THE PLACE OF SURROGATE ENDPOINTS IN THE DEVELOPMENT NEW NEUROLOGICAL MEDICINES

P. Feldschreiber
UK
P.Feldschreiber@4newsquare.com

This paper examines the potential regulatory acceptability of biomarkers as surrogate end-points in the assessment of clinical efficacy and safety of new treatment for neurological and psychiatric pathologies. There is increasing pressure from the pharmaceutical industry for the regulator to facilitate the accelerated approval of new medicines by the acceptance of shorter and smaller clinical trial programmes. To this end the use of biomarkers as surrogate endpoints together with 'efficient' clinical endpoints would be majorly helpful in this endeavour. We review the categories of biomarkers as surrogates and in particular attempt to suggest criteria which would predict there regulatory acceptability for new neurological and psychiatric medicines. There have been a number of literature reviews and meta-analyses of clinical trials and investigations of biomarker endpoints in the areas of ischaemic brain injury, neurotrauma and neurodegenerative disease, multiple sclerosis, advanced metastatic brain tumours and the dementias, including the validation of neuroproteomic techniques. On reviewing this literature base we conclude that prognostic value is the most important prerequisite for surrogacy. Ideally prognostic value, biological plausibility and effects of therapeutic intervention should all be considered holistically on their effect on risk benefit.