CONCLUSIONS

Data from this global, cross-sectional, mixed-methods study involving PWSCA (convenience sample) highlight the significant burden that gross motor challenges (including loss of inguinal activities, impaired mobility, lack of balance, and falls) have on QOL.

Mean summary scores for physical and mental components of the SF-36 were numerically lower in PWSCA across all SCA types, and lowest in P3CA3, compared to the general population.

PWSCA were also most likely to be wheelchair-bound.

In follow-up surveys, 53 of 70 participants (75.7%) reported experiencing at least 1 fall during the past year. Among PWSCA, 81.2% experienced at least 5 falls in the past year. Most participants indicated that fear of falls affected their daily lives.

The majority of participants reported that they would find an approved medication that slowed or stabilized the progression of SCA to be extremely meaningful. Furthermore, most PWSCA and P3CA6 rated falling as an outcome of an approved therapy extremely important.

“…If I was not so fearful of falls (and the potential damage that the SCA to be approved medication that slowed or stabilized the progression of SCA may cause), I would open up a steadily closing door in my activities.” (PWSCA3)

“…To have medication availability would improve the life of myself and my family. I constantly worry about the future. We are not able to plan because I don’t know how my health will be.” (P3CA6)

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Table 1. Participant Attrition

Table 2. Demographic Data and SF-36 Scores for Study Participants Who Completed Phases 1 and 2

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Figure 3. Number of Falls by SCA Type in the Past Year

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