Safety and efficacy of human apotransferrin infusion in patients with β -thalassemia intermedia: the AIM study

Authors

Kadère Konté,¹ Dorine W. Swinkels,²,³ Ilona Kleine Budde,⁴ Erfan Nur¹,⁵ and Bart J. Biemond¹

¹Department of Clinical Hematology, Amsterdam University Medical Centres, University of Amsterdam, Amsterdam; ²Department of Laboratory Medicine, Radboud University Medical Center, Nijmegen; ³Sanquin Blood Bank and Sanquin Diagnostics BV, Amsterdam; ⁴Prothya Biosolutions Netherlands B.V., Amsterdam and ⁵Department of Blood Cell Research, Sanquin Research, Amsterdam, the Netherlands

Correspondence:

B.J. BIEMOND - b.j.biemond@amsterdamumc.nl

https://doi.org/10.3324/haematol.2024.285045

Received: January 15, 2024. Accepted: August 6, 2024. Early view: August 22, 2024.

©2025 Ferrata Storti Foundation

Published under a CC BY-NC license

Apotransferrin dose	170 mg/kg (n=3)	340 mg/kg (n=4)
Age, years	43 (26- 30)	44 (27-53)
Gender, F/M	3/0	2/2
Transfusion dependent (chronically), n, %	1	2
Chelation therapy, n, %	3	5
Liver iron content, mg/g	6 (4.3 - 7.3)	5.8 (4.3 - 6)

Table S1: Baseline characteristics, data are presented as median and IQR.

	170mg/kg study population (n=3)	340mg/kg study population (n=4)
Any TEAE at least possibly related (per patient)	3 (100%)	2 (50%)
TEAE occurring in entire		•
group		
Fatigue	1 (33.3%)	1 (25%)
Dizziness	1 (33.3%)	1 (25%)
Cold extremities	-	1 (25%)
Pyrexia	1 (33.3%)*	-
Oral dysesthesia	1 (33.3%)	-
Muscle spasm	1 (33.3%)	-
Any serious adverse event	-	-

Table S2: Treatment emergent adverse events at least possibly related to study treatment, split in any treatment emergent adverse event per patient and treatment emergent adverse events occurring in the entire group Presented: number of subjects (percent of subjects) (* temperature already elevated pre-infusion). Abbreviations: treatment emergent adverse event (TEAE).

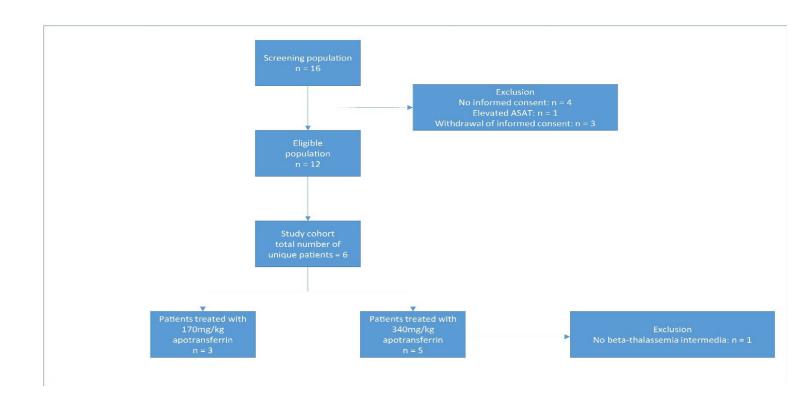


Figure S1: Study flow. Abbreviations: aspartate-aminotransferase (ASAT).