Risk factors for endocrine complications in transfusion-dependent thalassemia patients on chelation therapy with deferasirox: a risk assessment study from a multi-center nation-wide cohort

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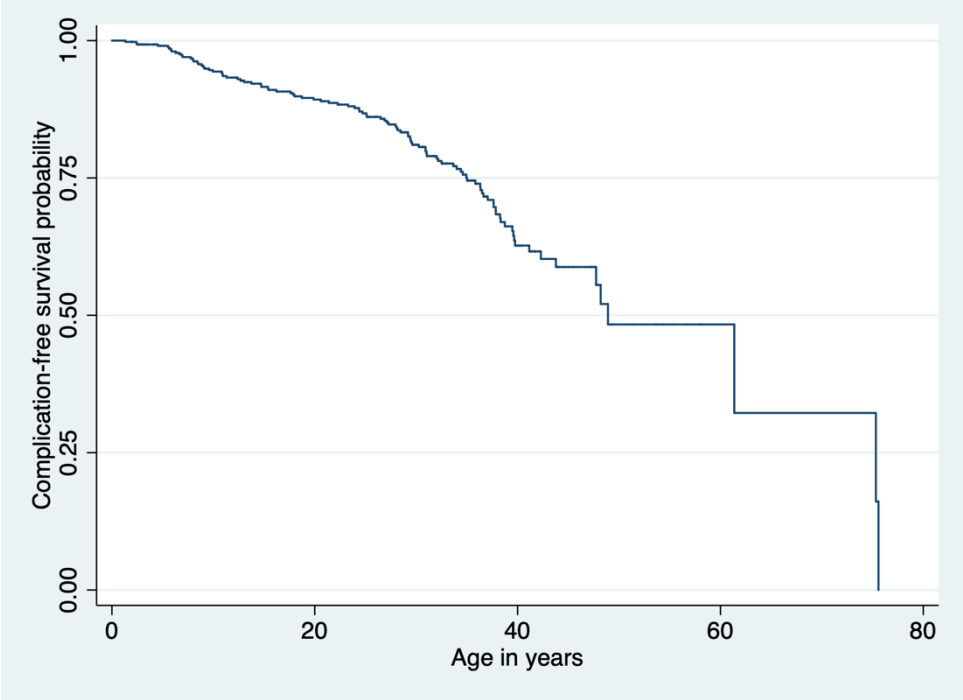
Supplemental Material

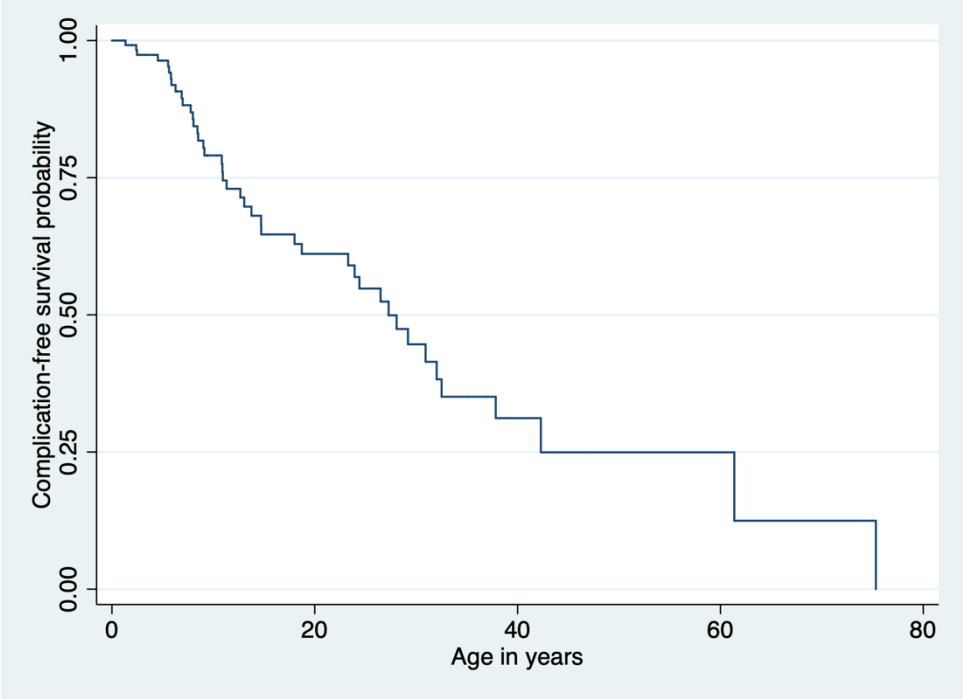
Online Supplementary Methods

Standardized protocols for the management of the disease were shared and discussed during meetings hold with clinicians from each centre. All consecutive patients visited at the participating sites since September 2009 were recruited into the cohort provided the inclusion criteria were met: affected by TDT and assigned to long- term deferasirox monotherapy. All patients included in the study had to maintain the same chelator during the observation in order to avoid biases related to the change of chelator drug. In the event of patient death, the collection was performed until the last available assessment. In the event of deferasirox discontinuation, endocrine data were collected until the time of discontinuation, along with reasons for discontinuation, such as adverse events, therapy failure, poor compliance, medical decision, etc. For those patients who started deferasirox therapy prior to 2009 and were still on the same chelation therapy at the study enrolment, the observation time was extended to the start of the deferasirox therapy. All centres followed standardized protocols, also for the transfusion program,[18] maintaining pre-transfusion haemoglobin above 9-10.5 g/dl or higher levels for children or patients with cardiac complications (≥ 10.5 g/dl). Some variables were routinely measured in adults, such as estradiol, testosterone, LH, and FSH, BMD.

