Appendix C. Summaries of Published Registry Studies

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

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| **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=213  Q1: 53  Q2: 54  Q3: 54  Q4: 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 2012  N = 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 |  | ● |