

## Phase IV open-label study of the efficacy and safety of deferasirox after allogeneic stem cell transplantation

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## Supplemental Information and Tables

### Supplemental Information

#### Inclusion Criteria

>18 years old

Allogeneic HSCT at least 6 months prior to enrollment

Transfusional iron overload

Serum ferritin >1000 ng/mL or

>20 units red blood cell transfusions

Absolute neutrophil count >1000/mm<sup>3</sup>

#### Exclusions Criteria

Iron overload not related to transfusion

Uncontrolled hypertension

Active viral hepatitis

Human immunodeficiency virus infection

Serum creatinine >2x ULN or creatinine clearance <50 mL/min

Urine protein/creatinine ratio >0.5 mg/mL in 2 samples (separated by > 1 week)

Aspartate aminotransferase >5x ULN

Active concomitant malignancy

Prior use of iron chelators after HSCT

History of ocular toxicity related to iron chelation

Treatment with investigational systemic drugs within  $\leq$  4 weeks or investigational topical drugs within the previous 7 days

Systemic or psychiatric disease which may preclude the patient from carrying out the

investigational treatment

Surgical or medical history which may impact absorption, distribution, metabolism or excretion of any drug

History of non-compliance

Drug or alcohol abuse within the prior 12 months

Pregnant or nursing women or adults of child-bearing potential not using effective methods of contraception

HSCT: hematopoietic stem cell transplant; ULN: upper limit of normal

Study Visit Schedule

	Treatment initiated														Study end	
	<b>Deferasirox</b>															
	Baseline															
Week	1	2	3	4	8	12	16	20	24	28	32	36	40	44	48	52
Physical examination	X			X			X		X		X		X			X
Relevant clinical history / Current medical condition / Pregnancy test / Height	X															
Eye examination and audiometry	X															X
Hematology and biochemistry	X	X		X	X	X	X	X	X	X	X	X	X	X	X	X
Transfusion	As necessary															
Iron metabolism <sup>1</sup>	X			X	X	X	X	X	X	X	X	X	X	X	X	X
Serum creatinine and creatinine clearance	X	X	X	X	X	X	X	X	X	X	X	X	X	X	X	X
MRI <sup>2</sup>	X															X
Proteinuria	X			X	X	X	X	X	X	X	X	X	X	X	X	X
Adverse events	X	X	X	X	X	X	X	X	X	X	X	X	X	X	X	X
GVHD / Infections / SOS <sup>3</sup>	X	X	X	X	X	X	X	X	X	X	X	X	X	X	X	X

<sup>1</sup> Serum ferritin, transferrin, transferrin saturation  
<sup>2</sup>MRI: Magnetic Resonance Imaging, at selected sites  
<sup>3</sup> SOS: sinusoidal obstruction syndrome

Supplemental Figure 1 Patient Disposition