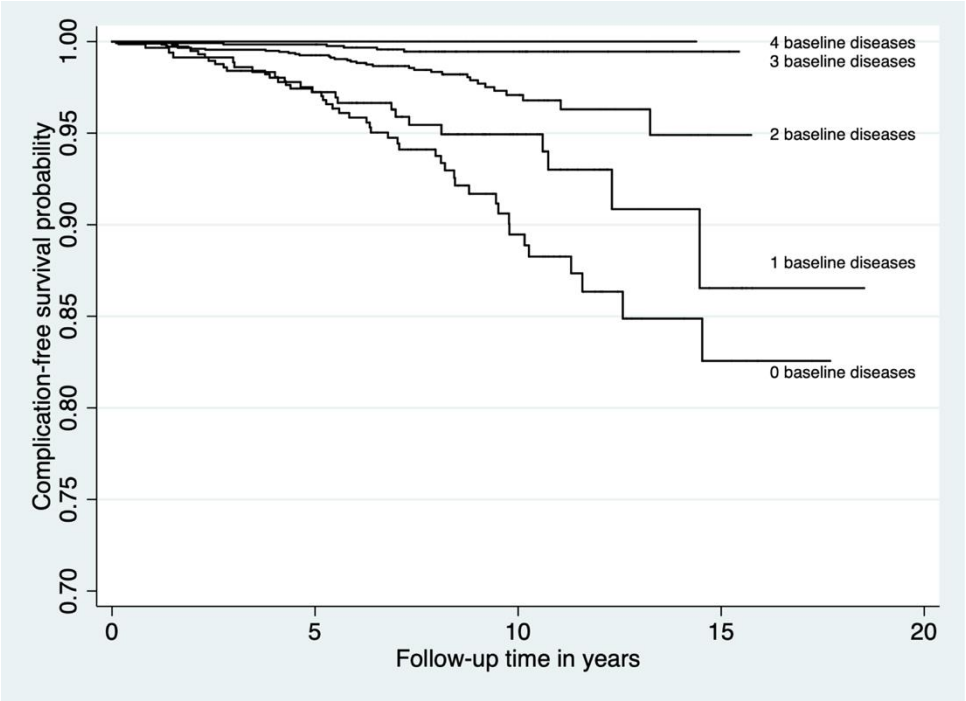
Supplemental Material 1. Crude survival curve for all patients (n=426, events=104), with age as the underlying time variable.



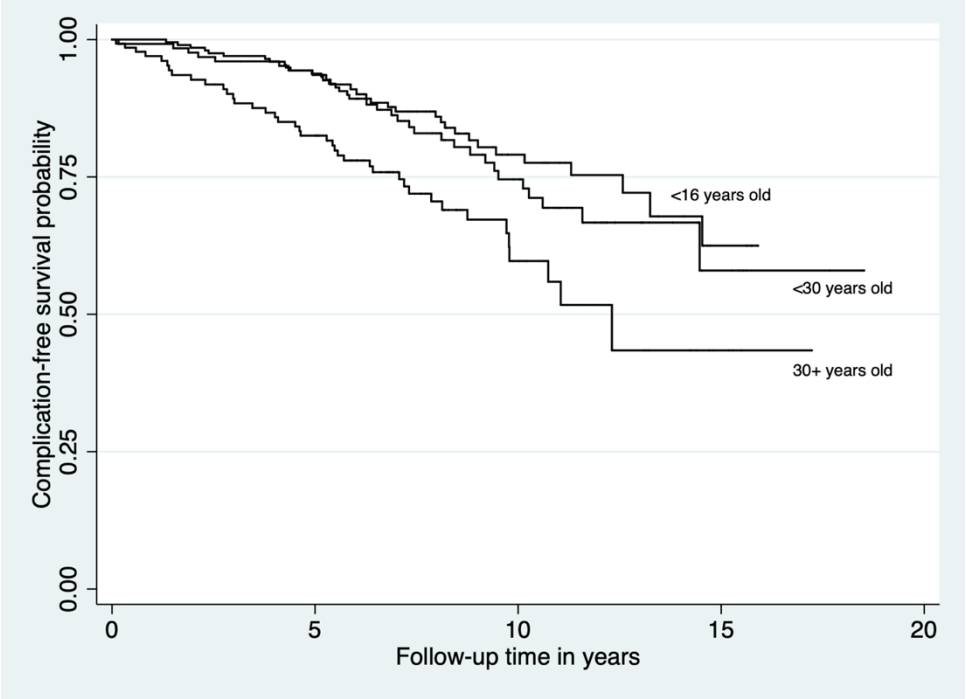
Supplemental Material 2. Crude survival curve for patients with no endocrinopathies at baseline (n=118, events=43), with age as the underlying time variable.



Supplemental Material 3. Survival curves by number of endocrinopathies at baseline, adjusted for age and TSH.



Supplemental Material 4. Survival curves by age group, adjusted for TSH and number of endocrine diseases at baseline.



Supplemental Material 5. Survival curves by levels of TSH, adjusted for age and number of endocrine conditions at baseline.