February 23, 2007

Division of Docket Management (HFA-305)
Food and Drug Administration
5630 Fishers Lane, Room 1061
Rockville, MD 20852

Submitted to http://www.fda.gov/dockets/ecomments

Re: Docket No. 2007N-0005

To Whom It May Concern:

The National Organization for Rare Disorders (NORD) appreciates this opportunity to comment on the FDA's proposal for reauthorization of the Prescription Drug User Fee Act (PDUFA IV).

We applaud FDA's efforts to include the views of its many stakeholders as it prepared this draft. We particularly welcome the FDA and industry's agreement that post-approval drug safety is an area of compelling need for which additional funds can be drawn from user fees.

**NORD's primary concern – of which FDA is well aware – is that PDUFA IV not undercut the *Orphan Drug Act (ODA)*, which has led to the development and approval of more than 300 drugs and biologics for treatment of rare diseases.**

A significant part of orphan drug development is done by smaller and newer companies. Their activities are often constrained by a combination of modest working capital, extremely small treatment populations and reluctance to price their drug at a very high level. User fees hold strong potential to be a barrier to orphan drug development because these fees are most burdensome on those companies that the *ODA* specifically intended to encourage.

Orphan drugs are exempt from application user fees. Because of this and because the "small business waiver" applies only to application fees, we have not considered whether the revised "small business waiver" is problematic. It does hint at an approach and philosophy that – if carried over into product and facility fees – might well be adverse to orphan drug development (i.e., that any company with any revenue stream is prejudged to be capable of paying user fees, regarding of circumstance). Because a company with other products, including other orphan drugs, can pursue orphan drug development without an application fee, it is not an issue for NORD in the context of FDA's current proposal.

Unlike application fees, orphan drugs are subject to both product and facility fees. Under the PDUFA IV agreement, these changes are likely to increase by a considerable amount, sharpening our concern that PDUFA IV may inadvertently undercut the *ODA*.

When an orphan drug has subsequently found larger uses and markets, we have no problem with product and facility user fees being charged. Likewise, we see no reason to provide reduced or waived product or facility fees when an orphan drug, by dint of top-rung pricing, produces substantial revenue for the company involved.

However, these categories do not describe every circumstance under which orphan drugs are developed… and certainly do not describe situations where user fees may be a deterrent for the development of an orphan drug with modest revenue potential. Congress recognized this in the original PDUFA law, when the House Report stated that the "public health" and "barriers to innovation" waivers "will give the FDA sufficient authority to waive fees for orphan drugs." (H.R. Report No. 102-895, at 17, 1992)

We remain concerned that the implementation of product and facility user fees has not been sufficiently friendly to the development of orphan drugs. We intend to continue to explore these issues with FDA and Congress during the current legislative renewal process. We are most concerned that: for small patient populations, the cost of user fees are added to the price of the drug, sometimes making the treatment extraordinarily expensive; and the FDA is defining "small business" differently than the Small Business Administration, thereby precluding "public health" and "barriers to innovation" user fee waivers for businesses that government customarily considers to be "small businesses."

Again, we appreciate this opportunity to comment, and are available for further discussion.

Sincerely,

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