

Food and Drug Administration  
Rockville MD 20857

NOV 12 1998 35 '98 NOV 17 P2:26

- The Honorable Kenneth E. Bentsen, Jr.  
House of Representatives  
Washington, D.C. 20515-4325

Dear Mr. Bentsen:

Thank you for your letter of September 11, 1998 to Dr. Michael A. Friedman, Acting Commissioner of Food and Drugs, commenting on the Food and Drug Modernization Act (FDAMA or the Act) Docket #98N-0339D regarding life-threatening diseases and the drug approval process for them. Docket #98N-0339D pertains to the Food and Drug Administration's (FDA or the Agency) Plan for Statutory Compliance (with FDAMA) and Annual Report. Docket #98N-2067 relates to Section 112 of FDAMA, relating to "fast track" programs to facilitate the development and expedite the review of new drugs that are intended to treat serious or life-threatening conditions. We have submitted your comments to Docket #98N-2067. In addition, on behalf of several constituents who suffer from amyotrophic lateral sclerosis (ALS), you expressed your concern that FDA should give the highest priority to expediting treatments for serious and fatal diseases such as ALS.

For your information, as the sponsor has acknowledged in public meetings, FDA designated Myotrophin, for the treatment of ALS, as a "fast track" product. This reflects the high priority the Agency gives to treatments for serious and fatal diseases such as ALS. As you know, FDAMA requires the Secretary of Health and Human Services to issue a guidance for "fast track" products describing the policies and procedures that pertain to this section of the Act by November 21, 1998. FDA is in the process of developing this guidance. Please be assured that your comments will be considered.

We very much appreciate your interest in this important provision and understand the significance of this provision to patients with serious or life-threatening illnesses. We recognize that Section 112 of FDAMA builds upon the existing accelerated approval mechanism provided for in current FDA

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regulations. In part, Section 112 amends the Federal Food, Drug, and Cosmetic Act by providing explicit statutory authority for FDA to approve a drug based on surrogate or clinical endpoints if applicable, and to require post-marketing clinical studies. This statutory authority contains language similar to FDA's current "accelerated" approval regulations, found at Title 21, Code of Federal Regulations (CFR) Section 314.510, under which approval may be based on substantial evidence of effect on a surrogate endpoint or on a clinical endpoint other than survival or irreversible morbidity. 21 CFR Section 314.510 further provides that applicants granted accelerated approval may be required to "study the drug further, to verify and describe its clinical benefit, where there is uncertainty as to the relation of the surrogate endpoint to clinical benefit, or of the observed clinical benefit to ultimate outcome." If the clinical benefit is not verified, then the accelerated approval can be withdrawn.

We look forward to working with you and all interested parties in the interpretation and implementation of FDAMA. We hope this information has been helpful. If we may be of further assistance, please let us know.

Sincerely,

Diane E. Thompson  
Associate Commissioner  
for Legislative Affairs

cc: Dockets Management Branch  
(Docket No. 98N-2067)

KENNETH E. BENTSEN, JR.  
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BUDGET

Dr. Michael A. Friedman  
Lead Deputy Commissioner  
Food and Drug Administration  
Parklawn Building  
5600 Fishers Lane, Room 15-55  
Rockville, Maryland 20857

Dear Commissioner Friedman:

I am writing to comment on the Food and Drug Modernization Act Docket # 98N-0339D regarding life-threatening diseases and the drug approval process for them.

I urge the Food and Drug Administration (FDA) to expedite treatments for life-threatening diseases especially when no other medical treatments are available. As you know, the Food and Drug Modernization Act includes a provision that requires expedited reviews for drugs used to treat life-threatening diseases. I have been contacted by several constituents who are suffering from Amyotrophic Lateral Sclerosis (ALS), a fatal neurological disease. These constituents are concerned that the FDA is not acting in a timely manner to provide new medical treatments for this disease. I believe the Center for Drug Evaluation and Research (CDER) and the Center for Biologics Evaluation and Research (CBER) should give their highest priority to expediting drug treatments for serious and fatal diseases such as ALS. I also believe it is critical that FDA Advisory Panels should include experts who are treating patients with these diseases.

Thank you for your consideration of my views. If I may be of any further assistance on this or any other matter, please do not hesitate to contact me.

With kindest personal regards,

Sincerely,



Kenneth E. Bentsen, Jr.  
Member of Congress

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No. 98-6907

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Congress of the United States  
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September 11, 1998

CROSS FILE SHEET

FILE NO: 98N-339D/C4

SEE FILE NO: 98D-0267/C5