A Review of Human Prion Disease Treatment
The Past, Present & Possible Future

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Everyone at this conference, either from a personal or a professional viewpoint, understands the awful nature of human prion disease and, therefore, the desire for effective treatment.

There have been many case reports and small studies of various treatments over the last 30 years. A systematic review of these, from 1996 to 2007, along with reports describing the natural history of prion illnesses, has been published recently (Stewart et al Neurology 2008; 70:12761-1281). The results of this review will be presented. In summary: 140 publications have described the disease in around 9000 patients in 32 countries, with varying durations of untreated illness, and 33 publications have described treatments given to 149 patients. Many of the treatment reports have been unsatisfactory in some way and must be interpreted cautiously. The described treatments involve 14 drugs, with relatively detailed data on only 4 (Amantadine, Quinacrine, Pentosan Polysulphate, Flupirtine).

Another recent publication has described the experience with Pentosan Polysulphate (PPS) in the UK (Bone et al, European Journal of Neurology 2008, 15:458-464). This suggests that intracerebroventricular PPS may slow disease progression, at least in variant CJD.

The UK Prion-1 Trial of quinacrine has closed and results are awaited. Currently, there are other treatment trials with quinacrine and other drugs being undertaken in a few countries, including the USA.

There are a number of problems in designing and managing treatment trials in human prion disease. These include the relative rarity of prion diseases and the typically relatively advanced stage of illness at diagnosis, especially in the commonest disease of sporadic CJD. In addition, there are problems in how to assess partial efficacy of treatments in the face of significant neurological disability. The lessons of the past should inform any future plans.