFV gene that causes FMF without suffering from the condition, and the mutation also occurs in Ashkenazi Jews such as my father. About 1 in 200 Sephardic Jews, especially those with North African ancestry, experience the disease, which is typically inherited in an autosomal recessive pattern — meaning that two copies of the mutated gene are necessary for the disease to break out.

Mutations in the MEFV gene cause FMF by reducing the effects of the body's natural pyrin proteins, which regulate the body's response to inflammation. On top of the fevers, FMF patients usually experience attacks of severe abdominal and joint pain resulting from exacerbated inflammation. Unless treated with the anti-inflammatory medication colchicine, the episodes, which usually last from 12 to 72 hours, return with variable frequency. Sometimes years can pass between episodes.

Despite its fairly standardized set of symptoms, many doctors still do not recognize FMF at first glance. Dr.

Terri Getzug, an FMF specialist who treats dozens of FMF patients each year at UCLA's Ronald Reagan Medical Center, explained that the disease is rare enough (she estimates that about 10,000 Americans are affected) that doctors have not seen it very often, if at all. "It's just not on their radar," Getzug said.

A rheumatologist eventually recommended that my father take colchicine, a drug that also treats gout and other forms of inflammation. His condition continuously improved, and he has now been symptom-free for over ten years. Although it is not a cure for FMF (there currently is none), colchicine controls the symptoms and for many patients eradicates them altogether. But when it is left untreated, FMF can cause amyloidosis, a potentially fatal buildup of excess amyloid proteins in vital organs. For some FMF patients, amyloidosis can cause fatal kidney failure. Doctors believe that there is a small window of time in which colchicine can stop amyloidosis after it has started in FMF patients.

However, obtaining colchicine has become increasingly complicated since 2010, when Mutual Pharmaceutical Co., a large pharmaceutical company based in Philadelphia, took advantage of the drug's unapproved status. First extracted in 1820, colchicine had been prescribed for gout — without having been tested and approved by the FDA. Since 1962, the FDA has been eliminating untested drugs from the market, but it has only been interested in colchicine for the past ten years or so, says Getzug. Mutual Pharmaceutical ran a series of studies and tests to garner the official approval of the FDA, and in 2010 the company was given exclusive rights to colchicine for seven years — even though it did not test its version of the drug on FMF patients. The pharmaceutical company gave the drug a new name — Colcrys — and successfully sued other companies to remove the cheaper, untested versions from the

market. Soon, the price of the drug skyrocketed from a few cents to nearly five dollars per tablet.

“It’s an example of a company that decided to find a loophole,” said Dr. Stephen Goldfinger, an FMF specialist at Massachusetts General Hospital. “It’s just greed, that’s all it is.”

My father’s insurance package covers the newly branded colchicine at an affordable flat rate, but not everyone is so lucky. Getzug said that the costs initially made people frantic, and patients began hoarding supplies of unapproved colchicine while it was still available. She advised her patients to look to other countries where the drug was cheaper. “It’s something that we hate to tell people to do, but they need their colchicine,” Getzug said. Sonia Batalian Chookoorian, one of Getzug’s FMF patients of Armenian heritage, decided to purchase colchicine from Canada.

Mutual Pharmaceutical eventually set up an assisted payment plan for patients without insurance. However, Getzug said that many patients with insurance that did not cover colchicine failed to meet the financial criteria

necessary for the program’s assistance. “For the people who qualify, the patient assistance plan has worked,” she said. “But many others fall into a hole.”

On top of being too expensive, Getzug said that Colcrys has not been as effective as the untested colchicine for several of her FMF patients, an observation confirmed by Dr. Goldfinger. For Batalian Chookoorian, Colcrys did not suppress the FMF attacks and produced unbearable side effects. Getzug suggested that this could be due to the fact that Mutual Pharmaceutical tested Colcrys on gout patients only. The company, whose exclusive patent on colchicine will run out in 2017, did not respond to several requests for comment.

Patients and doctors eagerly await the return of the older forms of colchicine that are cheaper and more effective to the marketplace, but they know that treatment is only half the battle: Without sufficient awareness, doctors cannot diagnose the dangerous disease in the first place.

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