The Association of British Neurologists welcomes recent research into autologous haematopoietic stem cell treatment of multiple sclerosis. Despite many advances in the treatment of this disease, for some people it is disabling and life-limiting. New therapies which combine high efficacy with acceptable side-effects are certainly needed. However, as a recent commentary put it “the jury is still out regarding the appropriateness and indications of haematopoietic stem cell treatment for multiple sclerosis” (Soldán & Weinshenker, 2015).

Autologous haematopoietic stem cell treatment should only be seen as a potential immunotherapy in multiple sclerosis; there is no suggestion that these stem cells are reparative. Therefore there is no rationale for its use in people with progressive multiple sclerosis. As the experience from Sheffield shows, some people with relapsing-remitting multiple sclerosis anecdotally report considerable benefit from autologous haematopoietic stem cell treatment. Similar successes with this treatment have been reported from many small open-label studies since the early 1990s, but these are not powered to identify a true clinical benefit (Snowden 2012). However, there is a treatment-associated mortality. This has decreased from 6% to approximately 1.5%, probably because of the reduced intensity of contemporary immunoablation regimens (some of which include alemtuzumab, itself a licensed treatment of multiple sclerosis). The only controlled trial to date, the small (n=21) Autologous Stem cell Transplantation International MS Trial, demonstrated that transplantation was more effective at reducing new MRI lesions than mitoxantrone (Mancardi 2015). But larger controlled studies are needed to explore the optimum transplant conditioning regime, and its relative efficacy and safety compared to other licensed therapies. Professor Sharrack and others are participating in one such trial (ClinicalTrials.gov Identifier: NCT00273364).
