

Three-year safety, efficacy, and renal outcomes of mitapivat treatment in sickle cell disease: results from the phase 2 open-label study

Authors

Geoffrey Z.L. Kuppens,¹ Maria Armila D. Ruiz,² Myrthe J. van Dijk,^{1,3} Minke A. E. Rab,^{3,4} Cleo Derichs,¹ Joline Saes,¹ Anita W. Rijnveld,⁴ Marjon H. Cnossen,⁵ Erfan Nur,^{6,7} Bart J. Biemond,⁶ Marije Bartels,¹ Santosh L. Saraf² and Eduard J. van Beers¹

¹Center for Benign Hematology, Thrombosis and Hemostasis - Van Creveldkliniek, University Medical Center Utrecht, Utrecht University, Utrecht, the Netherlands; ²Division of Hematology/Oncology, University of Illinois Hospital & Health Sciences System, Chicago, IL, USA; ³Department of Central Diagnostic Laboratory - Research, University Medical Center Utrecht, Utrecht University, Utrecht, the Netherlands; ⁴Department of Hematology, Erasmus University Medical Center, Rotterdam, the Netherlands; ⁵Department of Pediatric Hematology and Oncology, Erasmus MC Sophia Children's

Hospital University Medical Center Rotterdam, Rotterdam, the Netherlands; ⁶Department of Hematology, Amsterdam University Medical Center, University of Amsterdam, Amsterdam, the Netherlands and ⁷Department of Blood Cell Research, Sanquin Research, Amsterdam, the Netherlands

Correspondence:

E.J. VAN BEERS - e.j.vanbeers-3@umcutrecht.nl

<https://doi.org/10.3324/haematol.2025.289105>

Received: September 2, 2025.

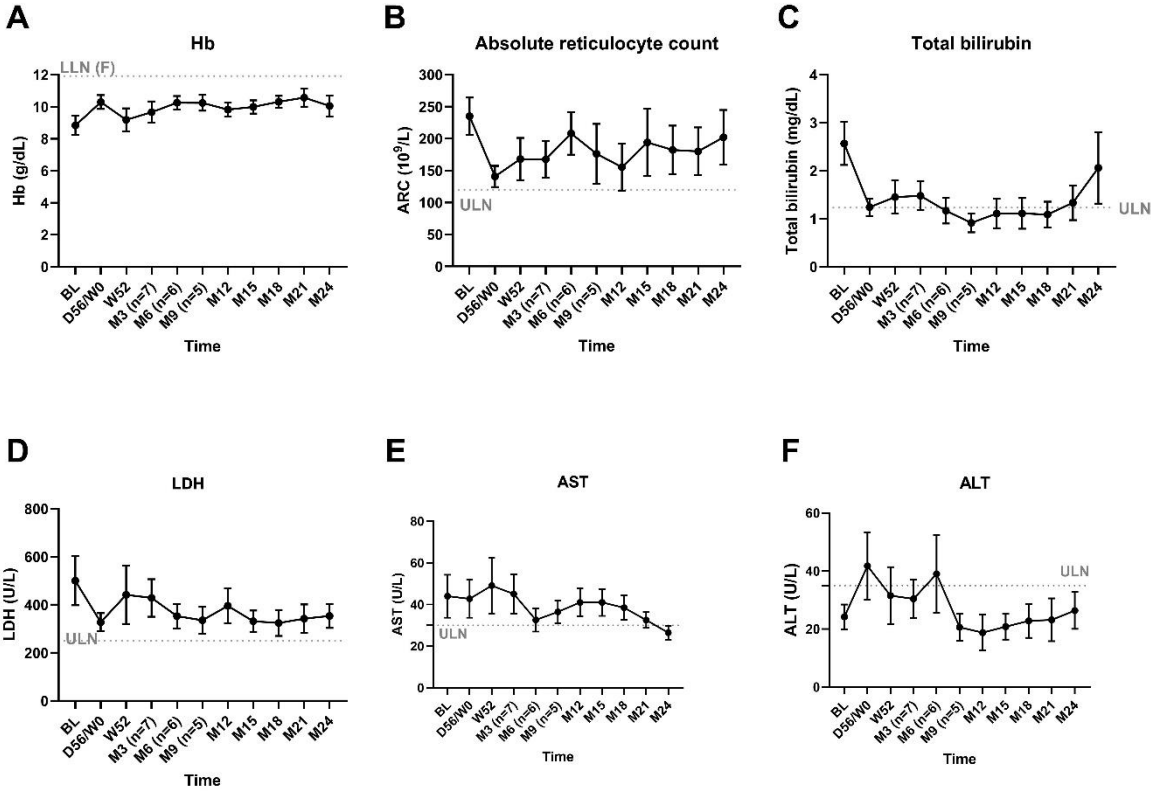
Accepted: February 19, 2026.

