Clinical Outcomes of Patients with Desmoplastic Small Round Cell Tumor of the Peritoneum Undergoing Autologous HCT: a CIBMTR Retrospective Analysis

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Abstract

Desmoplastic small round cell tumor of the peritoneum (DSRCTP) is a rare, frequently fatal tumor. This retrospective study, based on CIBMTR registry data, describes the largest reported cohort of DSRCTP patients who have undergone autologous stem cell transplant (ASCT). The
probabilities of disease-free survival (DFS) at one year for patients in complete remission (CR) and not in CR were 75% (95% confidence interval: 48–94%) and 35% (15–59%), respectively. The probability of overall survival (OS) at three years was 57% (29–83%) and 28% (9–51%) for patients in CR and not in CR respectively. Median survival for the entire cohort was 31 months (36 months and 21 months for those in CR and not in CR respectively). Engraftment at 42 days was 97% (88–100%). Treatment-related mortality was low, with only one death in the first 100 days. ASCT is a tolerable approach in patients with DSRCTP, with the greatest benefit seen in those patients who obtain CR. For those not in CR, the median OS in this series is greater than previously reported (21 months versus 17 months), suggesting ASCT is useful in prolonging DFS and OS, even in patients with residual or persistent disease pre transplant.

Keywords
Autologous; Transplant; Outcomes; Desmoplastic; Tumor

INTRODUCTION

Desmoplastic small round cell tumor of the peritoneum (DSRCTP) is a rare tumor in the family of small round cell tumors that classically affects adolescent males, and it has a poor prognosis. It is distinguished from other small round cell tumors by the balanced chromosomal translocation t(11;22)(p13;q12) involving the Ewing’s sarcoma/primitive neuroectodermal tumor gene (EWS) and Wilms’ tumor gene (WT1). This fusion product leads to production of a chimeric protein that can upregulate transcriptional activity. The tumor characteristics were first described in 1989 by Gerald and Rosai. The first literature review of DSRCTP in 1996 identified 101 cases, with a median survival of 17 months (range 3–72), in patients with a median age of 21 years (6–38), none of whom underwent stem cell transplant. The most common initial sites of disease are within the abdomen, frequently involving the peritoneum. CT imaging often demonstrates multiple heterogeneous masses in the abdomen, often without clear origin. Subsequent spread of the disease typically is identified in regional lymph nodes, followed by hematogenous spread to distant sites. Other solid organs have also been known to give rise to desmoplastic small round cell tumors, including the lung, kidney, pancreas, bladder, and ovary. Small round blue cells, fibrosclerotic stroma and a nesting pattern of cellular growth are typical. Immunohistochemical staining demonstrates reactivity to epithelial, mesenchymal, myogenic and neural markers that is relatively specific for DSRCTP.

Poor outcomes, mainly due to disease recurrence, are seen in patients with DSRCTP. Yet, with increasingly aggressive therapies, there is evidence for improved survivorship. Initial studies using intensive alkylator regimens with optional incorporation of autologous stem cell transplant (ASCT) suggested a survival benefit. A recent French review demonstrates the frequency of different chemotherapy regimens with cyclophosphamide, Adriamycin, vincristine and ifosfamide, vincristine, actinomycin being the most common. The Memorial Sloan-Kettering Cancer Center experience demonstrated three-year overall survival (OS) of 55% in patients who received chemotherapy, surgery and radiotherapy, as
compared to 27% three-year OS in patients who received fewer than three modalities.\(^{(11)}\) The persistently relapsing nature of the disease, even with the most aggressive multimodality therapies, has led to recommendations to balance potential side effects of therapy with the presumed palliative nature of currently available therapies – particularly in those patients with advanced disease at presentation.\(^{(12)}\) Recently, there has been interest in a broad range of therapies, from hyperthermic intraperitoneal chemotherapy to temsirolimus to imatinib, in an attempt to improve initial disease control.\(^{(13–16)}\)

Known prognostic factors for identifying patients at high risk for disease recurrence include metastatic disease to sites other than the lung, tumor volume >100ml, axial site involvement, lack of response to first-line therapy, and relapsed disease.\(^{(17)}\)

Small studies have identified a number of long-term survivors, many of whom have been treated with high-dose chemotherapy followed by ASCT.\(^{(18–20)}\) Attempts to intensify therapy with sequential ASCTs have not improved outcomes.\(^{(21)}\)

**PATIENTS AND METHODS**

**Data Sources**

The CIBMTR is a research affiliation of the International Bone Marrow Transplant Registry and the National Marrow Donor Program (NMDP) established in 2004, which comprises a voluntary working group of more than 450 transplantation centers worldwide that contribute detailed data on consecutive allogeneic and autologous hematopoietic cell transplantations to a Statistical Center at the Medical College of Wisconsin in Milwaukee and the NMDP Coordinating Center in Minneapolis. Centers are required to report all transplants consecutively; compliance is monitored by on-site audits. Patients are followed longitudinally. Computerized checks for discrepancies, physicians’ review of submitted data and on-site audits of participating centers ensure data quality. Observational studies conducted by CIBMTR are performed in compliance with all applicable federal regulations pertaining to the protection of human research participants. Protected Health Information used in the performance of such research is collected and maintained in CIBMTR’s capacity as a Public Health Authority under the HIPAA Privacy Rule.

