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Trial record **4 of 9** for: umbilical cord blood and fanconi anemia
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Hematopoietic Stem Cell Transplantation in High Risk Patients With Fanconi Anemia

This study is currently recruiting participants.

Verified May 2013 by Masonic Cancer Center, University of Minnesota

Sponsor:
Masonic Cancer Center, University of Minnesota

Information provided by (Responsible Party):
Masonic Cancer Center, University of Minnesota

ClinicalTrials.gov Identifier:
NCT00258427

First received: November 22, 2005
Last updated: May 29, 2013
Last verified: May 2013
History of Changes

Full Text View | Tabular View | No Study Results Posted | Disclaimer | How to Read a Study Record

Purpose

RATIONALE: A bone marrow or **umbilical cord blood** transplant may be able to replace **blood**-forming cells that were destroyed by chemotherapy. Giving combination chemotherapy before a donor stem cell transplant may make the transplant more likely to work. This may be an effective treatment for patients with high risk **Fanconi's anemia**.

PURPOSE: This clinical trial is studying how well combination chemotherapy works in treating high risk patients who are undergoing a donor stem cell transplant for **Fanconi's anemia**.

Condition	Intervention	Phase
Fanconi Anemia	Biological: anti-thymocyte globulin Biological: filgrastim Drug: busulfan Drug: cyclophosphamide Drug: fludarabine phosphate Drug: methylprednisolone Biological: Hematopoietic stem cell transplantation	Phase 2

Study Type: Interventional
Study Design: Endpoint Classification: Safety/Efficacy Study
Intervention Model: Single Group Assignment
Masking: Open Label
Primary Purpose: Treatment

Official Title: Hematopoietic Stem Cell Transplantation in High Risk Patients With **Fanconi Anemia** MT2002-02

Resource links provided by NLM:

Genetics Home Reference related topics: Fanconi anemia

MedlinePlus related topics: Anemia

Drug Information available for: Cyclophosphamide Prednisolone Prednisolone acetate Methylprednisolone acetate Busulfan Methylprednisolone Prednisolone sodium phosphate Prednisolone phosphate Prednisolone sodium succinate Methylprednisolone sodium succinate Fludarabine Fludarabine phosphate Filgrastim Lenograstim Granulocyte colony-stimulating factor Antilymphocyte Serum

U.S. FDA Resources

Further study details as provided by Masonic Cancer Center, University of Minnesota:

Primary Outcome Measures:

- Percent of Graft Failure [Time Frame: Day 30] [Designated as safety issue: No]

  Graft failure = ANC <5 x 10^8/L by day 30.

Secondary Outcome Measures:

- Incidence of Acute and Chronic Graft-Versus-Host Disease [Time Frame: Day 42 and 1 Year] [Designated as safety issue: No]
- Incidence of Relapse [Time Frame: 1 Year] [Designated as safety issue: No]
- Incidence of Major Infections [Time Frame: Day 1 through End of Treatment] [Designated as safety issue: Yes]
- Transplant-Related Toxicity [Time Frame: Day 100] [Designated as safety issue: Yes]
- Overall Survival [Time Frame: 1 Year] [Designated as safety issue: No]

  cumulative proportion surviving

- Incidence of Chronic Graft-Versus-Host Disease [Time Frame: Day 42 and 1 Year] [Designated as safety issue: No]

Estimated Enrollment:	25
Study Start Date:	March 2002
Estimated Study Completion Date:	July 2015
Estimated Primary Completion Date:	July 2014 (Final data collection date for primary outcome measure)

Arms	Assigned Interventions
Experimental: Transplant in **Fanconi Anemia** Patients Hematopoietic stem cell transplantation (HSCT) in high risk patients with **Fanconi Anemia** (FA)- transplanted with related or unrelated CD34+ selected HSCT after Busulfan, Cytoxan, Fludarabine and Antithymocyte globulin.	Biological: anti-thymocyte globulin Given 15 mg/kg/day intravenously every 12 hours on Days -5 through -1. Other Name: ATG Biological: filgrastim given 5 mcg/kg/day intravenously on Day 1 (continue until absolute neutrophil count (ANC) ≥2.5 x 10^9/L) Other Name: G-CSF Drug: busulfan Busulfan 0.8 mg/kg intravenously (IV) every 12 hours on Days -7 and -6 (1.0 mg/kg IV if <4 years old) Drug: cyclophosphamide 10 mg/kg intravenously (IV) on Days -5 through -2. Other Name: Cytoxan Drug: fludarabine phosphate 35 mg/m^2 intravenously (IV) on Days -5 through -2. Other Name: Fludara Drug: methylprednisolone 1 mg/kg intravenously (IV) every 12 hours on Days -5 through -1. Biological: Hematopoietic stem cell transplantation Infused on Day 0 - Donor bone marrow or **umbilical cord blood** will be collected in the usual sterile manner using established parameters determined by the National Marrow Donor Program. Other Name: HSCT

Detailed Description:
OBJECTIVES:

Primary

- Determine whether the incidence of neutrophil engraftment is acceptable in high-risk patients with Fanconi's anemia treated with busulfan, cyclophosphamide, fludarabine, and antithymocyte globulin followed by allogeneic hematopoietic stem cell transplantation.

