Therapeutic efficacy guide

Haematology	Increase hemoglobin levels within 12 to 24 months to >11.0 g/dL for women and children, >12.0 g/dL for men Patients with splenectomy: normalization of platelet count by 1 year of treatment Patients with an intact spleen: • Moderate baseline thrombocytopenia: the platelet count should increase by 1.5- to 2.0-fold by year 1 and approach low-normal level by year 2 • Severe baseline thrombocytopenia: the platelet count should increase by 1.5-fold by year 1 and continue to increase slightly during years 2 to 5 (doubling by year 2), but normalization is not expected
Visceral	Reduce and maintain the liver volume to 1.0 to 1.5 times normal Reduce the liver volume by 20% to 30% within year 1 to 2 and by 30% to 40% by year 3 to 5 Reduce and maintain spleen volume to <2 to 8 times normal Reduce the spleen volume by 30% to 50% within year 1 and by 50% to 60% by year 2 to 5
Skeletal	Lessen or eliminate bone pain within 1 to 2 years Prevent bone crises Prevent osteonecrosis and subchondral joint collapse Improve BMD
General	Increased feelings of well-being Increased growth in growth-retarded children Decreased fatigue Decreased cachexia

Pastores et al. Seminars in Hematology 2004