A total of 36 patients treated with ASCT for DSRCTP were reported to the CIBMTR from 1999–2007. Disease and transplant-related characteristics were obtained from the CIBMTR database. The CIBMTR collects transplant-essential (registration) data on all registered patients. It is estimated that CIBMTR captures 65% to 75% of autologous transplants in North America at the registration level.\(^{(22)}\) Additional research data is collected on a subset of registered patients. For this study, supplemental disease-specific data was requested for all patients and received in 18 cases. The PIs confirmed histology and the presence of the EWS-WT1 gene fusion in each of these 18 patients by reviewing each pathology report. All pathology reports reviewed were consistent with the data reported on the registration and research forms. Patient characteristics and outcomes were similar between the larger data set and those 18 patients for whom more detailed data was submitted.
Study Endpoints

Outcomes analyzed included disease-free survival (DFS), treatment-related mortality (TRM) and OS. DFS was defined as survival without disease relapse or progression. TRM was defined as death without evidence of disease relapse or progression. OS was defined as death from any cause. Surviving patients were censored at last contact or at time of allogeneic transplant.

Statistical Analysis

A descriptive analysis was used to describe the patient-, disease-, and treatment-related variables. For discrete variables, number and proportions were calculated. For continuous variables, the median and range are presented. Survival probabilities were calculated using the Kaplan-Meier estimator, and 95% confidence intervals were calculated using variance estimated by Greenwood's formula. TRM was calculated using the cumulative incidence function. Estimates of TRM were calculated using disease relapse or progression as the competing risk, and cumulative incidence for disease progression/relapse was calculated treating TRM as the competing risk. Prognostic variables were not identified given the small study size, although univariate probabilities of relapse, TRM, DFS, and OS were calculated separately for patients who entered transplant in CR and those who were not in CR.

RESULTS

The 36 patients who were identified underwent ASCT at 29 transplant centers from 1999–2007, with 50% of the transplants taking place from 2002–2004. The median follow-up time for surviving patients was 44 months (range 4–89) post ASCT. Patient characteristics are summarized in Table 1, where data for the entire cohort (n=36) and for patients with supplemental information (n=18) are displayed in separate columns.

The median age at time of transplant was 19 (8–46) years. Eighty-one percent of the patients were male. At the time of transplant, 13 of the 31 patients (42%) were considered to have CR. For the patients with supplemental information available, pre-transplant treatment included surgery in 72% of patients and radiation in 17%. Sixty-seven percent were known to have metastatic disease at time of initial presentation. Twenty conditioning regimens were identified for the 30 patients for whom data was available. The most common agents were thiotepa (n=19), etoposide (n=12), melphalan (n=12), cyclophosphamide (n=9) and carboplatin (n=8). The graft source was peripheral blood in 33 patients, bone marrow in two patients and information was missing for one patient.

OUTCOMES

Outcomes are summarized in Table 2. TRM was low overall, with only one death in the first 100 days. All patients engrafted (defined by an absolute neutrophil count ≥0.5 x 10⁹/L for three consecutive days) by day 42, for a total engraftment rate of 97% (95% confidence interval: 88–100%), except the patient who died on day six from bacterial infection. Engraftment at day 28 was 90% (78–98%). Probability of DFS declined dramatically in those patients who had not attained a pre-transplant CR. The estimated probability of DFS at one year post transplant was 75% (48–94%) in those who attained a pre-transplant CR,
versus 35% (15–59%) in those who did not. DFS was 40% (15–69%) versus 9% (0–29%) at three years post transplant in the two groups, respectively. Differences in OS rates were most notable at three years post transplant between patients with CR 57% (29–83%) and without CR 28% (9–51%). Causes of death were relapse (83%), bacterial infection (4%) and unknown (13%). The three-year follow-up completeness index was 89%.

**DISCUSSION**

DSRCTP is a rare disease with heterogeneous presentations. Although a wide variety of therapies has been explored, there has not been dramatic progress in improving overall survival. Bisogno et al. recently published the largest clinical trial to date that included ASCT for treatment of DSRCTP.(21) Fourteen patients were enrolled on the trial, which administered initial chemotherapy, followed by three sequential intensive chemotherapy courses, then autologous peripheral blood progenitor infusions. After ASCT, the patients underwent surgery and radiotherapy if possible, followed by six cycles of maintenance chemotherapy with vincristine, actinomycin and cyclophosphamide. Three-year event-free and overall survival rates were 15.5% and 38.9% respectively, with a median follow up of 27 months. As noted, the initial presentation of DSRCT with frequent organ invasion and metastases tends to preclude early complete remission, even with the most aggressive combined modality regimens.

