Developing Performance Outcome Measures for Use with Regulatory Agencies: The Aging In Motion (AIM) Coalition Experience

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AIM is a diverse group of patient, caregiver, health and aging organizations working together to:

- Press for greater levels of research and innovation around sarcopenia and age-related functional decline
- Raise awareness of sarcopenia and its impact in the community
- Raise awareness of potential for behavioral or pharmacologic interventions to improve physical function and quality of life for the elderly
- Advance consensus among clinicians and regulators on outcome measures for functional decline and effectiveness of potential therapies or treatments
AIM Application for Qualification of Gait Speed and SPPB as Outcomes (1)

- 2012 – Letter of Intent - primary outcomes of clinical trials aimed at improving or preserving functioning in older persons

- FDA Response - target the instrument or instruments to a particular disease or condition
2013 – 2\textsuperscript{nd} LOI – focus on lower-extremity functional decline due to loss of muscle mass and strength as the target condition

FDA Response – LOI accepted and invited to move to next step and submit initial briefing package, with multiple suggestions, including:

- Encourage you to identify condition-specific patient populations
- Consider the causal pathways through which disease and treatment affect performance on the tests
- It will be important to establish clinically relevant connections between the SPPB and related activities of daily living measures, providing a clear linkage of patient-centered benefit to the performance outcome
AIM Application for Qualification of Gait Speed and SPPB as Outcomes (3)

- 2014/15 – Initial Briefing Package –
  - (1) hip fractures
  - (2) status post elective total hip arthroplasty
  - (3) intensive care unit-acquired weakness
  - (4) sarcopenia of aging with reduced mobility

- FDA Response –
  - We are concerned that it may be difficult to develop and implement a single lower extremity function assessment with applicability across these multiple populations
  - In order to gain a better understanding of how pain or other factors may affect the assessment of lower extremity function related to strength and muscle mass, we recommend that you perform qualitative research to include patient input from each target population
AIM Application for Qualification of Gait Speed and SPPB as Outcomes (4)

- 2015 – Meeting with Qualification Review Team
  - Decision to limit application to hip fracture recovery
  - Performance Outcome Measures (PerfOs) are new territory for the FDA and therefore more compelling evidence that PerfOs represent important change in a patient’s condition are required and the application of qualitative data for this purpose is recommended.
AIM Application for Qualification of Gait Speed and SPPB as Outcomes (5)

- 2016 – Request from AIM to QRT to consider alternatives to qualitative research to validate performance measures

- Proposed analyses of data from 3 types of studies
  - Post-fracture observational studies
  - Cohort studies with selection of participants who suffered a fracture
  - Clinical trials of rehabilitation in hip fracture