

Conference Abstracts

Vol. 6, Issue Supplement 2, 2026

Durable Complete Remission Represents Treatment-Responsive Biology Rather Than Baseline Disease Characteristics in Higher-Risk Myelodysplastic Syndromes

Julie Braish, MD^{1,a}; Najla Al Ali, MS.¹; Zena Komrokji, RA¹; Zhuoer Xie, MD¹; Onyee Chan, MD¹; Andrew Kuykendall, MD¹; Seongseok Yun, MD¹; Rory Shallis, MD¹; Sam Reynolds, MD¹; Alison Walker, MD¹; Jeffrey Lancet, MD¹; Eric Padron, MD¹; David Sallman, MD¹; Rami Komrokji, MD¹

¹ H. Lee Moffitt Cancer Center and Research Institute, Tampa, FL, USA

Corresponding author

^a Julie Braish, MD, H. Lee Moffitt Cancer Center and Research Institute, Tampa, FL, USA
Email: juliebreish.24@gmail.com

Keywords: High-Risk MDS, Durable Complete Response, Drug Development

Submission received: 2026-08-06 / Accepted: 2026-08-25 / Published: 2026-09-18

<https://doi.org/10.53876/001a.129788>

Abstract

Background

Complete remission (CR) is widely used as a surrogate endpoint in higher-risk myelodysplastic syndromes (HR-MDS). Despite promising CR rates in early-phase trials, numerous therapies for HR-MDS have failed to improve overall survival (OS) in phase III studies, highlighting limitations of CR as a surrogate endpoint. We hypothesized that durable CR, rather than CR alone, captures meaningful clinical benefit and that the biology underlying durable remission may not be fully explained by baseline disease characteristics.

Methods

We retrospectively analyzed 980 adults with HR-MDS treated with first-line hypomethylating agent (HMA)-based therapy at Moffitt Cancer Center (2005–2024). Durable CR was defined as CR lasting ≥ 6 months. Survival was assessed using a prespecified 6-month landmark analysis. Baseline clinical, cytogenetic, and molecular variables were evaluated using multivariable logistic regression and exploratory gradient boosting.

Results

Among 980 patients (median age, 72 years), 25% harbored TP53 mutations, 44% had very-high-risk cytogenetics, and 83% of evaluable patients were high risk by IPSS-M. Overall, 98 patients (10%) achieved durable CR. At the 6-month landmark, durable CR was associated with significantly improved OS versus no durable CR (HR 0.66, 95% CI 0.53–0.82; $P < 0.001$), representing a 34% reduction in mortality and improved median post-landmark OS (14.0 vs 9.2 months), whereas CR lasting < 6 months conferred no survival benefit. Prediction from baseline characteristics was modest (AUC 0.665) and remained poor with gradient boosting (cross-validated AUC 0.56). Higher hemoglobin (OR 1.14, 95% CI 1.01–1.29; $P = 0.042$) and bone marrow blast percentage (OR 1.05, 95% CI 1.00–1.09; $P = 0.032$) independently predicted durable CR, whereas TP53 status, recurrent myeloid mutations, cytogenetic risk, and frontline venetoclax exposure did not.

Conclusion

Durable CR captures clinically meaningful treatment-responsive biology beyond baseline disease characteristics, supporting its adoption as a surrogate endpoint for early-phase HR-MDS

trials while underscoring the need for dynamic biomarkers to guide therapeutic development.

© 2021–2026 Binaytara. Website design and layout all rights reserved. Articles published in the International Journal of Cancer Care and Delivery are open access. Authors retain copyright. All articles are distributed under the terms of the Creative Commons Attribution-ShareAlike 4.0 International (CC BY-SA 4.0) license, which permits unrestricted use, distribution, and reproduction in any medium, provided the original work is properly cited and derivative works are shared under the same license.