DSRCTP is a chemosensitive disease, particularly at high doses, so incorporation of ASCT is a reasonable treatment option. This study describes the largest series of reported patients who have undergone ASCT for DSRCTP. High engraftment rates and low TRM suggest that ASCT is feasible and tolerable. This approach appears to be useful for the full spectrum of disease states, though unsurprisingly is associated with the best results in those patients who enter transplant in CR. This study supports findings of case series, in which patients only proceeded to ASCT when they had attained CR.(24)

For those patients not in CR, the median OS in this series is longer (21 months) than previously reported (17 months) in a heterogeneous group not treated with ASCT, suggesting a potential role for ASCT to prolong DFS and OS even in patients with residual or persistent disease pre-transplant.(2)

This study is limited by its retrospective nature and relatively small patient numbers. A prospective study incorporating complete surgical resection when possible, followed by a standardized alkylator-based chemotherapy regimen and autologous transplant would allow for more robust data collection and a meaningful standard of care for treatment and comparative trials in the future. The lack of a complete supplemental data set limits the strength of the conclusions and is a reminder of the importance of data collection and submission, particularly in rare diseases.

Although OS has increased since the initial literature reviews, disease-free outcomes remain poor for patients with DSRCTP, even with the incorporation of ASCT. Entering the transplant phase in CR appears to be the most important criterion for long-term survival and should be a focus of further study.
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| Variables                      | Patients with supplemental information | Entire Cohort |
|-------------------------------|----------------------------------------|---------------|
|                               | Median (range) N (%)                   | Median (range) N (%) |
| Number of patients            | 18                                     | 36            |
| Number of teams               | 12                                     | 29            |
| Age, median (range), years    | 18 (12 – 46)                           | 19 (8–46)     |
| Age, years                    |                                        |               |
| <10                           |                                       | 1 (3)         |
| 10–19                         | 10 (56)                                | 18 (50)       |
| ≥20                           | 5 (28)                                 | 12 (33)       |
| Male                          | 3 (17)                                 | 5 (14)        |
| Region                        |                                        |               |
| North America                 | 18 (100)                               | 33 (91)       |
| Europe                        | 0                                      | 1 (3)         |
| South America                 | 0                                      | 2 (6)         |
| Location of primary tumor     |                                        |               |
| Pelvic                        | 5 (28)                                 |               |
| Abdominal wall                | 7 (39)                                 |               |
| Retroperitoneum               | 4 (22)                                 |               |
| Other viscera                 | 2 (11)                                 |               |
| Metastatic disease            |                                        |               |
| No                            | 6 (33)                                 |               |
| Yes                           | 12 (67)                                |               |
| Largest dimension of the tumor, median (range), cm | 11 (7–16) | |
| Largest dimension of the tumor, cm |                        |               |
| <10                           | 6 (33)                                 |               |
| ≥10                           | 8 (44)                                 |               |
| Unknown                       | 4 (22)                                 |               |
| Year of transplant            |                                        |               |
| 1999–2001                     | 1 ( 6)                                 | 5 (14)        |
| 2002–2004                     | 10 (56)                                | 18 (50)       |
| 2005–2007                     | 7 (39)                                 | 13 (36)       |
| Radiation therapy             |                                        |               |
| No                            | 15 (83)                                |               |
| Yes*
| 3 (17)                         | |
| Surgical therapy              |                                        |               |
| No                            | 2 (11)                                 |               |
### Variables

| Variables                                      | Patients with supplemental information | Entire Cohort |
|------------------------------------------------|----------------------------------------|---------------|
| Biopsy only                                    | Median (range) N (%)                    | Median (range) N (%) |
| Partial resection                              | 3 (17)                                 | 3 (17)        |
| Gross total resection with involved margins    | 8 (44)                                 |               |
| Total resection clean margins ≤ 2 cm           | 1 (6)                                  |               |
| Total resection clean margins > 2 cm           | 1 (6)                                  |               |

### Best response to therapy

| Best response to therapy                      | Median (range) N (%) |
|-----------------------------------------------|----------------------|
| Complete response\(^b\)                       | 6 (33)               |
| CR with persistent imaging abnormality        | 3 (17)               |
| Partial response                              | 8 (44)               |
| Not assessed                                  | 1 (6)                |

### Number of lines of therapy

| Number of lines of therapy | Median (range) N (%) |
|----------------------------|----------------------|
| 1                          | 11 (61)              |
| 2                          | 5 (28)               |
| ≥2                         | 2 (11)               |

