**Supplementary material for Byrne et al., “Cardiac responses in paediatric Pompe disease in the ADVANCE participant cohort,” *Cardiology in the Young***

**Table S2.** Left ventricular mass and systolic blood pressure z*-*scores (mean ± standard deviation) at enrolment, Week 52, and change from enrolment by subgroups

|  |  |  |
| --- | --- | --- |
| Subgroup | LVM z*-*score | SBP z*-*score |
| n | Baseline | Week 52 | Change from baseline to Week 52 | n | Baseline | Week 52 | Change from enrolment to Week 52 |
| Fraction of life on 160L alglucosidase alfa |
| <0.79 | 29 | +0.1 ± 3.01 | −1.1 ± 2.01 | −1.2 ± 2.10 | 24 | +1.2 ± 1.91 | +1.0 ± 1.46 | −0.1 ± 1.76 |
| ≥0.79 | 38 | −0.9 ± 1.45 | −0.9 ± 1.4 | 0.0 ± 1.15 | 31 | +0.8 ± 0.90 | +0.9 ± 0.89 | +0.1 ± 1.11 |
| Baseline ventilation status |
| Invasive ventilation | 13 | −0.8 ± 1.78 | −1.4 ± 1.70 | −0.6 ± 1.63 | 16 | +1.3 ± 1.99 | +1.6 ± 1.05 | +0.3 ± 1.86 |
| Non-invasive ventilation | 6 | +0.2 ± 3.36 | −2.1 ± 0.74 | −2.3 ± 3.12 | 5 | +1.4 ± 0.95 | +0.3 ± 1.27 | −1.1 ± 1.57 |
| None | 48 | –0.4 ± 2.30 | –0.7 ± 1.70 | −0.3 ± 1.40 | 34 | +0.7 ± 1.13 | +0.7 ± 1.07 | 0.0 ± 1.09 |
| Genotype\* |
| Null/null | 6 | –1.0 ± 2.07 | –0.8 ± 1.73 | +0.2 ± 1.91 | 9 | +0.9 ± 0.57 | +1.0 ± 0.98 | +0.1 ± 1.08 |
| Null/missense | 21 | –0.3 ± 1.37 | –0.9 ± 1.21 | −0.6 ± 1.35) | 14 | +1.2 ± 1.31 | +0.6 ± 0.98 | −0.6 ± 1.06 |
| Null/Intronic | 7 | –0.7 ± 1.25 | –0.8 ± 1.45 | 0.0 ± 1.01 | 4 | +1.6 ± 1.04 | +0.5 ± 0.52 | −1.1 ± 0.78 |
| Missense/missense | 19 | +0.2 ± 3.52 | –1.0 ± 2.32 | −1.2 ± 2.40 | 17 | +1.1 ± 1.86 | +1.0 ± 1.41 | −0.1 ± 1.44 |
| Missense/intronic | 8 | –0.9 ± 1.74 | −0.7 ± 1.30 | +0.1 ± 1.31 | 9 | +0.4 ± 1.46 | +1.6 ± 1.15 | +1.3 ± 1.70 |
| Intronic/intronic | 1 | –3.3 | –3.6 | –0.3 | 1 | −0.3 | −0.9 | −0.6 |
| Disease phenotype |
| IOPD | 50 | −0.1 ± 2.39 | −0.8 ± 1.65 | −0.8 ± 1.83 | 44 | +1.1 ± 1.45 | +1.0 ± 1.19 | −0.1 ± 1.49 |
| LOPD | 15 | −1.6 ± 1.54 | −1.5 ± 1.73 | +0.1 ± 1.13 | 10 | +0.4± 1.24 | +0.6 ± 1.03 | +0.2 ± 1.13 |
| Age at enrollment, participants with IOPD |
| <2 years | 11 | +2.1 ± 3.76 | 0.4 ± 2.15 | −1.7 ± 2.86 | 14 | +1.6 ± 1.54 | +1.4 ± 1.33 | −0.1 ± 1.59 |
| ≥2 years | 39 | −0.7 ± 1.37 | −1.2 ± 1.31 | −0.5 ± 1.36 | 30 | +0.8 ± 1.37 | +0.8 ± 1.09 | 0.0 ± 1.47 |

IOPD = infantile-onset Pompe disease; LOPD = late-onset Pompe disease; LVM = left ventricular mass; SBP = systolic blood pressure.