



## Alpha-1 Foundation

### Transmissible Spongiform Encephalopathies Advisory Committee

#### Statement Regarding the suitability of blood donors who have lived or traveled in various countries based on recent information concerning new-variant Creutzfeldt-Jakob disease (vCJD) and bovine spongiform encephalopathy (BSE) in those countries.

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Alpha<sub>1</sub>-Antitrypsin Deficiency (Alpha-1) is a genetic disorder that results in devastating and often fatal lung and liver disease. Individuals with the pulmonary destruction of Alpha-1 demonstrate precocious onset of pulmonary emphysema with symptoms including shortness of breath particularly on exertion, cough, wheeze, repeated lung infections, and sputum production. Therapy consists of augmentation of the Alpha<sub>1</sub>-Antitrypsin protein via weekly infusions of a plasma derivative. In the absence of therapy, the pulmonary emphysema of Alpha-1 tends to be relentlessly progressive, often leading to premature respiratory death in affected individuals. It is estimated that untreated individuals can have their life expectancy reduced by 20 or more years.

With onset between the third and fifth decades of life the pulmonary impairment can cause disability leading to loss of employment, frequent hospitalizations, family disorganization, and the suffering known only to those unable to catch their breath. Lung transplantation, with all its associated risks and costs, is the most common final option.

The Alpha-1 Foundation is a not-for-profit organization founded in 1995 to promote research towards a cure for Alpha-1. The majority of the Board of Directors is either diagnosed with Alpha-1 or is a family member of an individual with Alpha-1. To date the Foundation has funded over \$9 million in a broad range of research grants and awards. The Foundation's Public Policy program is aimed at increasing research funding, addressing product shortages, ensuring blood safety, development of new therapies and advocating for access to care, which includes insurance reimbursement and genetic discrimination.

With recognition of the widening European incidence of bovine spongiform encephalopathy (BSE) and its anticipated risk for variant Creutzfeldt-Jakob Disease (vCJD) in humans, the Alpha-1 Foundation is supportive of efforts to ensure the safety of the US blood supply and plasma derivatives.

\* Diagnosed alpha<sub>1</sub>-antitrypsin deficient

+ Diagnosed family member

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Currently only a single therapeutic agent exists and as a representative of those citizens suffering the consequences of Alpha<sub>1</sub>-Antitrypsin Deficiency the Foundation expresses extreme concern that this life-preserving medication continues to be in critically short supply. As a consequence, the Foundation believes that efforts aimed at providing a theoretical increase in the safety of the US blood supply must be combined with consideration of the risks of reducing the availability of blood and plasma products. Clearly, the evaluation of any plan to further increase donor deferrals must include consideration of the risks to the plasma user community as well as mechanisms to expand blood and plasma donation in order to avoid further compromise to the availability of blood and its derivative medications.

The Foundation regards the proposed expansion of the donor deferral policy by exclusion of donors at risk of vCJD on the basis of foreign residency and travel as inefficient and it makes weighing of issues related to risk-benefit more difficult.

The Foundation recommends that the focus be an increase in funding for research in the areas of testing methodologies for the vCJD infectious agent, studies of vCJD transmission and infectivity, and evaluations of methods for inactivation/segregation of the vCJD infectious agent during blood product fractionation.

The Foundation also cautions that efforts to ensure safety may be misconstrued by the lay public and actually reduce the willingness of individuals to donate blood and plasma, as well as alarming donors who are deferred.

Finally, the Foundation encourages funding to increase health surveillance of the population for vCJD, recommends the evaluation of a blood safety compensation system, and suggests education to reassure the US public of the safety of blood donation and the blood supply.

Thank you.