DISEASE MODIFYING TREATMENT IN MULTIPLE SCLEROSIS

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Multiple sclerosis (MS) is the most common cause of physical disability in young adults. The "Holy Grail" of MS research is to find a treatment that stops relapses, halts progression of the disease, and induces recovery from any disability. At the moment a number of treatments have been investigated that have aimed at a target some way short of this, slowing progression of the disease. The interpretation of these studies has been controversial with debates over clinical efficacy often being sidelined by issues of health economics.

In this supplement we are not going to review the trials of drugs aimed at disease modification, as this is still an evolving field that is still being widely discussed in the neurological literature. Neurological trainees need to familiarise themselves with the primary trial data that underpins this debate, and the main trials are listed below. We have also included some of the papers discussing the health economics effects of these drugs.

To help evaluate these studies it might be worth considering some of the features of an idealised and unfortunately impossible trial of an intervention in a chronic disabling disease.

- The trial would be very large, sufficiently powerful to minimise any effect of chance in what is a variable disease, and to allow multiple prospectively defined subgroups to be studied.
- All eligible patients would be entered. Randomisation would be stratified according to easily recognisable clinical groups with parallel placebo treated control groups.
- The trial duration would be appropriate to the natural history of the disease. As MS is a disease which progresses over decades one would expect this to be reflected in the trial's duration.
- All patients would complete the study or those who failed this would be comprehensively documented with a watertight intention to treat analysis.
- Patients and assessors would be blind to the treatment. The study would include groups on combinations of potentially synergistic treatments.
- The outcomes would be as objective as possible, for example death or wheelchair use and disability scales (which would need to be improved), with as few surrogate markers, such as laboratory results or MRI, as possible.
- A perfect reliable and valid quality of life measure would be used.
- The study would include an appropriate prospective health economic evaluation rather than a post-hoc analysis. There would be appropriate financial control groups so that any effect of an expensive drug on quality of life could be compared with spending the same amount on other treatments or simply in additional financial support for the patient.
- The study would be designed, conducted and reported by researchers with no financial or other interest in the outcome of the study.
- And, of course, the results would be available now.

DRUGS OTHER THAN INTERFERONS AND GLATIRAMER ACETATE

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Interferons and glatiramer acetate

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- **PRISMS Study Group**. Randomised double-blind placebo controlled study of interferon-1a in relapsing/remitting multiple sclerosis. *Lancet* 1998;**352**:1498–1504.

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MS: pharmoeconomic

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- **Schwind SR**, Bever CT. The cost of delaying treatment in multiple sclerosis: what is lost is not regained. *Neurology* 2001;**56**:1620.