INTRODUCTION

- Slow vital capacity (SVC) is often used to evaluate ventilatory function in patients with amyotrophic lateral sclerosis (ALS)1 2.
- However, participation in clinical trials requiring frequent in-clinic visits can prove challenging for patients with ALS, leading to a desire to identify remotely performed outcome measures.
- A portable spirometer may allow SVC to be measured at home, thereby reducing the burden of participating in a clinical trial.
- In an ongoing phase 2 clinical study, FORTITUDE-ALS, we evaluate SVC measured by patients at home as well as in the clinic.

OBJECTIVE

- To assess the reliability of SVC measured at home (hSVC) using SVC measured in clinic (cSVC) as the standard in a phase 2 trial evaluating riluzole in patients with ALS.

METHODS

FORTITUDE-ALS: Patients are trained on Day 1 to use portable home spirometers (GoSpiré®, Monitreat Therapeutics, Inc., Dublin, OH) to measure hSVC weekly.
- GoSVCs are measured at screening, Day 1, and Weeks 2, 4, 8, and 12, and the follow-up visit and are paired with cSVC measurements conducted within a 7-day window. No baseline measurement was made for hSVC.
- Outliers that are lower than -0.5 times or greater than 1.5 times the highest acceptable cSVC are displayed but have been excluded from the analysis.
- The Pearson correlation coefficients between hSVC and cSVC are calculated overall and by visit. The differences between the two measures are analyzed using a paired t-test.
- To evaluate the utility of hSVC, change from baseline in percent predicted SVC measured at home and in clinic have been compared by visit, using cSVC prior to the first dose of study drug as baseline.

RESULTS

- The current compliance rate of hSVC measures is approximately 70%. While the expected total number of hSVC tests is 16 per patient for those who have completed the study, the average number of tests done is 9.2.
- A survey was designed to better understand the lower than expected compliance rate (Table 1).
- Responses were collected from 346/65 sites (52.3%).

Table 1. Site survey to better understand compliance rates

| Item                                                                 | Percentage of Sites (%)
|----------------------------------------------------------------------|-----------------------
| Plays a role in why patients are compliant (defined as performing at least 75% of tests) (Top 3 reasons) | 1. Motivated patient (75.5%)
|                                                                      | 2. Patient not feeling well (72.9%)
|                                                                      | 3. Written instructions (41.8%)
| Plays a role in why patients are noncompliant (Top 3 reasons)        | 1. Spirometer and tablet won’t sync (55.9%)
|                                                                      | 2. Tablet won’t connect to Wi-Fi (32.7%)
|                                                                      | 3. Patient forgets (52.3%)

Free text themes for obstacles to use:
- Machines are not user-friendly
- Solutions not always provided by tech support
- Testing may be daunting for some patients
- Seen as an added burden for patients and caregivers, particularly for the patients with more severe disease

- SVC and percent predicted SVC measured at home and in the clinic were significantly correlated (Figure 1).

CONCLUSIONS

- The current compliance rate of hSVC is suboptimal.
- Although the correlation between hSVC and cSVC is good, there is a significant discrepancy between them, though this discrepancy decreases slightly over time. Differences in percent predicted SVC change were more than 10 percentage points.
- There was no significant difference in percent predicted SVC change from baseline between patients who used a home spirometer and those who did not (baseline) (Table 3).
- The frequency of home spirometer use did not significantly impact the measurement of percent predicted SVC.

References

- No personal information is stored.

Table 2. Change from baseline in percent predicted SVC measured in the clinic versus home

<table>
<thead>
<tr>
<th>Parameter</th>
<th>Predicted SVC percentage points</th>
<th>Baseline</th>
<th>Week 2</th>
<th>Week 4</th>
<th>Week 8</th>
<th>Week 12</th>
<th>Follow-up</th>
</tr>
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<tbody>
<tr>
<td>SVC measured in clinic</td>
<td>92.08±0.69</td>
<td>90.05±0.75</td>
<td>91.12±0.74</td>
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<td>SVC measured at home</td>
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<td>85.42±0.75</td>
<td>86.49±0.73</td>
<td>87.55±0.72</td>
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Table 3. Change from baseline in percent predicted SVC measured in the clinic in patients who used a home spirometer (user) and those who did not (non-user)

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Figure 1. Relationship between SVC in liters measured at home vs in the clinic.

Figure 2. Differences between SVC (liters) measured in the clinic and at home, (A) all data, (B) excluding outliers (note difference in scale of x-axes).

Figure 3. Difference in SVC measured in the clinic and at home over time.