Secondary

- Determine the tolerability of mycophenolate mofetil in these patients.

- Determine the incidence of acute and chronic graft-vs-host disease in patients treated with this regimen.
- Determine the incidence of major infections in patients with a history of major infections treated with this regimen.
- Determine the incidence of relapse in patients with refractory anemia with excess blasts, refractory anemia with excess blasts in transformation, or acute myeloid leukemia treated with this regimen
- Determine the probability of 1-year survival of patients treated with this regimen.

OUTLINE: Patients are stratified according to donor/recipient HLA type (identical vs other).

- Cytoreductive combination chemotherapy: Patients receive busulfan intravenously (IV) over 2 hours twice daily on days -7 and -6 and cyclophosphamide IV over 2 hours and fludarabine IV over 30 minutes once daily on days -5 to -2.
- Graft failure prophylaxis: Patients receive methylprednisolone IV twice daily on days -5 to 30 and anti-thymocyte globulin IV over 4-6 hours twice daily on days -5 to -1.
- Graft-vs-host disease prophylaxis: Patients receive cyclosporine IV over 2 hours twice daily on days -3 to 100 (if patient has a matched sibling donor) or days -3 to 180 (if patient has another donor type). Patients also receive mycophenolate mofetil orally or IV twice daily on days -3 to 45.
- Allogeneic hematopoietic stem cell transplantation (HSCT): Patients undergo allogeneic HSCT (using bone marrow or umbilical cord blood) on day 0. Patients receive filgrastim (G-CSF) subcutaneously beginning on day 1 and continuing until blood counts recover.

After completion of study treatment, patients are followed periodically for 3 years.

Eligibility

Ages Eligible for Study:	up to 44 Years
Genders Eligible for Study:	Both
Accepts Healthy Volunteers:	No

Criteria

Inclusion Criteria:

- Patients must be <45 years of age with a diagnosis of Fanconi anemia with:
  - Biallelic BRCA2 mutations, or
  - Aplastic anemia, or advanced myelodysplastic syndrome (MDS) (MDS with ≥5% blasts), or acute leukemia who are ineligible for total body irradiation. Aplastic anemia is defined as having at least one of the following (with or without cytogenetic abnormalities): platelet count <20 * 10^9, - absolute neutrophil count (ANC) <5 * 10^8/L, - Hgb <8 g/dL /
- Patients must have an HLA-A, B, DRB1 identical or 1 antigen mismatched related or unrelated BM donor or have an HLA-A, B, DRB1 identical, 1 antigen or 2 antigen mismatched related or unrelated umbilical cord blood (UCB) donor. Patients and donors will be typed for HLA-A and B using serological level typing and for DRB1 using high resolution molecular typing.
- Adequate major organ function including:
  - Cardiac: ejection fraction >45%
  - Hepatic: no clinical evidence of hepatic failure (e.g. coagulopathy, ascites, no cirrhosis)
  - Karnofsky performance status >70% or Lansky >50%
- Women of child bearing potential must be using adequate birth control and have a negative pregnancy test.

Exclusion Criteria:

- Active CNS leukemia at time of HSCT.
- Active uncontrolled infection within one week of hematopoietic stem cell transplant (HSCT).
- Pregnant or lactating female.

Donor Inclusion Criteria:

- Donor must be in good health based on review of systems and results of physical examination.
- Donor must have a normal hemoglobin, white count, platelet count and partial thromboplastin time (PTT), and a negative diepoxybutane (DEB) test.
- HIV-NAT negative, HTLV-1, HTLV-2 negative, Hepatitis B and C negative.
- Female donors of childbearing potential must have a negative pregnancy test.
- Unrelated donors must agree to peripheral blood stem cell (PBSC) donation

Donor Exclusion Criteria:

- Donor is a lactating female.

Contacts and Locations

Please refer to this study by its ClinicalTrials.gov identifier: NCT00258427

Locations

United States, Minnesota

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Sponsors and Collaborators

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Investigators

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More Information

No publications provided

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ClinicalTrials.gov Identifier:	NCT00258427 History of Changes
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Study First Received:	November 22, 2005
Last Updated:	May 29, 2013
Health Authority:	United States: Food and Drug Administration

Keywords provided by Masonic Cancer Center, University of Minnesota:
Fanconi anemia

Additional relevant MeSH terms:

Anemia	Busulfan
Fanconi Anemia	Cyclophosphamide
Fanconi Syndrome	Fludarabine monophosphate
Anemia, Hypoplastic, Congenital	Lenograstim
Anemia, Aplastic	Fludarabine
Hematologic Diseases	Methylprednisolone Hemisuccinate
Bone Marrow Diseases	Prednisolone
Genetic Diseases, Inborn	Methylprednisolone acetate
DNA Repair-Deficiency Disorders	Prednisolone acetate
Metabolic Diseases	Methylprednisolone
Kidney Diseases	Prednisolone hemisuccinate
Urologic Diseases	Prednisolone phosphate
Renal Tubular Transport, Inborn Errors	Vidarabine
Metabolism, Inborn Errors	Immunosuppressive Agents
Antilymphocyte Serum	Immunologic Factors

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