Early view: February 26, 2026.

Published under a CC BY license 

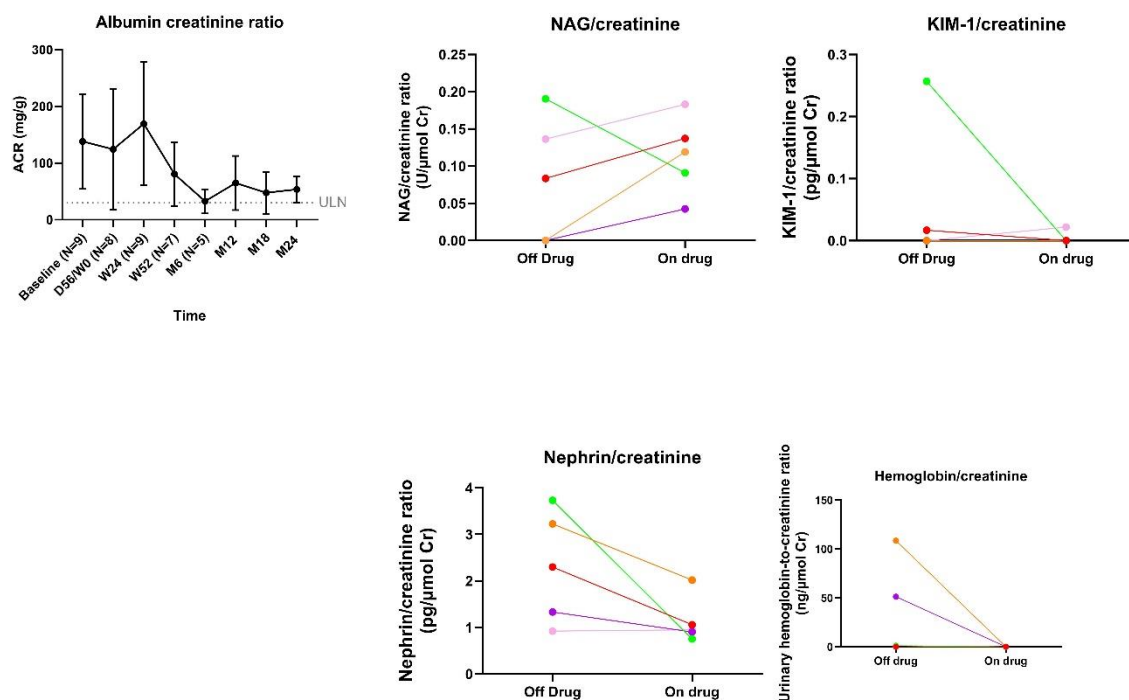
Supplementary Figures:

Supplementary Figure 1



Supplementary Figure 1: Improvements in laboratory parameters during the three-year study duration. (A) Hemoglobin (Hb) levels over time. (B) Absolute reticulocyte count (ARC) over time. (C) total bilirubin over time. (D) Lactate dehydrogenase (LDH) over time. (E) Aspartate aminotransferase (AST) over time. (F) Alanine aminotransferase (ALT) over time. BL: Baseline, D: Day, W: Week, M: Month, LLN(F): Lower limit of normal, F: female, ULN: upper limit of normal

Supplementary Figure 2



Supplementary Figure 2. Top left figure mean albumin creatinine ratio (ACR) over time. ULN: Upper limit of normal. Ratio of absolute changes in renal biomarkers, corrected for urinary creatinine, comparing baseline values with samples collected either at the end of the FDEP period (year 1) or mid-PFDEP (year 2). NAG =b-N-acetylglucosaminidase, KIM-1=Kidney injury molecule-1, FDEP= Fixed Dose Extension Period until year 1. PFDEP= Prolonged Fixed Dose Extension Period