



## BMT CTN PROTOCOL #0301

### Fludarabine-based Conditioning for Allogeneic Marrow Transplantation from HLA-compatible Unrelated Donors in Severe Aplastic Anemia

#### Changes from Version 8.0 to Version 9.0

The DSMB acknowledged that the BMT CTN 0301 study has momentum, and approved the protocol team's plan to extend accrual beyond the original goal of 78-81 patients to 94 patients with study completion anticipated in spring 2014. The objective is to rebalance HLA matched (8/8) and HLA-mismatched (7/8) transplants and gain more data on engraftment in the 50 mg/kg CY dose level.

Deletions to the protocol are indicated in strike-out text; additions are noted in underlined text.

*Cover page:* Posted on [clinicaltrials.gov](http://clinicaltrials.gov) as NCT00326417; updated list of participating centers; updated version number; added signature lines

#### *§Protocol Synopsis*

- Study Design: The Phase II portion of the trial refines the dose selection and allocates an additional ~~5470~~ 94 patients to the optimal dose, at which two year post transplant survival will be assessed. The combined enrollment in Phase I and II will total ~~78-81~~ 94 patients.
- Dose Finding Plan: The total sample size of ~~78-81~~ 94 patients enrolled in Phases I and II of the trial contribute to the selection of the optimal dose.
- Accrual Objective: A maximum of ~~78-81~~ 94 patients will be enrolled and followed for 24 months post-transplant.
- Accrual Period: The estimated accrual period is ~~two to four~~ eight years.

*§Treatment Schema:* N=~~78-81~~ 94 (total including Phase I)

*§5.3 Study Design Synopsis:* Phase II allocates ~~5470~~ 94 additional patients to the optimal dose, bringing the maximum sample size to ~~81~~ 94 in total.

*§5.4 Accrual:* A maximum of ~~81~~ 94 patients, up to 65 years of age with severe aplastic anemia ...completed in ~~2-4~~ eight years.

*§Appendix B, Informed Consent:* How many people will take part in the study? As many as ~~81~~ 94 patients will take part in this study at different hospitals in the United States.