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| --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- |
| Table 1 Patient demographics and treatment course | | | | | |  |  |  |  |  |  |  |  |  |  |  |
| Pt No. | Type | Sex | Baseline | Splenect-omy | Diagnosis age | Genotype | Imiglucerase treatment (U/kg/2 weeks) | | | | | | | | | |
| 2009 | 2010 | 2011 | 2012 | 2013 | 2014 | 2015 | 2016 | 2017 | 2018 |
| P1 | I | F | 2004 | N | 14 | c.475C>T/c.907G>T | Stop | Stop | Stop | Stop | 10.8 | 12.5 | 17.8 | 17.8 | 13.1 | 31.2 |
| P2 | I | F | 2006 | N | 22 | c.383C>G/c.1363A>G | 15.0 | 33.0 | 37.5 | Stop | 10.7 | 17.9 | 17.9 | 17.9 | 17.5 | 35.0 |
| P3 | III | M | 2000 | Y | 18 | - | stop | Stop | Stop | 15.4 | 12.3 | 16.7 | 16.7 | 18.5 | - | - |
| P4 | I | F | 2004 | N | 28 | c.1283G>A/c.1448T>C | 15.0 | 30.0 | stop | 11.5 | 11.5 | 15.9 | 15.9 | - | 15.4 | 18.5 |
| P5 | I | F | 1999 | Y | 32 | c.226T>G/c.1265 del 55bp | 22.5 | 22.0 | 16.0 | 16.0 | 10.0 | 20.0 | 20.0 | - | 19.6 | 39.1 |
| P6 | I | F | 2004 | N | 30 | - | 15.0 | Stop | Stop | Stop | 5.0 | 15.7 | 15.7 | 15.7 | 19.3 | - |
| P7 | III | M | 2001 | Y | 32 | - | 15.0 | 14.0 | 16.5 | 13.4 | 15.3 | 23.4 | 19.4 | 19.4 | 22.6 | 32.2 |
| P8 | I | M | 2000 | Y | 35 | c.1240G>C/c.1240G>C | 15.0 | 17.5 | Stop | 17.3 | 17.3 | 17.3 | 17.2 | 40.0 | - | 60.0 |
| P9 | I | M | 2001 | Y | 22 | c.907C>A/c.1174C>T | 16.7 | Stop | Stop | 16.4 | 16.4 | 16.4 | 16.4 | 21.4 | 21.4 | 32.0 |
| P10 | I | F | 2006 | Y | 21 | c.762-1G>C/c.1226A>G | 15.0 | - | 26.7 | 13.4 | 13.4 | 17.8 | 17.0 | 15.7 | 25.0 | 41.5 |
| P11 | I | F | 2006 | Y | 30 | c.762-1G>C/c.1226A>G | 15.0 | 35.0 | 20.0 | 14.7 | 13.7 | 15.9 | 18.2 | 18.2 | 22.7 | 36.2 |
| P12 | I | M | 2005 | Y | 31 | - | 15.0 | 16.0 | Stop | 15.6 | 17.2 | 18.8 | 15.4 | 18.8 | 17.9 | 26.9 |
| P13 | I | F | - | N | - | c.648C>A/c.1448T>C | 15.0 | 30.5 | Stop | Stop | 13.0 | 16.4 | 18.4 | 22.7 | 28.7 | 44.5 |
| P14 | I | M | 2001 | Y | 30 | c.1240G>C/c.1240G>C | 20.0 | 30.0 | Stop | 10.0 | 17.3 | 17.3 | 20.7 | 17.3 | 17.3 | 17.3 |
| P15 | III | M | 2005 | Y | 30 | c.754T>A/c.1448T>C | 15.0 | 16.0 | 18.5 | 14.8 | - | 14.8 | 14.8 | 14.8 | 14.8 | - |
| P16 | III | F | 2005 | Y | 35 | c.754T>A/c.1448T>C | 15.0 | Stop | Stop | 13.0 | 5.0 | 15.4 | 10.0 | 10.0 | 15.4 | 15.4 |
| P17 | I | F | 2000 | N | 10 | c.680A>G/c.1448T>C | Stop | Stop | Stop | Stop | 5.0 | 10.3 | 12.8 | 17.9 | - | 24.0 |
| -, data unavailable | | |  |  |  |  |  |  |  |  |  |  |  |  |  |  |