**Appendix Table C87. Participant characteristics: Comparator, inherited metabolic diseases**

| **Study (Investigator, country, year)** | **Record Number** | **Group (N)** | **Age (mean)** | **Age (med)** | **Age (Rng)** | **Age (SD)** | **Race (%)** | **Gender M, F (%)** | **Disease Stage/category** | **Disease Histology/Site (%)** | **Comment** |
| --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- |
| Arvio M, Finland, 2001 | 14180 | 12 |  |  |  |  | white (100%) | M (58.3%) F (41.7%) |  |  |  |
| Autti T, Finland, 1999 | 15540 | 6 non-HSCT, 7 non-diseased | non-HSCT: 6.0 yrs | non-HSCT: 5.8 yrs | non-HSCT: 3.0-10.0 yrs |  |  |  |  |  |  |
| Banjar H, Saudi Arabia, 1998 | 17920 | 3 | 2.6 yrs | 2.8 yrs | 2.0-3.0 yrs |  |  | Male (33%), Female (67%) |  |  |  |
| Chan LL, Malaysia, 2002 | 11330 | 1 | 7.6 |  |  |  | Asian (100%) | Female (100%) |  |  |  |
| El-Beshlawy A, Egypt, 2006 | 5750 | 11 | 6.14 yrs |  | 1-16 yrs |  |  |  |  |  | Mean age and range are for the whole study population of 22, which includes 11 Gaucher Type 1 pts. |
| Erikson A, Sweden, 1995 | 21630 | 3 | 7.4 yrs | 4.8 yrs | 3.8-13.7 yrs |  |  | Male (33%), Female (67%) |  |  |  |
| Goker-Alpan O, US, 2008 | 1790 | 30 |  |  | 0.2-2.5 yrs at dx |  | Hispanic (36.7%), Black (6.7%), White (56.7%) | Male (53.3%), Female (46.7%) |  |  |  |
| Maegawa GHB, Canada, 2009 | 56590B | 2 | 13.1 yrs | 13.1 yrs | 10.1-16.0 yrs |  |  | Female (100%) | juvenile form |  |  |
| Maegawa GHB, Canada, 2009 | 56590A | 3 | 15.6 yrs | 18 yrs | 8.7-20.1 yrs |  |  | Male (67%), Female (33%) | juvenile form |  |  |
| Muenzer J, US, 2006 | 57160 | 96 | placebo, n=32: 13.1 +/- 1.22 yrs ERT EOW, n=32: 14.4 +/- 1.2 yrs ERT wkly, n=32: 15.1 +/- 1.11 yrs |  | placebo, n=32: 5.0-29.0 yrs ERT EOW, n=32: 5.4-30.9 yrs ERT wkly, n=32: 6.3-26.0 yrs |  | placebo: Asian (9.4%), Black (12.5%), White (75.0%), Other (3.1%)ERT EOW: S Amer Ind (6.3%), Asian (6.3%), Black (3.2%), White (84.3%)ERT wkly: S Amer Ind (3.1%), Black (6.3%), White (87.5%), Other (31%) |  | Disease score (2-6):placebo: 3 (22%), 4 (44%), 5 (28%), 6 (6%)ERT EOW: 2 (6%), 3 (19%), 4 (34%), 5 (28%), 6 (13%)ERT wkly: 2 (6%), 3 (22%), 4 (31%), 5 (31%), 6 (9%) |  | Age stratification:placebo: 5-11 yrs (46.9%), 12-18 yrs (31.3%), 19-31 yrs (21.9%)ERT EOW: 5-11 yrs (43.8%), 12-18 yrs (31.3%), 19-31 yrs (25.0%) ERT wkly: 5-11 yrs (43.8%), 12-18 yrs (28.1%), 19-31 yrs (28.1%) |
| Muenzer J, US, 2007 | 57070 | 12 | placebo: 16.7 yrs ERT .15 mg/kg: 11.0 yrs ERT .5 mg/kg: 20.0 yrs ERT 1.5 mg/kg: 10.0 yrs | placebo: 17 yrs ERT .15 mg/kg: 10 yrs ERT .5 mg/kg: 20 yrs ERT 1.5 mg/kg: 8 yrs | placebo: 13-20 yrs ERT .15 mg/kg: 9-14 yrs ERT .5 mg/kg: 20 yrs ERT 1.5 mg/kg: 6-10 yrs |  | White (100%) | Male (100%) | attenuated |  |  |
| Paciorkowski AR, US, 2008 | 2980 | 1 | 3.3 yrs |  |  |  |  | Female (100%) |  |  |  |
| Patterson MC, US, 2007 | 56970 | 12 | 7.2 |  | 4-11 | 2.5 |  | Male (42%), Female (58%) |  |  |  |
| Patterson MC, US, 2010 | 56500 | 12 | 7.2 yrs | 7 yrs | 4-11 yrs | 2.5 yrs |  | Male (42%), Female (58%) |  |  |  |
| Pineda M, Spain, 2009 | 56560 | 66 | 12.8 yrs | <6 yrs: n=206-11 yrs: n=14>=12 yrs: n=27 | 0.6-43.0 yrs | 9.5 yrs |  | Male (47%), Female (53%) |  |  | Cannot separate pediatric and adult pt data. |
| Schiffman R, Netherlands, 2008 | 56750 | 30 | substrate reduction therapy (n=21): 10.4 yrs no treatment (n=9): 9.9 yrs |  |  | substrate reduction therapy (n=21): 5.1 yrs no treatment (n=9): 4.0 yrs |  | substrate reduction therapy (n=21): Male (48%)no treatment (n=9): Male (22%) |  |  | Age distribution grps:substrate reduction therapy: 2-11 yrs (52%), 12-17 yrs (33%), >=18 yrs (14%)no treatment: 2-11 yrs (88%), 12-17 yrs (0%), >=18 yrs (11%) |
| Schiffmann R, Netherlands, 1997 | 58150 | 5 | 6.6 yrs | 7.5 yrs | 3.5-8.5 yrs |  |  | Male (75%), Female (25%) | aggressive systemic disease |  | 3 had partial splenectomy prior to ERT |
| Seto T, Japan, 2001 | 13460A | 7 | 7 yrs | 6 yrs | 4-12 yrs |  | Asian (100%) | Male (100%) | 2 severe2 intermediate3 mild |  |  |
| Seto T, Japan, 2001 | 13460B | 3 | 11.7 yrs | 13 yrs | 4-18 yrs |  | Asian (100%) | Male (66.7%), Female (33.3%) | Type A |  |  |
| Sivakumar P, England, 1999 | 16200 | 1 | 5 yrs |  |  |  |  | F (100%) | type IIIA |  |  |
| Takahashi, Japan, 2001 | 14030 | 2 | 5.9 yrs |  | 5.8-6.0 yrs |  | Asian (100%) |  |  |  |  |