|  |
| --- |
| Tabel e-1. Data availability statement |
| CSL will only consider requests to share Individual Patient Data (IPD) that are received from systematic review groups or bona-fide researchers.  CSL will not process or act on IPD requests until 12 months after article publication on a public website.  An IPD request will not be considered by CSL unless the proposed research question seeks to answer a significant and unknown medical science or patient care question.  Applicable country specific privacy and other laws and regulations will be considered and may prevent sharing of IPD.  Requests for use of the IPD will be reviewed by an internal CSL review committee.  If the request is approved, and the researcher agrees to the applicable terms and conditions in a data sharing agreement, IPD that has been appropriately anonymized will be made available.  Supporting documents including study protocol and Statistical Analysis Plan will also be provided.  For information on the process and requirements for submitting a voluntary data sharing request for IPD, please contact CSL at [clinicaltrials@cslbehring.com](mailto:clinicaltrials@cslbehring.com). |

|  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- |
| Tabel e-2. Efficacy outcomes. | | | | | |
| **Visit**  **Change From Baseline** | **All 0.2 g/kg**  **Subjects**  **N = 73** |  | **All 0.4 g/kg**  **Subjects**  **N = 72** |  | **Overall**  **N = 82** |
| **MRC Sumscore** | | | | | |
| Baseline, n | 71 |  | 69 |  | 78 |
| Median (min, max) | 76.0 (47, 80) |  | 76.0 (47, 80) |  | 76.0 (47, 80) |
| Relapse Visit&, n | 35 |  | 7 |  | 40 |
| Median (min, max) change from baseline | −3.0 (−16, 6) |  | −8.0 (−23, 0) |  | −3.5 (−16, 6) |
| 4 weeks after relapse#, n | 32 |  | 4 |  | 36 |
| Median (min, max) change from baseline | 0.0 (−16, 8) |  | −7.5 (−20, 0) |  | 0.0 (−20, 8) |
| LPDO, n | 70 |  | 69 |  | 78 |
| Median (min, max) change from baseline | −1.0 (−20, 18) |  | 0.0 (−23, 19) |  | 0.0 (−23, 18) |
| *Non-relapsing subjects$* |  |  |  |  |  |
| LPDO, n | 35 |  | 29 |  | 38 |
| Median (min, max) change from baseline | 0.0 (−7, 18) |  | 0.0 (−4, 19) |  | 0.0 (−7, 18) |
| **I-RODS Centile Score** | | | | | |
| Baseline, n | 64 |  | 62 |  | 71 |
| Median (min, max) | 65.0 (19, 100) |  | 65.0 (19, 100) |  | 65.0 (19, 100) |
| Relapse Visit&, n | 33 |  | 7 |  | 39 |
| Median (min, max) change from baseline | −8.0 (−50, 9) |  | −13.0 (−58, 8) |  | −8.0 (−50, 9) |
| 4 weeks after relapse#, n | 30 |  | 3 |  | 33 |
| Median (min, max) change from baseline | −0.5 (−37, 21) |  | −2.0 (−5, 0) |  | −1.0 (−37, 21) |
| LPDO, n | 63 |  | 62 |  | 71 |
| Median (min, max) change from baseline | −3.0 (−50, 9) |  | 0.0 (−76, 33) |  | 0.0 (−76, 33) |
| *Non-relapsing subjects$* |  |  |  |  |  |
| LPDO, n | 28 |  | 22 |  | 31 |
| Median (min, max) change from baseline | 0.0 (−35, 9) |  | 0.0 (−12, 28) | - | 0.0 (−12, 12) |
| **Mean Grip Strength of the Dominant Hand (kPa)** | | | | | |
| Baseline, n | 71 |  | 70 |  | 79 |
| Median (min, max) | 68.7 (8, 157) |  | 66.0 (8, 157) |  | 66.7 (8, 157) |
| Relapse Visit&, n | 34 |  | 7 |  | 40 |
| Median (min, max) change from baseline | −6.3 (−71, 21) |  | −12.0 (−80, 5) |  | −6.8 (−71, 21) |