### Interval from diagnosis to transplant, median (range), months

| Interval from diagnosis to transplant, median (range), months | Median (range) N (%) |
|--------------------------------------------------------------|----------------------|
| <1 year                                                      | 13 (72)              |
| ≥1 year                                                      | 5 (28)               |

### Disease status prior to transplant

| Disease status prior to transplant                      | Median (range) N (%) |
|---------------------------------------------------------|----------------------|
| Complete response                                       | 6 (33)               |
| CR with persistent imaging abnormality                  | 2 (11)               |
| Partial response                                        | 6 (33)               |
| Stable disease                                          | 1 (6)                |
| Progressive disease                                     | 2 (11)               |
| Not assessed                                            | 1 (6)                |
| Missing                                                 | 4                    |

### Total number of transplants

| Total number of transplants | Median (range) N (%) |
|-----------------------------|----------------------|
| 1                           | 15 (83)              |
| 2                           | 1 (6)                |
| 3                           | 1 (6)                |
| 4                           | 1 (6)                |

### Transplant type

| Transplant type | Median (range) N (%) |
|-----------------|----------------------|
| Auto only       | 17 (94)              |
| Auto + allo\(^c\) | 1 (6)                |

### Follow-up of survivors, median (range), months

| Follow-up of survivors, median (range), months | Median (range) N (%) |
|-------------------------------------------------|----------------------|
|                                                | 50 (24–89)           |

\(^a\) Two local radiation, one site unknown.

\(^b\) Five patients in CR1, 1 patient in CR2.
Patient data was censored at time of allogeneic transplant, undertaken for myelodysplastic syndrome.
Table 2

Univariate outcomes based on pre-transplant disease status

|                  | Entire cohort | Not in CR | % (95% CI) | All patients N=18* | % (95% CI) | All patients N=36** | % (95% CI) |
|------------------|---------------|-----------|------------|--------------------|------------|---------------------|------------|
| **Pre-transplant status** |               |           |            |                    |            |                     |            |
| Relapse          | N=18         | 18        |            |                    |            |                     |            |
| @ 100 days       | 0            | 0         | 38 (8–71)  | 9 (4–52)           | 56 (32–79) | 6 (4–23)            | 88 (50–100)|
| @ 1 year         | 14 (0–47)    | 14 (0–47)| 6 (1–23)   | 8 (0–30)           | 13 (0–41) | 6 (1–23)            | 88 (50–100)|
| @ 3 years        | 43 (11–78)   | 100       | 0          | 19 (4–41)          | 43 (11–78) | 0                   | 22 (5–52) |
| Relapse @ 100 days | N=18         | 18        |            |                    |            |                     |            |
| Treatment related mortality |          |           |            |                    |            |                     |            |
| @ 100 days       | 14 (0–47)    | 14 (0–47)| 6 (1–23)   | 8 (0–30)           | 13 (0–41) | 6 (1–23)            | 88 (50–100)|
| @ 1 year         | 71 (35–96)   | 71 (35–96)| 19 (4–41) | 43 (11–78)         | 71 (35–96) | 19 (4–41)          | 43 (11–78)|
| @ 3 years        | 86 (51–100)  | 86 (51–100)| 0          | 19 (4–41)          | 86 (51–100) | 0                   | 22 (5–52) |
| Disease-free survival | N=18        | 18        |            |                    |            |                     |            |
| @ 100 days       | 96 (51–100)  | 96 (51–100)| 0          | 19 (4–41)          | 96 (51–100) | 0                   | 22 (5–52) |
| @ 1 year         | 38 (6–82)    | 38 (6–82)| 3 (0–11)   | 1 (0–3)            | 38 (6–82) | 3 (0–11)           | 1 (0–3)   |
| @ 3 years        | 75 (48–94)   | 75 (48–94)| 7 (0–29)   | 4 (0–14)           | 75 (48–94) | 7 (0–29)           | 4 (0–14)  |
| Overall survival | N=18         | 18        |            |                    |            |                     |            |
| @ 100 days       | 88 (50–100)  | 88 (50–100)| 0          | 19 (4–41)          | 88 (50–100) | 0                   | 22 (5–52) |
| @ 1 year         | 88 (50–100)  | 88 (50–100)| 0          | 19 (4–41)          | 88 (50–100) | 0                   | 22 (5–52) |
| @ 3 years        | 75 (48–94)   | 75 (48–94)| 7 (0–29)   | 4 (0–14)           | 75 (48–94) | 7 (0–29)           | 4 (0–14)  |

*Patient data for whom disease status was unknown (N=1) (either not assessed or missing) prior to transplant is included here, making N=18
**Patient data for whom disease status was unknown (N=5) (either not assessed or missing) prior to transplant is included here, making N=36