ELSEVIER

Blood 142 (2023) 6894–6896

The 65th ASH Annual Meeting Abstracts

ONLINE PUBLICATION ONLY

705.CELLULAR IMMUNOTHERAPIES: LATE PHASE AND COMMERCIALLY AVAILABLE THERAPIES

Omidubicel-Onlv for Allogeneic Transplantation (allo-HCT) in Patients with Hematologic Malignancies: Results of a Multicenter Open Label Expanded Access Program

Mitchell Horwitz, MD[1], *Stephanie B. Tsai, MD MS*[2], *Andrew R. Rezvani, MDBA*[3], *Richard T Maziarz, MD*[4], *Uri Goshen*[5], *Stuart Levy*[5], *Aurelie Schwarzbach*[5], *Roei D. Mazor*[5], *Patrick J. Stiff, MD*[6], *Gary J. Schiller, MD*[7]

[1] Adult Stem Cell Transplant Program, Division of Cellular Therapy, Department of Medicine, Duke University Medical Center, Durham, NC
[2] Loyola University Medical Center, Maywood, IL
[3] Stanford University Cancer Institute, Palo Alto, CA
[4] Knight Cancer Institute, Oregon Health & Science University, Portland, OR
[5] Gamida Cell, Jerusalem, Israel
[6] Division of Hematology and Oncology, Loyola University Medical Center, Maywood, IL
[7] Division of Hematology and Oncology, University of California, Los Angeles, Los Angeles, CA

Introduction
Omidubicel-onlv is a nicotinamide-modified allogeneic hematopoietic progenitor cell therapy donor source derived from umbilical cord blood (UCB). A phase 3 randomized study (NCT02730299) demonstrated improved hematopoietic recovery and decreased infections in patients transplanted with omidubicel compared to UCB. An expanded access program (EAP) was conducted to provide access to omidubicel in advance of commercial availability in the United States (US) in April 2023.
Objective
This was an open-label, single-arm EAP evaluating clinical outcomes in patients with hematologic malignancies following allo-HCT with omidubicel.
Methods
Eligible patients (≥12 years) with hematologic malignancies received myeloablative conditioning, prophylactic medications, and supportive care as per institutional guidelines. Patients were monitored for neutrophil and platelet engraftment, infections, graft versus host disease (GVHD), and additional clinical events for up to 2 years post-transplantation. Results were retrospectively compared with previously described outcomes in patients from the phase 3 study comparing transplant with omidubicel (P3-OMI, n=52) and unmanipulated UCB (P3-UCB, n=56).
Results
Between July 2020 and April 2023, 36 patients were enrolled and 29 (18 males, 11 females) were transplanted at 5 US sites. Median age was 39 years (range: 20-73); 55% were White, 21% Asian, 17% Black, and 7% other. Diagnoses were acute myelogenous leukemia (38%), acute lymphocytic leukemia (28%), myelodysplastic syndrome (21%), and other (14%). All patients received myeloablative conditioning (total body irradiation-based: 58.5%, chemotherapy alone: 34.5%). Median transplanted CD34+ cell dose was 5.6x10 ^{6} cells/kg (range: 1.8-18.7x10 ^{6} cells/kg) and HLA match was 4/6 in 52%, 5/6 in 45%, and 6/6 in 3%.
Median follow-up was 6.3 months (range: 0.3-27.7). No infusion reactions were reported. Median time to neutrophil (≥500/µl x3 days) and platelet (>20,000/µl x3 days) engraftment were 13 and 34 days, respectively. Primary graft failure occurred in 2 (6.9%) patients, both alive >100 days after second haploidentical transplant. The incidences of first grade 2-3 bacterial and invasive (grade 3) fungal infection through 100 days post-transplant were 16.9% and 0%, respectively. The incidence of grade 3 viral infections 1-year post-transplant was 13.4%. At 100 and 365 days post-transplant, disease-free survival (DFS) rates were 95.2% and 82.5%, and overall survival (OS) rates were 96.3% and 84.2%, respectively.
Acute GVHD was reported in 13 (44.8%) patients (grade 2: 34.5%, grade 3: 10.3%) and mild chronic GVHD in 1 (3.5%) patient. There were 4 deaths (13.8%): 2 due to relapse (Days 275 and 381), 1 due to infection (Day 135) and 1 due to respiratory failure (Day 10). Post-transplant lymphoproliferative disease was reported in 1 patient.
Demographic and disease characteristics were similar to those in the phase 3 omidubicel registration trial, including racial and ethnic diversity (>39% minorities). Neutrophil engraftment time in EAP patients was similar to P3-OMI (10 days) and shorter

than P3-UCB (20.5 days). Similarly, median platelet recovery was similar to P3-OMI (37 days) and shorter than P3-UCB (50 days). Infection, GVHD, DFS, and OS in EAP were also similar to those observed in P3-OMI (Table 1).

Conclusion

In a real-world EAP setting, the outcomes of allo-HCT with omidubicel in patients with hematologic malignancies were consistent with those from the phase 3 registration study. These data support the role of omidubicel as a donor source, particularly for patients from diverse racial backgrounds.

