**Appendix Table C92. Time to event outcomes: Comparator, inherited metabolic diseases**

|  |  |  |  |  |  |  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- |
| **Study (Investigator, country, year)** | **Record Number** | **Group (N)** | **Outcome** | **1 yr** | **2 yr** | **3 yr** | **4 yr** | **5 yr** | **Outcome\_2** | **Med (mos)\_2** | **3 yr\_2** | **5 yr\_2** |
| Abu-Ghosh 2002 USA | 45610 | 11 |   | ~73% | 63.6 +/-14.5% | 63.6 +/-14.5% | 63.6 +/-14.5% | 63.6 +/-14.5% | PFS |   | 63.6 +/-14.5% |   |
| Park, Korea, 2006 | 5450 | 7 | DOD n=5 median 15 mos (2-30 mos)A NED n=1 20+ mosA with D n=1 130+ mos |   |   |   |   |   | EFS | median 8 months (2-20 mos) |   |   |
| Tucci, Brazil, 2007 | 3910 | 10 |   |   |   | 83.3% |   | 42.8% | DFS |   | 66.6% | 42.8% |