Appendix C. Summaries of Published Registry Studies

**Appendix Table C1. Published Registry Study of ERT for Fabry Disease**

|  |  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- | --- |
| **Author, Year, Country, Sample Size** | **ERT** | **Study Design** | **Inclusion Criteria** | **Treatment Groups** | **Mean Age at 1st Infusion (range) yrs** | **Renal fFnction** | **IgG Antibody** |
| Warnock,32, 33 2011, 2012 international N=213Q1: 53Q2: 54Q3: 54Q4: 52 | agalsidase beta | Observational, Fabry Registry | Patients in registry on ERT with baseline measure within 3 months before or after first infusion | Quartiles based on slope of estimated glomerular filtration rates (higher slope = more rapid renal disease progression) | Males:Q1: 35.3 (SD: 11.04)Q2: 40.7 (SD: 11.12)Q3: 37.0 (SD:10.91)Q4: 42.0 (SD: 9.22)Females:Q1: 43.2 (SD: 11.30)Q2: 41.0 (SD: 11.83)Q3: 40.5 (SD:15.12)Q4: 47.4 (SD: 13.09) | ● |  |
| Wilcox,34 2012N = 822 | agalsidase beta | Observational, Fabry Registry | Patients in registry on ERT | The development of anti-alphaGAL IgG antibodies was evaluated in 571 men and 251 women from the Fabry Registry who were treated with agalsidase beta | NR |  | ● |