Table e-2. Response to treatment in the validation cohort

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| --- | --- | --- | --- | --- | --- | --- |
|  | Patient 1 | Patient 2 | Patient 3 | Patient 4 | Patient 5 | Patient 6 |
| Treatment | Prednisone 1 mg/kg/day | Plasma exchanges X5 with 1 g methylprednisolone X3 followed by prednisone 1 mg/kg/day, MTX 0,3 mg/kg sc/w and IVIg 2g/kg monthly | High-dose steroids with multiple immunossuppressives agents trial since 2009 (MTX, MTX+tacrolimus, MTX+MMF, MTX+MMF+tacrolimus)  Monthly IVIg (2g/kg) since 07/2012 with progressive tapering of steroids to low-dose (pred 5 mg/d) and MTX 0,3 mg/kg/week | Refused by patient | MTX 0,3 mg/kg/w to be started | Refused by patient |
| Interval before re-evaluation | 6 months | 6 months | 2 years | Not applicable | Not applicable | Not applicable |
| Exam | Improved walking distance, improve muscle testing (deltoids: 4/5 vs 3-/5, psoas 4/5 vs 2+/5) | Improved dynamometer measures: deltoids +1Kg, psoas +4 Kg | Normal strength  (vs MRC-5 in 07/2012: deltoids 3/5, psoas 2/5, quads 3+/5, no distal involvement independent only for short distance < 50m, incapacity to rise, sit and climb chair) | Not applicable | Not applicable | Not applicable |
| CK before/after treatment (IU/L) | 5,030/228 | 164/47 | 2,000/119 | Not applicable | Not applicable | Not applicable |
| MRI | Not done | Not done | Not done | Not applicable | Not applicable | Not applicable |

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| --- | --- | --- | --- | --- | --- | --- |
|  | Patient 7 | Patient 8 | Patient 9 | Patient 10 | Patient 11 | Patient 12 |
| Treatment | Lost to follow-up | Methylprednisolone 1g X3, prednisone 1 mg/kg/d MTX 0,3 mg/kg sc/w, IVIg 2 g/kg/month | Plasma exchanges X5 with 1 g methylprednisolone X3 followed by prednisone 1 mg/kg/day, Rituximab 1 g DO-D15 and IVIg 2 g/kg monthly | Prednisone 1 mg/kg/d, MTX 0,3 mg/kg sc/w, IVIg 2g/kg/month | Plasma exchanges X6 with 1 g methylprednisolone X3 followed by prednisone 1 mg/kg/day, MTX 0,3 mg/kg sc/w and IVIg 2 g/kg monthly | Plasma exchanges X5 with 1 g methylprednisolone X3 followed by prednisone 1 mg/kg/day, MTX 0,3 mg/kg sc/w and IVIg 2 g/kg monthly |
| Interval before re-evaluation | Not applicable | 6 months | 14 months | 9 months | 6 months | 6 months |
| Exam | Not applicable | Normal strength (vs deltoid/psoas/neck flexors 4/5 before treatment) | Unchanged with severe proximal weakness (patient only taking prednisone 5 mg/day while rituximab/IVIg discontinued for side effects) | Improved: normal strength in upper limb, slight psoas and neck flexors weakness (4/5) | Unchanged | Improved walking distance, improve muscle testing (deltoids: 3/5 vs 2/5, psoas 3/5 vs 3-/5) |
| CK before/after treatment (IU/L) | Not applicable | 6,366/337 | 1,591/600 | 5,400/121 | 1,100/63 | 154/106 |
| MRI | Not applicable | Not done | Not done | Not done | Persistent STIR hyperintensity | STIR hyperintensity normalized |

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| --- | --- | --- | --- | --- | --- |
|  | Patient 13 | Patient 14 | Patient 15 | Patient 16 | Patient 17 |
| Treatment | 1 mg/kg/d AZA 2 mg/kg, IVIg 2 g/kg/month | Plasma exchanges X6 with 1 g methylprednisolone X3 followed by prednisone 1 mg/kg/day, MTX 0,3 mg/kg sc/w and IVIg 2 g/kg monthly | Plasma exchanges X6 with 1 g methylprednisolone X3 followed by prednisone 1 mg/kg/day, MTX 0,3 mg/kg sc/w and IVIg 2 g/kg monthly | Plasma exchanges X6 with 1 g methylprednisolone X3 followed by prednisone 1 mg/kg/day, MTX 0,3 mg/kg sc/w and IVIg 2 g/kg monthly | Plasma exchanges X5 with 1 g methylprednisolone X3 followed by prednisone 1 mg/kg/day, Rituximab 1 g DO-D15 and IVIg 2 g/kg monthly |
| Interval before re-evaluation | 6 months | 7 months | 6 months | 6 months | 12 months |
| Exam | Improvement of walking distance, stability, falls ceased and improved autonomy | Strenght improvement (psoas 4+/5 vs 3/5, neck flexors: 5/5 vs 3/5) | Improvement of muscle testing (deltoid 3/5 vs 2/5, psoas 1+/5 vs 1/5) | Improvement of walking distance and muscle testing (deltoid 5/5 vs 3+/5, psoas 3/5 vs 2/5 and neck flexors 4/5 vs 3/5) | Improved exercise endurance |
| CK before/after treatment (IU/L) | 1,070/511 | 3,300/220 | 404/30 | 3,500/130 | 604/106 |
| MRI | STIR hyperintensity normalized | Not done | Not done | Not done | STIR hyperintensity normalized |

IVIg= Intravenous immunoglobulin, CK=creatine kinase, STIR= Short tau inversion recovery, MTX= methotrexate, MMF= mycophenolate mofetil, AZA= azathioprine, sc= subcutaneous, w= week, D0= day 0, D15= day 15