

FDA Requires Review and Approval of Pancreatic Enzyme Supplements: Information for People With CF

In April 2004, a new rule was issued requiring makers of pancreatic enzyme supplements to get their drugs approved by the Food and Drug Administration (FDA) within the next four years. For enzymes to receive FDA approval, companies must test them in clinical trials in people with pancreatic diseases, which includes cystic fibrosis (CF). These clinical trials would confirm the safety and effectiveness of the enzymes. People with CF who take enzymes to digest their food still will be able to get their prescribed enzymes during the next four years. This document will help answer questions you may have about the FDA's rule and its possible effects on the enzymes you take.

Why did the FDA make this rule?

The goal of this new rule is to make sure that pancreatic enzymes have the right amount of active ingredients to digest food; inconsistencies in enzyme formulation can cause problems with digestion. There are several types of enzymes on the market now, which vary in what they contain, how they are made and how many pills must be taken. It is difficult to know the exact amount of active ingredients in the different enzymes. By conducting clinical trials and requiring FDA approval, the manufacturing process of pancreatic enzymes will be standardized, which ensures the consistency of the capsules from batch to batch. In addition, by having more precise information on these enzymes and how effective they are, doctors will be better able to prescribe the right amount of enzymes.

When will the rule take effect?

The FDA's rule will take effect immediately. Enzymes that are available now will likely remain so over the next four years. During this time, makers that choose to do so will conduct clinical trials to make sure their enzyme products are safe and effective. Once the clinical trials are complete, the FDA will look at the information and decide if that company's enzyme or enzymes should be approved. At the end of four years, if a maker has not received FDA approval, its enzyme product will no longer be available and your doctor will prescribe an appropriate enzyme product for you.

Why haven't enzymes been approved by the FDA before?

Pancreatic enzymes, like a number of older medications, were first made before the FDA required them to be tested in clinical trials to see how well they work. The FDA allowed these medications to be "grandfathered" products, so they could be put on the market without its review and approval. This new rule means that the FDA requires enzymes to meet the same standards of testing as any other new drug. Because of the importance of pancreatic enzymes for people with CF, the FDA will let these enzymes remain available in the meantime.

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Are clinical trials of pancreatic enzymes really necessary? Isn't this a step backward?

This is a step forward in improving the quality of care for people with CF. The CF Foundation has received reports of health problems in people with CF when an enzyme product was used that did not work as it should. By requiring enzymes to be consistent in their formulation and to work properly, people with CF can be assured that they are getting the right amount of enzymes for their digestive and nutritional needs.

How can my child or I participate in the clinical trial of a particular enzyme product?

The clinical trials for enzymes will be designed soon. You can ask your CF care team about participation in clinical trials of enzymes or other drugs. It is critical for people with CF to participate in clinical research to help with the discovery of a drug or drugs that will improve the quality of life for people with CF. Information about CF clinical trials is regularly updated on the CF Foundation Web site at www.cff.org.

Will I still be able to buy the enzymes that my child or I take now?

The enzymes you or your child currently are taking still should be available for the next four years. However, the maker voluntarily can remove its enzyme products from the market before that time if they so choose. After four years, if the FDA approves your enzyme, it will remain available. However, if the maker decides not to apply for FDA approval or if the enzyme does not receive approval, your doctor will not be able to prescribe it and will work with you to prescribe a different enzyme.

How do I know which enzyme is best for my child or me?

The amount of enzymes needed for good nutrition varies from person to person. Your CF doctor will work with you to decide which enzyme is best for you or your child. The enzyme product given to you from your pharmacist may be different than that prescribed by your doctor. To minimize the possibility that you are getting a different enzyme than your doctor prescribed, ask your doctor to write "Do Not Substitute" on the prescription. Ask your doctor if you have questions about what was given to you by a pharmacist.

What happens if my insurance will not cover a different enzyme?

Once the FDA approves specific enzymes, we expect insurance will cover them according to your insurance policy. CF Services, the pharmacy subsidiary of the CF Foundation, can help people with CF work with their insurance companies to get enzymes covered; for more information visit www.cfservicespharmacy.com or call 1 (800) 541-4959.

Was the CF Foundation involved in the FDA's decision?

The CF Foundation offered its expertise about CF and pancreatic enzyme supplements to the FDA. We shared our concerns about the variations and inconsistencies in the available pancreatic enzymes. We identified experts who could provide the FDA with more information about the importance of reliable and effective pancreatic enzymes for people with CF. The CF Foundation supports the FDA's decision because it will make enzymes better and help improve nutrition and digestion for people with CF. We will continue to offer our expertise to the FDA to ensure the best health for everyone with CF.

Why is good nutrition important for people with CF?

Although enzymes help people with CF digest their food, there are still many people with CF who need more help with digestion. It is important that people with CF have the energy and fat stores to help fight infection and regain strength. Several studies have shown that good nutrition can have a positive impact on the health of the lungs and the body overall. Without good nutrition, people with CF may not be able to stay as healthy as possible. The CF Foundation has a paper called “Nutrition: Pancreatic Enzyme Replacement in People With Cystic Fibrosis” available on its Web site at http://www.cff.org/living_with_cf/child_focus.cfm or call (800) FIGHT CF.

If the CF Foundation is focusing on enzymes, will this take away from efforts toward a cure?

No, the CF Foundation is looking at all aspects of CF research and care to ensure a better quality of life for people with CF and to find a cure. We take a broad and varied approach in the fight against CF to help ensure the success of our mission. Currently, there are nearly two dozen potential therapies being tested in the lab or in clinical trials. Many of these address the basic defect in the CF cells and could, one day, have a dramatic impact on the lives of people with CF. In the meantime, we continue to look for ways to improve the quality of life for people with CF by testing products to control and lessen the symptoms and complications associated with CF.

For more information, you may visit the FDA’s Web site at <http://www.fda.gov/bbs/topics/news/2004/NEW01058.html>

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