(n=76) were 0-23 mo, 48.6% (n=124) 2-11y, and 21.6% (n=55) 12-16y. 83.9% received allogeneic transplants (98.7% of pts 0-23 mo; 66.7% of pts 2-11y; 100% of pts 12-16y); 16.1% received autologous transplants (1.3%, 0-23 mo; 32.3%, 2-11y; none, 12-16y).

Overall day +100 survival was 51.4% (95% CI, 45.2%-57.5%; n=131/255). Day +100 survival by age subgroup was 52.6% (95% CI, 41.4%-63.9%; n=40/76) in pts aged 0-23 mo, 53.2% (95% CI, 44.4%-62.0%; n=66/124) in those aged 2-11y, and 45.5% (95% CI, 32.3%-58.6%; n=25/55) in pts aged 12-16y.

Safety analyses pooled data from the dose-finding and pivotal trials (with on-site data monitoring): 65 DF-treated (25 mg/kg/day), 14 HC children. 93.8% (61/65) of DF-treated pts (95.5%, 0-23 mo; 89.7%, 2-11y; 100%, 12-16y) and all HC pts had ≥1 treatment-emergent AE (TEAE). Overall AE profiles were similar in treated and HC pts. 67.7% of pts receiving 25 mg/kg/day DF had ≥1 serious TEAE (77.3%, 0-23 mo; 58.6%, 2-11y; 71.4%, 12-16y). 44.6% of pts (31.8%, 0-23 mo; 44.8%, 2-11y; 64.3%, 12-16y) had ≥1 treatment-related TEAE (TR-AE), most commonly pulmonary alveolar hemorrhage (9.1%, 0-23 mo; 6.9%, 2-11y; 14.3%, 12-16y). TEAEs leading to death were reported in 49.2% (32/65) of pts (72.7%, 0-23 mo; 31.0%, 2-11y; 50.0%, 12-16y) and 57.1% of HC pts (8/14). In the expanded-access program, 98 (51.9%) pts ≤16y old had ≥1 serious TEAE; 41 (21.7%) pts ≤16y old had ≥1 TR-AE, most commonly pulmonary hemorrhage (6.9%).

Conclusions: In this pooled analysis of data from ped pts with VOD/SOS and MOD, day +100 survival was generally consistent across ped age subgroups. The safety profile was as expected for this critically ill population.

Support: Jazz Pharmaceuticals

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Cross-Sectional Patient Survey on the Need for a Long-Term Follow-up Program after Autologous Hematopoietic Cell Transplantation

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Background: With a growing number of long-term survivors after hematopoietic cell transplantation (HCT), more attention has been paid to monitor and prevent late adverse effects in order to maintain patients' quality of life. The long-term follow-up (LTFU) program plays a role in screening of complications and patient education. While the importance of the LTFU program has been increasingly recognized after allogeneic HCT that may predispose patients to graft-versus host disease and other late effects, its role after autologous HCT is less apparent. To evaluate the patient's need for a LTFU program after autologous HCT as well as the incidence of late effects, we conducted a single-center cross-sectional questionnaire survey.

Methods: We included adult patients who received autologous HCT for hematological malignancy at our center from January 1993 to February 2014, stayed free of relapse and are making regular visits to our clinic. Questionnaires were designed to ask about complications/disorders (diagnosis, year of diagnosis, occasion of diagnosis and treatment), annoying symptoms or troubles they experienced after discharge, thoughts and expectations on LTFU clinic, and social background (marital status, employment, and conception/delivery). The patient background was extracted from the transplant registry database.

Results: Among 231 patients who received autologous HCT, 114 were alive free of relapse. The questionnaires were mailed to 71 patients who had visits to our clinic, and 43 (61%) responded. Male accounted for 51%, the median duration after HCT was 4 years (1-19 years), and the median age at survey was 59 years (32-73). Twenty-one patients (56%) reported 44 disorders diagnosed after discharge (the median time of onset, 1.1 years from HCT; range, 0.1-8.9 years). Infection was the most frequent episode (n=19) and VZV accounted for 11 of them. Chronic renal failure (n=4) and endocrine disorders (n=4) were also documented. To the questions regarding "daily life" and "physical condition" after discharge, 24 (63%) and 35 (81%) patients, respectively, answered that they had experienced troubles. To the question asking the need for a LTFU program after autologous HCT, 39 patients (91%) answered "Yes". As the role of the LTFU clinic, they wished to get information on daily life including food and exercise, to learn how to cope with specific symptoms, and to learn how similar patients were doing.

Conclusions: More than 80% of the participants answered that they had experienced disorders or troubles other than the primary disease after discharge, and 91% of participants suggested the need for a LTFU program after autologous HCT. Although we must acknowledge the potential selection bias and under- or over-estimation due to the small cohort size as well as the nature of self-administered questionnaires, these results would help to construct a LTFU program after autologous HCT.

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Secondary SOLID Tumors after Allogeneic STEM CELL Transplantation: A CROSS-Sectional Evaluation in 260 Adults at 1-Year Follow-up

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Introduction: Allogeneic hematopoietic cell transplantation (HCT) is an effective therapeutic option for high-risk hematological malignancies; 80% of those who survive the first 2-years are expected to become long-term survivors.

