Dear Secretary Geithner and Mr. Bostick:

The Immune Deficiency Foundation (IDF), the national patient organization dedicated to improving the diagnosis, treatment and quality of life of persons with primary immunodeficiency diseases (PIDD) through advocacy, education, and research, has serious concerns regarding Treasury’s pending implementation of the new annual fee on pharmaceutical manufacturers. Our concerns are that the intended protection for orphan drug research and development (R&D) that Congress enacted with respect to the new annual fee for manufacturers of branded prescription drugs under the Patient Protection and Affordable Care Act (PPACA), as amended by the Health Care and Education Reconciliation Act of 2010 will be further diminished. As advocates for individuals with rare diseases who depend upon such drugs are continued research and development of new drugs, we want to ensure that the voice of patients is heard in critical discussions so that unintended consequences do not befall those with special needs.

Primary immunodeficiency diseases (PIDD) occur in patients born with an immune system that either is absent or poorly functioning. There are over 150 different types of PIDD, all caused by genetic (or intrinsic, as opposed to acquired) defects. People with PIDD live their entire lives more susceptible to infections—enduring recurrent health problems and often developing serious and debilitating illnesses if not treated appropriately. Although many PIDDs are either present at birth or have symptoms that occur in early childhood, some patients may develop a primary immunodeficiency disease as adults, even later in life.

We were pleased to see that § 9008(e)(3) of the PPACA excludes sales of “orphan drugs” from the definition of “branded prescription drug sales” for purposes of calculating the annual fee for manufacturers of branded prescription drugs. However, we understand that Treasury appears poised to interpret this provision in a manner that would deny the new law’s fee exemption to any investment in an orphan drug if the drug has already been approved by FDA to treat an orphan or non-orphan disease. We believe this would be a misinterpretation of the statutory language that would undermine the intent of Congress—and, critically, that would seriously reduce the incentive to invest in R&D on existing drugs and develop them for use in orphan populations. This result would be devastating to patients with rare and orphan diseases, such as PIDD, whose hope and health depend upon the continuation of ongoing
incentives that spur further development and improvement of therapies for diseases that affect very small patient populations. We therefore urge you to adopt an interpretation of § 9008(e)(3) that gives full effect to the text of the statute and to the long-standing Congressional policy to foster the development of orphan treatments.

Thank you for considering this important issue for patients with rare primary immune deficiency diseases and other rare and orphan disorders. On behalf of these individuals, we urge you to interpret the orphan drug exemption in a manner that does not discourage or undermine the incentives of the ODA and long-standing Congressional policy to support orphan drug R&D. Please preserve the existing incentives to invest in R&D to develop new and improved orphan drug therapies on which patients with rare diseases across the country depend. Please adopt the interpretation that if the most recent FDA approval of a drug is for the treatment of an orphan disease, the treatment qualifies for an exemption from the new annual fee.

Attached to this letter is a more detailed review of our position on this issue. If you have any questions, or if we may provide you with additional information on this very important issue, please do not hesitate to contact me.

Sincerely,

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cc: The Honorable Kathleen Sebelius
Secretary, U.S. Department of Health and Human Services

J. Mark Iwry
Senior Advisor to the Secretary, Deputy Assistant Secretary for Retirement and Health Policy
U.S. Department of the Treasury

Helen H. Morrison
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U.S. Department of the Treasury
Implementation of the Orphan Drug Exemption to the Annual Fee for Branded Prescription Drugs

IDF Comments to the Department of Treasury
October 13, 2010

I. Background

The new healthcare reform laws generally require pharmaceutical companies to pay an annual fee (excise tax) based on the market share of their brand-name prescription drugs. Congress, however, wisely created an exemption for orphan drugs, recognizing that it did not want to discourage or undermine the R&D incentives created by the Orphan Drug Act (ODA). In enacting the exemption from this fee for orphan drugs, and in its earlier passage of the ODA and an R&D tax credit, Congress expressed its strong support for the development of treatments for orphan diseases, precisely because it understands that the size of orphan disease populations are so small that, without Congressional support, orphan diseases will simply not be studied, and effective treatments will not be developed.

In enacting these critically important provisions, Congress acknowledged that both the initial development of orphan drugs and important improvements to these therapies require extraordinary investments in both financial and scientific resources. Because of the significant costs associated with developing treatments for orphan diseases, and the crucial role they play in promoting public health, Congress correctly concluded that the steps it took were necessary to ensure that patients with rare and life-threatening diseases would benefit from new and important orphan drug treatments.

II. Based on the Statutory Text and Strong Public Policy Considerations, Treasury Should Interpret the Statute Such That the Orphan Exclusion Applies So Long as a Product’s Most Recently Approved Indication Was an Orphan Indication

Despite the well-established, long-term Congressional policy to foster the development of orphan treatments, the Treasury Department appears to be developing an extremely narrow interpretation of the orphan drug exemption from the new annual fee. In essence, Treasury seems to construe the healthcare reform law as protecting an orphan drug only where the original FDA-approved use (indication) is for an orphan use and there is no subsequent indication, whether that subsequent indication is an orphan one or a non-orphan one. We fear that this interpretation of the law, in addition to being inconsistent with Congressional policy, will inevitably undermine the effort to produce new and improved orphan drug therapies. As explained further below, the more appropriate interpretation, based on both the statutory text and strong public policy considerations, is that the exclusion applies only if the most recently approved indication is an orphan indication.

PPACA section 9008(e)(3) creates the orphan drug exemption to the annual fee wherever a drug has received an R&D tax credit under section 45C of the Tax Code. In an apparent effort to balance the need for raising revenue against the need to foster orphan drug R&D, section 9008(e) seems designed to terminate the orphan drug exemption if, after the approval of the orphan drug, FDA also approves that same drug to treat a non-orphan disease.

IDF understands this “balancing” policy that terminates the orphan drug exemption in certain situations. If an FDA orphan approval is followed by an FDA non-orphan approval, there is some logic to
removing the exemption. But if, thereafter, the drug receives an additional orphan approval, the exemption should apply again. Similarly, if a drug’s first approval is a non-orphan one, a subsequent orphan approval should qualify the drug for the exemption. In other words, if the most recent approval is an orphan approval, the exemption should apply. If not, manufacturers will be discouraged from pursuing promising new and improved orphan drug therapies.

Significantly, both the text of section 9008(e)(3) and important policy considerations support this common-sense approach to encouraging the development of orphan drugs. Under the narrow interpretation that Treasury apparently is considering, once a drug is approved to treat a non-orphan disease, it can never qualify for the fee exemption, even if (1) the company invests in R&D to develop a treatment for an orphan disease, (2) those R&D expenses qualify for the R&D tax credit, and (3) FDA approves the drug as an orphan drug. Under Treasury’s approach, it seems that even a drug whose only indication is an orphan one will lose the fee exemption upon the approval of a second orphan indication. We are deeply concerned that such an interpretation of a law designed to protect orphan drug development will, in fact, inevitably stifle those drug development efforts.

Finally, and importantly, PPACA section 9008 dictates the total amount that will be collected each year through the annual fees, meaning that the interpretation we urge you to adopt will not in any way affect the total revenue Treasury receives from the annual fees, regardless of how many products are excluded under the “orphan” exemption. It is a budget-neutral approach. The only difference would be which manufacturers pay which proportion of the fixed amount. Logical policy reasons—and the plain text of the statute—support an interpretation whereby manufacturers who have recently invested in orphan drug R&D are able to exempt sales of a drug that has received orphan designation and approval.