Disclosures Tsai: *Jazz Pharmaceutical:* Speakers Bureau; *Bristol Myers Squibb:* Speakers Bureau. **Rezvani:** *Pharmacyclics.:* Research Funding. **Maziarz:** *Athersys:* Other: Patent holder; *Orca Therapeutics:* Research Funding; *Gamida:* Research Funding; *Kite:* Consultancy; *AlloVir:* Consultancy, Research Funding; *Novartis:* Consultancy, Research Funding. **Goshen:** *Gamida Cell:* Current Employment. **Levy:** *Gamida Cell:* Current Employment. **Schwarzbach:** *Gamida Cell:* Current Employment. **Mazor:** *Gamida Cell:* Current Employment. **Stiff:** *Macrogenics:* Research Funding; *Incyte Corp:* Research Funding; *AtaraBiotherapeutics:* Research Funding; *Eisai:* Research Funding; *Gamida Cell:* Research Funding; *Takeda:* Research Funding; *Amgen:* Research Funding; *CRISPR:* Consultancy. **Schiller:** *AVM Biotechnology:* Research Funding; *Agios:* Research Funding; *Samus Therapeutics:* Research Funding; *Deciphera:* Research Funding; *Daiichi Sankyo:* Research Funding; *Mateon Therapeutics:* Research Funding; *Geron:* Research Funding; *Genentech/Roche:* Research Funding; *Sangamo Bioscience:* Research Funding; *Celator:* Research Funding; *Precog:* Research Funding; *REGiMMUNE:* Research Funding; *Trovagene:* Research Funding; *Tolero Pharmaceuticals:* Research Funding; *Takeda:* Research Funding; *Stemline Therapeutics:* Research Funding; *Sellas Life Sciences:* Research Funding; *Pfizer:* Research Funding; *Onconova Therapeutics:* Research Funding; *Gamida Cell:* Research Funding; *Fujifilm:* Research Funding; *FORMA Therapeutics:* Research Funding; *Delta-Fly Pharma:* Research Funding; *Arog:* Research Funding; *Actuate Therapeutics:* Research Funding; *Actinium Pharmaceuticals:* Research Funding; *Karyopharm Therapeutics:* Research Funding, Speakers Bureau; *Sanofi:* Research Funding, Speakers Bureau; *Stemline Therapeutics:* Speakers Bureau; *Kite:* Research Funding, Speakers Bureau; *Astellas Pharma:* Consultancy, Research Funding, Speakers Bureau; *AbbVie:* Consultancy, Research Funding, Speakers Bureau; *Novartis:* Consultancy, Research Funding; *Jazz Pharmaceuticals:* Consultancy, Research Funding, Speakers Bureau; *Constellation Pharmaceuticals:* Research Funding; *ElevateBio:* Research Funding; *Ono Pharmaceutical:* Research Funding; *Syros Pharmaceuticals:* Research Funding; *Kronos Bio:* Research Funding; *Incyte:* Consultancy, Research Funding, Speakers Bureau; *Celgene:* Consultancy, Research Funding; *Agios:* Consultancy; *Ono Pharmaceutical:* Consultancy; *Johnson & Johnson:* Current equity holder in publicly-traded company; *Amgen:* Current equity holder in publicly-traded company, Research Funding; *Bristol Myers Squibb:* Current equity holder in publicly-traded company, Research Funding, Speakers Bureau.

OffLabel Disclosure: midubicel-onlv, a nicotinamide-modified allogeneic hematopoietic progenitor cell therapy derived from umbilical cord blood (UCB), is FDA-approved for use in patients aged 12 years and older with hematologic malignancies who are planned for UCB transplantation following myeloablative conditioning to reduce the time to neutrophil recovery and the incidence of infection

Table 1: Clinical outcomes

	EAP	Phase 3 Omidubicel	Phase 3 UCB
Patients transplanted, n	29	52	56
Median follow up time, months (range)	6.3 (0.33-27.7)	13.9 (1-16.8)	14.3 (0.72-19.3)
Median neutrophil engraftment time, days (range)[1]	13 (7-35)	10 (6-35)	20.5 (13-40)
Median platelet engraftment time, days (range)[1]	34 (24-58)	37 (21-180)	50 (21-120)
Primary graft failure, n (%)	2 (6.9%)	1 (1.9%)	6 (10.7%)
First grade 2-3 bacterial / invasive fungal infections at 100 days post-transplant (%)[2]	16.9%	33.0%	59.9%
First severe viral infection at 1 year post-transplant (%)[2]	13.4%	8.2%	29.8%
Acute GVHD by day 100 post-transplant			
Grades 2-4, n (%)	13 (44.8%)	32 (61.5%)	24 (42.9%)
Grades 3-4, n (%)	3 (10.3%)	8 (15.4%)	12 (21.4%)
1-year DFS	82.5%	77.8%	87.3%
1-year OS	84.2%	76.9%	64.3%

[1] For EAP, based on 27 patients with sufficient follow-up for engraftment evaluation.

[2] Cumulative incidence

Figure 1

https://doi.org/10.1182/blood-2023-181167