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Division of Dockets Management  
U.S. Food and Drug Administration  
Department of Health and Human Services  
Room 1061, HFA-305,  
5630 Fishers Lane,  
Rockville, MD 20852

### **CITIZEN PETITION**

The undersigned (the “Petitioner”) submits this Citizen Petition under the provisions of section 21 CFR § 10.30 to request that the Commissioner of Food and Drugs add Sickle Cell Disease, a Neglected Tropical Disease that impacts a majority African American population in the US, to the Neglected Tropical Disease Priority Review Voucher List. The basis for this request is discussed below.

#### **A. Action Requested**

The Petitioner requests that the Commissioner of Food and Drugs add Sickle Cell Disease, a Tropical Disease that impacts a majority African American population in the US, to the Neglected Tropical Disease Priority Review Voucher List.

There is an urgent need for new treatments for Sickle Cell Disease, and for patients in Africa to gain access to the most basic of treatments, including Hydroxyurea,

The Petitioner also requests that the Commissioner of Food and Drugs adjust the title of the list so that it is more in line with the WHO List; the WHO list does not include the word *Infectious*. Sickle Cell Disease has recently been added to the *Access to Medicines Index* list of diseases that should be prioritized. *Access to Medicines Index* is supportive of this effort to add Sickle Cell Disease to FDA’s Neglected Tropical Disease PRV List. Alternatively, the Petitioner requests that the Commissioner of Food and Drugs create a new Priority Review List for diseases like Sickle Cell Disease that impact patients from predominantly under-served, and marginalized populations.

## **B. Statement of Grounds**

Sickle Cell Disease impacts approximately 20 million patients worldwide (Reference NIH website), most of them residing in Africa. Approximately 250 million people carry the gene for Sickle Cell Disease worldwide (Sickle Cell Disease Foundation US website).

Sickle Cell Disease qualifies to be added to the Neglected Tropical Disease PRV List. A summary of the basis for inclusion of Sickle Cell Disease to the Neglected Tropical Disease PRV List is presented below and is discussed in much greater detail in the supportive document attached to this Citizen Petition.

1. The Neglected Tropical Diseases Priority Review Voucher list was created because, in the words of the FDA, *“much of the global burden of disease falls on populations who lack the resources to develop, encourage development of, or purchase disease preventions or treatments. For this reason, many of the diseases afflicting these populations do not receive the same level of innovation investment as diseases afflicting wealthier or more empowered populations”* [Ref: Federal Register 0567].
2. When a disease is on the list, it incentivizes pharmaceutical and biotech companies to develop novel and much needed treatments for it. Companies invest on the front end, and receive a reward on the backend in the form of a voucher that can be used to expedite the review of another, much more profitable, new medicine.
3. Neglected Tropical Diseases Priority Review Vouchers will provide the motivation for small biotech and pharma companies to invest in the Neglected Tropical Diseases on the list.
4. Sickle Cell Disease is undoubtedly a Tropical Disease because of the population in which it is predominant. Nevertheless, PRDPF! has gone through the exercise of demonstrating that it is also a Tropical Disease according to the test required by FDA. The following criteria are met in this respect:
  - a. Sickle Cell Disease disproportionately impacts a poor and marginalized population, both in the US, and outside the US. The African American population has been demonstrated in numerous studies to be a marginalized and disadvantaged population in many respects. It also suffers from health disparities, again which are well studied, and documented.
  - b. There is no significant market in developed markets.
  - c. There is no significant market, indirect or otherwise, in developing markets.

5. Given the limited investment in new treatments when compared to diseases like Cystic Fibrosis, it is readily apparent that Sickle Cell Disease is a Neglected Tropical Disease
6. Sickle Cell Disease is not an *Infectious Disease*. We believe the title of the list should be amended to remove the word “Infectious”. This would bring it in line with the WHO List of Neglected Tropical Diseases.
7. Alternatively, FDA should create a new Priority Review List for diseases like Sickle Cell Disease that impact patients from predominantly under-served, and marginalized populations.
8. To leave the situation as it is, would mean patients with Sickle Cell Disease would be even more disadvantaged than they already are, by this debilitating disease.
9. The DALY numbers for Sickle Cell Disease show at least a comparable estimate when compared to Chagas Disease, and the other diseases on FDA’s Neglected Tropical Disease Priority Review List. In fact, for many of the countries, particularly in Africa, the DALYs show a far worse picture than for the diseases already on the list.
10. Sickle Cell Disease remains off the list, perhaps because a request for it to be added to the list has never been submitted. We are very pleased that Putting Rare Diseases Patients First!® has submitted the application that will address the needs of Sickle Cell Disease patients, in the US and in the rest of the world.

Please see the attached position paper that provides the detailed support for this Citizen Petition.

### **C. ENVIRONMENTAL IMPACT STATEMENT**

The Petitioner requests a categorical exclusion from the requirement to prepare an environmental assessment under 21 C.F.R. § 25 .31(a). To the best of Petitioner's knowledge, no extraordinary circumstances exist that would warrant the preparation of an environmental assessment.

### **D. ECONOMIC IMPACT STATEMENT**

As provided in 21 CFR §10.30(b), the Petitioner agrees to submit economic impact information if requested by the Commissioner of Food and Drugs following review of the Petition.

**E. CERTIFICATION**

The undersigned certifies that, to the best knowledge and belief of the undersigned, this Petition includes all information and views on which the petition relies, and that it includes representative data and information known to the Petitioner, which are unfavorable to the Petition.

Respectfully submitted,

Lorna Speid, Ph.D.  
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Putting Rare Diseases Patients First!®

Cc: David L. Rosen, BS Pharm, JD  
Foley & Lardner LLP