Results We were able to define 4 discrete clusters of patients based on 9 routinely collected clinical variables using data from 537 patients and 12200 encounters. Lung function outcomes were not used to define the clusters, however, there was a distinction between the different clusters, such that the cluster with the poorest outcomes also had the worst lung function. The cluster with the poorest outcomes also had the greatest risk of hospitalisation and pulmonary exacerbation, which suggests that the approach correctly identifies patients with a more severe disease phenotype. The results were consistent in the GOSH clinical data.

Conclusion Four clusters of pediatric CF patients were identified with corresponding differences in clinical characteristics and outcomes. Future work will identify risk factors for transitioning to a severe disease cluster, and those factors that may improve health outcomes.

INVESTIGATING TEMPORAL CHANGES IN PERCENT PREDICTED FVC AND RULM SCORE IN NON-AMBULANT SMA TYPE III CHILDREN

Spinal muscular atrophy (SMA) type III is a relatively mild form of SMA where a significant proportion lose ambulation during childhood. There is a paucity of studies investigating changes in both the respiratory and upper limb function within this population after loss of ambulation (LOA). The aim of this study is to investigate the change in the percentage of predicted forced vital capacity (FVC) and the change in the revised upper limb (RULM) score in these patients across a 24-month period after LOA. Retrospective analyses were performed on 24 non-ambulant SMA III patients on clinical data collected at two UK centres. Mean age at baseline was 10.9 years (4 to 16). The median FVC percentage predicted score at baseline was 90%. We observed a significant progressive deterioration of 14.7% in FVC over the 24-month period. Data on RULM was available in 16 patients with mean age at baseline of 11.5 years (6 to 16). The mean score at baseline was 30.3. We observed a significant progressive deterioration in upper limb function over the 24 months with a mean decrease in RULM score of 3 and a range from -8 to +1. Using a Wilcoxon signed rank test both results were significant (p<0.05). This study highlights that SMA type III patients demonstrate progressive deterioration in their upper limb and respiratory function even after LOA. Combining correlative data from these assessments may provide insight into clinical progression within this patient population which could help inform for clinical trials and be helpful in managing disease progression expectation for patients.