Danazol Research Study

• Danazol
  – Male hormone of the androgen class
  – May be less virilizing than the commonly used drug oxymetholone
  – Approved by the FDA for adults with other conditions
  – Never carefully studied in FA for efficacy or safety
Design/Purpose of Study

• 24 week dose escalation study of danazol in patients with Fanconi Anemia (FA) or Dyskeratosis Congenta (DC)

• Evaluate safety by carefully monitoring side effects

• Determine response of hemoglobin and platelets

• Gene expression studies of bone marrow cells to determine ‘signature’ of response to danazol
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• Study Design
  – 20 patients (FA or DC)
  – Danazol daily orally for up to 24 weeks
  – Outpatient hospital visits for labs/physical exams at:
    • Screening (with bone marrow aspirate)
    • Weeks 2, 5, 8, 12, (14*), 18, (20*), 24, 38, and 52
    • * if dose increase at previous visit
Eligibility

• Inclusion criteria
  – At least 3 years of age or ≥14 kg body weight
  – Low levels of either
    • Neutrophils - ANC < 500/μL
    • Hemoglobin - < 8 g/dL
    • Platelets - < 30,000/μL

• Exclusion criteria
  – Liver or kidney disease
  – Androgen therapy within last 3 months
  – Patients with HLA matched sibling donors
Contacts

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