

# Clinical trial protocol

## Title

Prospective Randomized Study of the Incidence and Outcome of Veno-Occlusive Disease (VOD) With the Prophylactic Use of Defibrotide (DF) in Pediatric Stem Cell Transplantation

## Inclusion Criteria

Ages Eligible for Study: up to 18 Years, Genders Eligible for Study: Both

- Age <18 years
- myeloablative conditioning and autologous or allogeneic stem cell transplantation with at least one of the following risk factors for VOD:
  1. Pre-existing liver disease
  2. Second myeloablative HSCT
  3. History of treatment with gemtuzumab ozogamicin (MYLOTARGÒ, GO, CMA-676, Wyeth)
  4. Allogeneic HSCT for leukemia beyond the second relapse
  5. Osteopetrosis (OP)
  6. Conditioning with busulfan and melphalan
  7. Macrophage activating syndromes (MAS, like hemophagocytic lymphohistiocytosis, Griscelli, Chediak-Higashi)
  8. Adrenoleukodystrophy (ALD)

## Exclusion Criteria

- Pregnant patients
- Patients who are transplanted but do not fulfill any of the above mentioned criteria

## Study start

December 2005

## Expected completion

January 2009

## Expected total enrollment

270

## International multicenter study