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 hospitalization 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.

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

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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.

85 AUGMENTING SOFT TOYS FOR SELF-REPORTING WELL-BEING

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It can be difficult for children to express and reflect on how they are feeling to others. As such, we hypothesise that children can use technology to express and reflect on their well-being. Existing research suggests that tangible interfaces offer a potential of being able to help capture well-being in hospitals for children, such as capitalising on young children's attraction to toys and play as a way for children to communicate and process their inner thoughts.

An aim was to develop a prototype to enable children to communicate and express how they are feeling with others. To ensure that prototypes were safe and appropriate, we involved The Young Persons' Advisory Group and the Play team to provide vicarious insights about a child's experiences as part of the design process. This process helped to identify user experience challenges such as when reflecting on the potential for negative experiences. From the insights gained, we developed a system that allows children to augment existing toys to make toys interactive and give children a sense of ownership. There were two key modes of interaction, firstly the soft toy acting as the input mechanism for data to be collected. Secondly, there was a visualisation display which would then render the data for children in an accessible way for them to reflect on.

To ensure that the system was safe and appropriate before children used it, we used a 'proxy' method to evaluate the system with researchers using prototypes in the wild. It was found that by having a two-pronged approach was considered valuable: (i) the tangible nature of collecting input provided a physical experience of being able to capture and reflect on well-being at the same time, and (ii) viewing visualisations from the system had a positive impact on the self-awareness of well-being.

86 PONSETI TECHNIQUE FOR CONGENITAL TALIPES EQUINOVARUS – A PHYSIOTHERAPY LED SERVICE

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Background Congenital Talipes Equinovarus (CTEV) deformity affects 1:1000 new-borns. Since the Ponseti technique became popular, the need for operative intervention beyond tenotomy has reduced. However, mid-long term results remain poorly reported. This prospective case series describes our patient population and outcomes since 2011 with an innovative physiotherapy-led Ponseti service.

Methods A prospective study of CTEV patients attending the Ponseti clinic. Primary outcomes included range of movement, calf circumference and functional status. Parents of children over the age of 5 were asked to complete the Oxford Ankle Foot questionnaire (OxFAQ).

Results Between Feb 2011-Aug 2020, 75 (M=43) patients were treated. Mean age at follow-up was 87.5 months (range 18–157 m). 42 cases were bilateral and thus 117 feet were treated. 45 (60%) were idiopathic and 30 (40%) non-idiopathic.

Of the 75 patients, 50 (67%) underwent tenotomy under local anaesthetic in clinic. Tenotomy rates were higher in non-idiopathic vs idiopathic patients (83% vs 56%) ($P<0.002$).

Mean dorsiflexion (knee extended) in affected feet was 14°. In unilateral cases mean dorsiflexion (knee extended) in unaffected foot vs affected foot was 17° vs 12°. All idiopathic feet were plantigrade.

Abnormal evertor function was seen in 3 of 66 (5%) idiopathic feet versus 22 of 51 non-idiopathic feet (43%) ($p<0.0001$).

Calf circumference discrepancy was higher in unilateral cases versus bilateral (mean 1.3 cm versus 0.6 cm respectively). Unilateral cases undergoing tenotomy had a greater mean circumference difference (1.5 cm) versus those managed without tenotomy (0.7 cm).

43 idiopathic patients were suitable for OXFORD scoring at time of last review (i.e. older than 5 years old). The overall median OxFAQ was 96% with points lost only in the physical scale. Function in non-idiopathic cases was determined by their overall condition.

Conclusion The idiopathic club foot is well treated by a physiotherapy-delivered Ponseti technique with excellent outcomes at 5 yr follow-up both subjectively and objectively.

87 COMPARISON OF FUNCTIONAL ABILITY IN SIBLINGS WITH DUCHENNE MUSCULAR DYSTROPHY

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Early diagnosis is important to optimise management and prolong function in patients with Duchenne Muscular Dystrophy (DMD). There is little research comparing functional outcomes of siblings diagnosed at different ages.

This study aims to analyse the effect of age at diagnosis and prescription of steroids on function in siblings with the same genetic diagnosis of DMD. Functional ability was measured using the North Star Ambulatory Assessment (NSAA) which is a validated outcome measure in ambulant children with DMD.

NSAA data from 24 siblings (48 patients) was collected from their first NSAA assessment at Great Ormond Street Hospital, including all scores until July 2020.

Age matched NSAA scores were collated at 6 monthly intervals (± 3 months) between 5.5–10.5 years in siblings, where one or more NSAA scores could be compared. 17 siblings (34 patients) met the inclusion criteria and were analysed.

Results show that younger siblings were diagnosed on average 2.7 years earlier and started steroids 0.8 years earlier. The median peak NSAA scores were higher in older siblings at ages 5.26–6.25 and 7.26–8.25 years. Between 6.26–7.25 and after 8.26 years, the younger siblings median NSAA scores were consistently higher, although there were small numbers

in each sub-group. Wilcoxon Signed Rank test showed no significant differences between groups.

Analysis of the graph suggests that despite the earlier age at diagnosis, younger siblings did not diverge from the older sibling in the first phase of the disease. Visual inspection of the NSAA score shows that after 8 years old, the younger siblings consistently scored higher values. This suggests that earlier initiation of steroids is likely to have played a role in this outcome, as previously demonstrated in the DMD population. Further research will assess long-term effects of these trends, regarding age at loss of ambulation, and of respiratory insufficiency.

88 THE RELATIONSHIP BETWEEN UPPER AND LOWER LIMB FUNCTION IN A COHORT OF CHILDREN WITH CHARCOT-MARIE-TOOTH DISEASE

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Charcot-Marie-Tooth (CMT) is a progressive disease with clinical signs presenting first in the distal lower extremities. Upper limb function in this population is also affected at a later stage of life but it is poorly researched and little is known about hand function limitations and loss of manual dexterity. The purpose of this study is to investigate the possible relationship between upper and lower limb function in a group of children and adolescents. The CMT natural history study at Great Ormond Street Hospital in London has been collecting longitudinal data of more than 120 children and adolescents with CMT (age range 4 to 21 years). Eighty one children with CMT type 1 (53% CMT1A), 22 with type 2 and 21 with other types of CMT have been assessed up to date. To evaluate upper limb function we used myometry, functional dexterity test and 9 hole peg test; to measure lower limb function the 6 minute walk test, long jump and plantar and dorsiflexion strength tests. In this study we will use correlation analysis to explore the concordance between the upper and the lower limb function. A comparison will be made between children and adolescents with demyelinating CMT and those with axonal CMT, and also individual genotypes, to look if variation in genetic subtype affects individuals in different ways. In our analysis we will assess longitudinally the correlation between upper and lower limb function in children with different subtypes of CMT. If a strong relationship between the two is found in individual conditions, we will assess further the predictive model of loss of function between upper and lower limbs in different genetically defined conditions. This study is a part of a MSc project being undertaken at UCL and a full report and results will be presented later in the year.

89 CHRONIC DISSEMINATED CANDIDIASIS TREATED WITH ADJUVANT CORTICOSTEROID TREATMENT

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