Oral presentation ECTRIMS 2019

Title: How to design RCTs for SPMS

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Abstract

It is an exciting time for the treatment of progressive multiple sclerosis (PMS) with three agents available in various countries: ocrelizumab, siponimod and cladribine. These follow an anti-inflammatory approach but of course address only one part of the pathobiology. Neuroprotection and remyelination remain as outstanding items, which means that further clinical trials are mandated. This talk will briefly consider recent trials in secondary progressive multiple sclerosis (SPMS) before going on to discuss the principles of how to design a randomised controlled trial (RCT) at phase 2 and 3. Particular consideration will be given to: cohort characteristics; outcomes to be measured; how to model the associations; and power. Some different trial designs will be discussed.