| 4 weeks after relapse#, n | 32 |  | 4 |  | 36 |
| Median (min, max) change from baseline | −0.7 (−59, 21) |  | −8.7 (−9, 14) |  | −1.2 (−59, 21) |
| LPDO, n | 70 |  | 70 |  | 79 |
| Median (min, max) change from baseline | −3.7 (−71, 21) |  | 0.7 (−80, 27) |  | 0.7 (−80, 27) |
| *Non-relapsing subjects$* |  |  |  |  |  |
| LPDO, n | 35 |  | 30 |  | 39 |
| Median (min, max) change from baseline | 0.7 (−43, 21) |  | 1.0 (−32, 21) |  | 0.0 (−43, 21) |
| &Relapse Visit was the visit at which relapse occurred. For subjects with more than 1 dosing period (0.2 and 0.4 g/kg), only relapses in the first dosing period were taken into account; #summarized based on treatment prior to relapse; $Includes all subjects who never relapsed during any time during the study; \*One more subject relapsed first on 0.2 g/kg and then again on 0.4 g/kg. Based on the definition of relapse visit, this subject was not included under “all subjects” in the 0.4 g/kg group for this visit. The subject was, however, counted in the 0.2 g/kg group; N = total number of subjects; n = number of subjects with assessments at the respective visit; LPDO = last post dose observation; kPa = kilopascal | | | | | |

|  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- |
| Table e-3. Adverse events | | | | | | |
|  | | **0.2 g/kg IgPro20** | | **0.4 g/kg IgPro20** | | **Overall** | | |
|  | | **Number (%) of subjects with an event** | **Number of events (rate/infusion)@** | **Number (%) of subjects with an event** | **Number of events (rate/infusion)@** | **Number (%) of subjects with an event** | **Number of events (rate/infusion)@** | |
| Category | | N=73 | n=1408 | N=72 | n=4145 | N=82 | n=5553 | |
| Any AE | | 33 (45.2) | 77 (0.055) | 46 (63.9) | 103 (0.025) | 62 (75.6) | 180 (0.032) | |
| Mild | | 26 (35.6) | 58 (0.041) | 37 (51.4) | 75 (0.018) | 51 (62.2) | 133 (0.024) | |
| Moderate | | 10 (13.7) | 14 (0.010) | 16 (22.2) | 21 (0.005) | 24 (29.3) | 35 (0.006) | |
| Severe | | 5 (6.8) | 5 (0.004) | 3 (4.2) | 7 (0.002) | 8 (9.8) | 12 (0.002) | |
| Local skin reactions | | 7 (9.6) | 24 (0.017) | 13 (18.1) | 16 (0.004) | 18 (22.0) | 40 (0.007) | |
| Any serious AE | | 4 (5.5) | 5 (0.004) | 3 (4.2) | 3 (< 0.001) | 7 (8.5) | 8 (0.001) | |
| Causally related and/or temporally associated serious AE | | 2 (2.7) | 2 (0.001) | 2 (2.8) | 2 (< 0.001) | 4 (4.9) | 4 (< 0.001) | |
| Causally related and/or temporally associated AE | | 22 (30.1) | 55 (0.039) | 37 (51.4) | 71 (0.017) | 51 (62.2) | 126 (0.023) | |
| Causally related AE | | 8 (11.0) | 32 (0.023) | 17 (23.6) | 29 (0.007) | 21 (25.6) | 61 (0.011) | |
| Temporally associated AE | | 22 (30.1) | 54 (0.038) | 34 (47.2) | 67 (0.016) | 49 (59.8) | 121 (0.022) | |
| AE leading to withdrawal of investigational product | | 2 (2.7) | 3 (0.002) | 1 (1.4) | 4 (< 0.001) | 3 (3.7) | 7 (0.001) | |
| AE leading to subject discontinuation | | 2 (2.7) | 3 (0.002) | 1 (1.4) | 3 (< 0.001) | 3 (3.7) | 6 (0.001) | |
| AE leading to death | | 0 | 0 | 0 | 0 | 0 | 0 | |
| AE = adverse event (treatment-emergent); N = total number of subjects; n = total number of infusions; @the rate per infusion is calculated as number of events divided by the overall number of infusions in the respective groups. | | | | | | |