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A Novel SCI Adaptive Trial Platform from Pragmatic to Randomized Controlled Clinical Trials: Accelerating Evidence for Disease Modifying Interventions (Sponsored by Scope)

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A number of promising disease-modifying therapeutic interventions have been identified and are awaiting investigation in human subjects living with spinal cord injury (SCI). Unfortunately, insights into therapeutic concepts have been incremental at best. Reasons for that are multifactorial. Major obstacles towards the expedited investigation of promising therapeutic interventions are the complexities and requirements of randomized controlled clinical trials (RCTs) and the often times unpredictable implementation steps of early clinical trial phases. In order to address logistics required for RCTs, pragmatic clinical trials (PCTs) (i.e., low-intervention clinical trials) have been proposed to investigate treatments with existing regulatory approval. The additional diagnostic or monitoring procedures of PCTs pose only minimal additional risk compared to normal clinical practice. PCTs are often used together with patient registries to better understand the effectiveness of interventions in real-world settings. Multicenter SCI patient registries such as the European Multicenter Study about Spinal Cord Injury (EMSCI) and the North American Clinical Trials Network (NACTN) do not only analyze the natural course of recovery, but in addition have served as platforms for interventional clinical trials including RCTs and PCTs. The aim of this workshop is to bring together complementary expertise to discuss the requirements and potential of existing SCI patient registries (EMSCI and NACTN) to serve as clinical trial platforms to test a number of therapeutic interventions, including running both PCTs and RCTs simultaneously. The Healey ALS Platform trial represents an excellent example of a clinical network which has successfully run concurrent RCTs incorporating adaptive trial design features. Requirements and challenges for such a platform will be presented. An example of an ongoing pragmatic/low-intervention clinical trial will be given to better understand the intricacies and benefits of study designs beyond RCTs. Finally, a brief overview will be provided to consider candidates for therapeutic interventions and their state of readiness for PCTs or RCTs. Agenda: SCOPE intro (Hsieh), Background/overview of alternative trial designs (Jones), EMSCI – from a data registry towards a clinical trial platform (Weidner) NACTN – incorporating the early phase after spinal cord injury (Guest) Approaches for target selection: Wishful thinking or justified hope? (Schwab) Pragmatic clinical trials (Darsaut). Learning Objective 1 Assess capabilities of SCI patient registries to serve as clinical trial platforms Learning Objective 2 Analyze the potential of clinical trial platforms to expedite clinical translation in SCI Learning Objective 3 Discuss drug candidates for randomized controlled versus pragmatic clinical trials

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