**Appendix Table C21. Design, participant selection and enrollment: Rhabdomyosarcoma**

| **Study (Investigator, country, year)** | **Record Number** | **Indication** | **Disease** | **Therapeutic Setting** | **Group (N)** | **Participant Selection (Treatment Period)** | **Design** | **n, Evaluated** | **n, Withdrawn (Lost to F/U)** | **Comment** |
| --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- |
| Bisogno, Italy, 2009 | 75340 | Malignant solid tumor | Rhabdomyosarcoma | Metastatic RMS | HSCT (70) | 1999-2006 | prospective single arm | 70 | 0 |  |
| Breneman, USA, 2003 | 75360 | Malignant Solid Tumor | Rhabdomyosarcoma | Metastatic RMS | Comparator (127) | 1991-1997 | Case series | 127 |  |  |
| Carli, Italy, 1999 | 16010 | Malignant Solid Tumor | Rhabdomyosarcoma | Metastatic RMS | HSCT (52) Comparator (44) | 1989- | single arm study | 96 |  | 52 transplanted and 44 failed to meet transplant requirements and served as comparators. |
| Doelken, Germany, 2005 | 6570 | Malignant Solid Tumor | Rhabdomyosarcoma | Relapsed | HSCT (2) |  | Case reports | 2 | 0 |  |
| Donker, Netherlands, 2009 | 1420 | Malignant Solid tumor | Alveolar Rhabdomyosarcoma | Stage IV RMS | HSCT (1) |  | case study | 1 | 0 | Allo transplant |
| Grundy, UK, 2001 | 14200 | Malignant solid Tumor | Rhabdomyosarcoma congenital | Congenital RMS | HSCT (1) |  | case report | 1 |  | This report also details three other children who died by 3 months of age. The paper also details all other known cases (7). In these cases all patients died |
| Hara, Japan, 1998 | 17950 | malignant Solid tumor | rhabdomyo  sarcoma | Stage III or IV or relapsed RMS | HSCT (7) | 1993-1997 | Case series | 7 | 0 | abstracted from a study of multiple solid tumors |
| Koscielniak, Germany, 1997 | 19800 | Malignant Solid Tumor | Rhabdomyosarcoma | Metastatic and recurrent | HSCT (36) | 1986-1994 | retrospective case series | 36 | 0 | This paper contains both Allo and Auto transplants. The data are reported together as they cannot be separated. |
| Kuroiwa, Japan, 2009 | 390 | Malignant Solid Tumor | Rhabdomyosarcoma | RMS with Beckwith-Weide-mann | HSCT (1) |  | case report | 1 | 0 |  |
| Kwan, Hong Kong, 1996 | 20800 | Malignant Solid Tumor | Rhabdomyosarcoma | Metastatic RMS | HSCT (1) |  | Case Report | 1 | 0 |  |
| Lucidarme, France, 1998 | 17610 | Malignant Solid Tumor | Rhabdomyosarcoma | Refractory or relapsed RMS | HSCT (8) | 1987-1995 | single arm phase II | HSCT (8) | 0 |  |
| Matsubara, Japan, 2003 | 10810 | Malignant Solid Tumor | Rhabdomyosarcoma | High-risk RMS | HSCT (22) | 1990-1999 | Case series | 22 | 0 | There is one patient who is 22 years old. He is included in these results. His survival is similar when compared to a 16 and a 20 year old with a similar site of relapse and status at transplant |
| McDowell, UK, 2010 | 75350 | Malignant Solid Tumor | Rhabdomyosarcoma | Metastatic RMS | HSCT (101) Comparator (45) | 1998-2005 | Two single arms | HSCT (101) Comparator (45) |  | This is not a comparative study but two single arms within the same study |
| Misawa, Japan, 2003 | 11040 | Malignant Solid Tumor | Alveolar RMS | Refractory RMS | HSCT (1) | 1997 | Case Study | 1 | 0 | Allogeneic transplant |
| Moritake, Japan, 1998 | 18280 | Malignant Solid Tumor | Rhabdomyosarcoma | relapsed | HSCT (1) | 1994 | Case report | 1 | 0 |  |
| Navid, USA, 2006 | 5930 | Malignant Solid tumor | Rhabdomyosarcoma | Metastatic RMS | HSCT (8) | 1996-2000 | case series | 8 | 1 LFU at 78 months post transplant 2 people were removed from the protocol (one due to fungal infection, one due to delayed hem. recovery) 1 patient was non-compliant | only two were transplanted as they achieved CR, one other achieved CR but was the LFU |
| Oue, Japan, 2003 | 10950 | Malignant solid Tumor | Rhabdomyosarcoma | Metastatic RMS | HSCT (1) | 1991-2001 | case series | 1 | one patient died prior to surgery due to progressive disease |  |
| Pappo, USA, 1999 | 48020 | Malignant Solid Tumor | Rhabdomyosarcoma | relapsed RMS | Comparator (605) | 1984-1997 | retrospective analysis of three single arm studies | 605 | 0 |  |
| Pappo, USA, 2001 | 47860 | Malignant Solid Tumor | Rhabdomyosarcoma | Metastatic RMS | Comparator (48) | 1994-1996 | Case series | 48 |  |  |
| Raney, USA, 2008 | 2440 | Malignant Solid Tumor | Rhabdomyosarcoma | Metastatic RMS | Comparator (91) | 1978-1997 | case series | 91 |  |  |
| Sandler, USA, 2001 | 12810 | Malignant Solid Tumor | Rhabdomyosarcoma | Metastatic RMS | Comparator (152) | 1988-1991 | Case series | 152 |  |  |
| Sato, Japan, 1998 | 48070 | Malignant Solid Tumor | Rhabdomyosarcoma | Stage III or IV RMS | HSCT (5) | 1993-1998 | case series | HSCT (5) | HSCT (5) | only abstracted treatment arm as comparator treatment was not specified for two historical controls |
| Scully, USA, 2000 | 14580 | Malignant Solid Tumor | Rhabdomyosarcoma | Recurrent RMS | HSCT (1) |  | Case report | 1 | 0 |  |
| Shaw, Israel, 1996 | 20050 | Malignant solid Tumor | Rhabdomyosarcoma | Stage IV RMS | HSCT (9) |  | prospective case series | 9 | 0 | this was a study with mixed solid tumors |
| Van Winkle, USA, 2005 | 43550 | Malignant Solid tumor | Rhabdomyosarcoma | Recurrent/refractory RMS | Comparator(27) | 1992-1996 | Case series | 27 | 0 | these patients were enrolled in three treatment protocols but will be reported together |
| Walterhouse, USA, 1999 | 17240 | Malignant Solid Tumor | Rhabdomyosarcoma | Metastatic RMS | HSCT (8) | 1992-1994 | case series | 8 | 0 |  |
| Williams, Canada, 2004 | 9010 | Malignant Solid Tumor | Rhabdomyosarcoma | Metastatic | 13 (comparator) 4 (HSCT) | 1989-1999 | retrospective review | 13 (comparator) 4 (HSCT) | 0 |  |