The prevalence of chronic health conditions approaches 75% among HCT survivors and that for severe or life-

threatening conditions exceeds 20%. Secondary tumors are among the most significant late effects.

Patients and Methods: A standardized follow-up of HCT survivors is applied at our center. We report the occurrence of secondary malignancies between July 2014 and July 2015 in 260 adult patients (pts) who underwent an HCT between 1992 and 2014.

Results: Median age of pts was 48y (r10-76) at transplant and 54y (r20-82) at follow-up. Acute leukemia was the most represented diagnosis (139/260). At transplant 169 pts were in complete remission. The donor was a family haploidentical donor in 100 cases. A treosulfan based conditioning regimen was administered in 203 pts and 64 pts received TBI. GvHD prophylaxis mostly relies upon the combination of rapamycin and mycophenolate (180 pts).

The median time of follow-up was 4.4y (r1–22; cumulative follow-up 1404y).

Second cancer screening was performed according to international guidelines. The incidence of new cases during the time of observation was 10% (26 pts) - 6 pts are actually under work-up. The prevalence of second cancer in our population was 18% (47 cases – male/female 28/19). The median age at secondary solid tumor diagnosis were 63y (r28-73); donor sources (haploidentical, HLA identical unrelated/relates) were evenly distributed. According to well-known risk factors 11 pts were receiving TBI as part of the conditioning regimen and 19 pts were affected by chronic GvHD.

Non-melanoma skin cancer was diagnosed in 25 pts overall, 12 new diagnosis in the past year, 3/12 with invasive behavior. Cervical intraepithelial neoplasia was documented in 7 pts - 4/7 new diagnosis - all HPV related, 5/7 with concomitant chronic GvHD. Prostate cancer was documented in 3 pts - 1/3 new diagnosis - and papillary bladder cancer in 2 pts. Two pts were diagnosed with lung adenocarcinoma – 1 died due to invasive disease.

We also documented a single case of: papillary carcinoma of the thyroid, parathyroid carcinoma, invasive renal cell cancer, larynx neoplasia, gastric cancer, colon adenocarcinoma, endometrial neoplasia and breast cancer.

All pts were treated according to standards for general population, 44/47 are alive. No difference was observed according to donor source (Chi-square test - p ns).

Discussion: HCT survivors are at a defined relevant risk of developing secondary malignancies. Active surveillance, adequate counseling, primary and secondary prevention are crucial within routine HCT long-term follow-up to enhance early diagnosis/treatment and overall outcome.

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Neurocognitive Functioning in Adults Who Underwent Nonmyeloablative HLA-Matched Sibling Hematopoietic Stem Cell Transplant (SCT) for Sickle Cell Disease (SCD)

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Background: Individuals with SCD are at risk for neurocognitive impairments. While potentially curative, SCTs come with their own risk of neurotoxicities, particularly from radiation and high-dose ablative chemotherapies. Newer, nonmyeloablative regimens may prevent some of these toxicities. This study examines the long-term cognitive impact of nonmyeloablative transplants in adults with SCD.

Methods: Seventeen adults (11 males; mean age = 32.2 years, *SD*=9.0, range 17–52) participated in neuropsychological evaluations before transplant and 12 months post-transplant, as part of a research protocol at the National Heart Lung and Blood Institute. Donors were HLA-matched siblings; the conditioning regimen consisted of alemtuzumab and 300cGy total body irradiation, and they received sirolimus for graft-versus-host disease prophylaxis. Evaluations assessed global cognition, processing speed, memory, attention, and executive functions. Patients completed the PROMIS Physical Functioning questionnaire.

Results: Global cognition was within normal limits. Mean Verbal and Performance IQ scores were average at baseline and follow-up, with no significant changes noted. Mean processing speed scores were average, and increased significantly from baseline (M=92.1, SD=10.1) to 12 months (M=99.2, SD=11.6, t=2.1, p=.013). Mean scores on memory, attention and executive tests were average and stable across time points. No group declines in mean cognitive scores were noted. Mean t-scores on the physical functioning questionnaire increased from 41.6 (SD=4.7) to 49.0 (SD=10.0; t=3.4, p=.01), indicating significant improvement from baseline to 12 months post-transplant. Hemoglobin levels increased significantly from pre-transplant (M=9.1, SD=0.9) to post-transplant (M=13.1, SD=2.2; t=8.9, $p<.0001$).

Conclusions: In this small sample, a comparison of neurocognitive scores from baseline to 12 months post-nonmyeloablative SCT does not indicate any deleterious effects from transplant. In fact, a significant improvement emerged in mental processing speed. Physical functioning also improved per patient questionnaires. As suggested by some prior research, the significant increase in hemoglobin levels may underlie improvements in processing speed.

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Improvement in Hematopoiesis with Treatment of Iron Overload Following Allogeneic Transplants

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Improvement in Hematopoiesis with Treatment of Iron Overload Following Allogeneic Transplants: Iron overload (IOL) is associated with increased transplant related morbidity and mortality. It is now recognized that IOL associated free iron catalyzes oxidative damage to hematopoietic cells and suppress hematopoiesis. To test clinical relevance of this hypothesis, we analyzed data on 224 patients receiving allogeneic hematopoietic transplantation (AHT) between January 2005 and December 2014. Total of 23