**Appendix Table C95. Adverse events: Comparator, inherited metabolic diseases**

| **Study (Investigator, country, year)** | **Record Number** | **Group (N)** | **Severity or Grade Infectious** | **F/U (mos)** | **%** | **Comment** | **Group (N) AGVHD** | **Severity or Grade\_2** | **%\_2** | **Group (N) CGVHD** | **Severity or Grade\_3** | **%\_3** | **Group (N) Engraftment failure** | **%\_4** |
| --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- |
| Arvio M, Finland, 2001 | 14180 | 12 |   |   |   |   |   |   |   |   |   |   |   |   |
| Banjar H, Saudi Arabia, 1998 | 17920 |   |   |   |   |   |   |   |   |   |   |   |   |   |
| Chan LL, Malaysia, 2002 | 11330 |   |   |   |   |   |   |   |   |   |   |   |   |   |
| El-Beshlawy A, Egypt, 2006 | 5750 |   |   |   |   |   |   |   |   |   |   |   |   |   |
| Erikson A, Sweden, 1995 | 21630 |   |   |   |   |   |   |   |   |   |   |   |   |   |
| Muenzer J, US, 2006 | 57160 |   |   |   |   |   |   |   |   |   |   |   |   |   |
| Muenzer J, US, 2007 | 57070 |   |   |   |   |   |   |   |   |   |   |   |   |   |
| Paciorkowski AR, US, 2008 | 2980 | 1 | viral infection (fever, transient leukopenia, thrombocytopenia) | 11 mos | 1 (100%) |   |   |   |   |   |   |   |   |   |
| Page KM, US, 2008 | 1280B | 2 |   |   |   | other complications (not infectious):2 developed autoimmune hemolytic anemia2 thrombocytopenia | 2 | 1 grade 11 grade 2 | 2 (100%) | 2 | 1 extensive cytopenia1 limited cytopenia (skin) | 2 (100%) | 2 | 0 (0%) |
| Patterson MC, US, 2007 | 56970 |   |   |   |   |   |   |   |   |   |   |   |   |   |
| Patterson MC, US, 2010 | 56500 |   |   |   |   |   |   |   |   |   |   |   |   |   |
| Pineda M, Spain, 2009 | 56560 |   |   |   |   |   |   |   |   |   |   |   |   |   |
| Schiffman R, Netherlands, 2008 | 56750 |   |   |   |   |   |   |   |   |   |   |   |   |   |
| Schiffmann R, Netherlands, 1997 | 58150 |   |   |   |   |   |   |   |   |   |   |   |   |   |

**Appendix Table C95. Adverse events: Comparator, inherited metabolic diseases Continued**

| **Study (Investigator, country, year)** | **Record Number** | **Group (N) TRM** | **Severity or Grade TRM** | **F/U (mos) TRM** | **% TRM** | **Group (N)Developmental Delay** | **Severity or Grade DD** | **% DD** | **Comments DD** | **Group (N)\_9** | **Severity or Grade SSGR** | **% SSGR** |
| --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- |
| Arvio M, Finland, 2001 | 14180 |   |   |   |   |   |   |   |   |   |   |   |
| Banjar H, Saudi Arabia, 1998 | 17920 | 3 |   |   | 0 (0%) |   |   |   |   |   |   |   |
| Chan LL, Malaysia, 2002 | 11330 | 1 |   |   | 0 (0%) |   |   |   |   |   |   |   |
| El-Beshlawy A, Egypt, 2006 | 5750 | 11 |   |   | 0 (0%) |   |   |   |   |   |   |   |
| Erikson A, Sweden, 1995 | 21630 | 3 |   |   | 0 (0%) |   |   |   |   |   |   |   |
| Muenzer J, US, 2006 | 57160 | 96 |   |   | 0 (0%) |   |   |   |   |   |   |   |
| Muenzer J, US, 2007 | 57070 |   |   |   |   |   |   |   |   |   |   |   |
| Paciorkowski AR, US, 2008 | 2980 |   |   |   |   |   |   |   |   |   |   |   |
| Page KM, US, 2008 | 1280B | 2 | 1 multi-system organ failure | 1.8 yrs | 1 (50%) |   |   |   |   |   |   |   |
| Patterson MC, US, 2007 | 56970 | 12 |   |   | 0 (0%) | 12 | lethargy, memory impairment, depression | 1 (8%) | withdrew from study | 12 | severe weight loss | 3 (25%) |
| Patterson MC, US, 2010 | 56500 | 10 |   |   | 0 (100%) |   |   |   |   |   |   |   |
| Pineda M, Spain, 2009 | 56560 | 66 |   |   | 0 (0%) |   |   |   |   |   |   |   |
| Schiffman R, Netherlands, 2008 | 56750 | 30 |   |   | 0 (0%) |   |   |   |   |   |   |   |
| Schiffmann R, Netherlands, 1997 | 58150 |   |   |   |   | 5 |   | 1 (20%) | One pt experienced precocious puberty due to human chorionic gonadotropin used in ERT preparation |   |   |   |