**Appendix Table C3. Published Registry Studies of ERT for MPS II**

|  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- |
| **Author, Year, Country, Sample Size** | **ERT** | **Study Design** | **Inclusion Criteria** | **Treatment Groups** | **Mean Age at 1st Infusion (range) yrs** | **Substrate level** | **Liver Volume** | **Sleep Study** | **Spleen Size** | **Mental Function** | **Growth** | **Range of Motion** | **Pulmonary Function** | **Cardiac Symptoms** | **Discontinua-tion of Home tx** |
| Muenzer,39 2011, international, N=124 | idursulfase | observational, Hunter Outcome Survey (HOS) | patients in HOS who started ERT prior to 6 yrs of age | all who met inclusion criteria | 3.6 (SD: 1.6) | ● | ● |  |  |  |  |  |  |  |  |
| Alcalde-Martin,40 2010, international, N=6 | observational, Hunter Outcome Survey | Spanish patients in HOS who started ERT prior to 5 yrs of age | all who met inclusion criteria | 3.7 (2.8-4.7) | ● | ● | ● | ● | ● | ● | ● | ● | ● |  |
| Burton,41 2010, international, N=92 | observational, Hunter Outcome Survey | patients in HOS who had received infusions at home or in nonhospital environment | all who met inclusion criteria | at 1st infusion: median: 8.5 (3.4-17.9)  at time of transfer to home tx: median: 9.4 (3.9-21.3) |  |  |  |  |  |  |  |  |  | ● |