|  |  |  |  |
| --- | --- | --- | --- |
|  |  |  **Historical Controls** | **ILC** |
|  |  |  |  |  |  |  |  |  |  | **6 month Treatment Difference**  | **12 month Treatment Difference** |
|  | **Parameter** | **n** | **1st Measurement Mean** | **Last Measurement Mean** | **Difference within 1 yr** | **n** | **Baseline (±2 mo)** | **6 mo (±2 mo)** | **12 mo (±2 mo)** | **Mean @ 6 mo** | **P-value** |  | **Mean @ 12 mo** | **P-value** |  |
| **2 to < 5 yo** |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |
|  | Height z-score | 245 | -0.98 | -0.83 | 0.15 | 18 | -0.83 | -0.53 | -0.74 | 0.30 | 0.023 |  | 0.09 | 0.602 |  |
|  | Weight z-score | 245 | -1.05 | -0.71 | 0.34 | 18 | -0.85 | -0.75 | -0.74 | 0.10 | 0.159 |  | 0.11 | 0.240 |  |
|  | BMI z-score | 245 | -0.26 | -0.09 | 0.25 | 18 | -0.31 | -0.28 | -0.10 | 0.04 | 0.462 |  | 0.22 | 0.903 |  |
| **5 to <12 yo** |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |
|  | Height z-score | 797 | -0.87 | -0.86 | 0.00 | 49 | -1.04 | -0.85 | -0.85 | 0.19 | 0.033 |  | 0.18 | 0.071 |  |
|  | Weight z-score | 797 | -0.74 | -0.65 | 0.08 | 49 | -0.86 | -0.58 | -0.59 | 0.28 | 0.051 |  | 0.27 | 0.181 |  |
|  | BMI z-score | 797 | -0.24 | -0.13 | 0.11 | 49 | -0.22 | -0.05 | -0.08 | 0.17 | 0.522 |  | 0.14 | 0.850 |  |
| **12 to <18yo** |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |
|  | Height z-score | 826 | -1.14 | -1.15 | -0.01 | 21 | -0.71 | -0.81 | -1.46 | -0.10 | 0.818 |  | -0.75 | 0.205 |  |
|  | Weight z-score | 826 | -1.19 | -1.16 | 0.03 | 21 | -0.71 | -0.86 | -0.69 | -0.16 | 0.226 |  | 0.01 | 0.955 |  |
|  | BMI z-score | 826 | -0.72 | -0.68 | 0.04 | 21 | -0.56 | -0.67 | -0.67 | -0.11 | 0.485 |  | 0.35 | 0.150 |  |

**Supplemental Table 2 - Comparison of Change in Height, Weight and BMI Z-scores for ILC to CFFPR Historical Controls by Age Group**

Patients who had consented to have data entered into the CF Foundation Patient Registry (CFFPR) and were receiving enteral feedings at any time during 2014 were eligible to be in the historical control group. Out of the 1868 patients, 1847 were identified as pancreatic insufficient based on the documented need for pancreatic enzyme therapy (PERT). To be included, patients had to have at least 2 measurements of height and weight at least 6 months apart and not be receiving treatment with ivacaftor. The duration of enteral feedings and the method of nutrient digestion was unknown. Subgroups of patients 2 to 5 years, 5 to < 12 years, and 12-18 years were evaluated and were compared to similar age groups in the ILC program who had full data available