CML Survivorship: Beyond Survival to Quality of Life

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The management of chronic myeloid leukemia (CML) has evolved significantly, with tyrosine kinase inhibitors (TKIs) transforming a once fatal diagnosis into a chronic, manageable condition. This success, however, necessitates a re-evaluation of treatment goals beyond mere survival, focusing instead on achieving a high quality of survivorship for patients living with CML.

KEY TAKEAWAYS

- The Pivot: CML management now prioritises quality of life and treatment-free remission (TFR) alongside survival.
- The Data: Approximately 50% of eligible CML patients can achieve TFR, reducing long-term TKI side effects.
- The Action: Clinicians should assess patient eligibility for TFR and actively manage TKI-related adverse events to improve patient experience.

The landscape of chronic myeloid leukemia (CML) treatment has undergone a profound transformation since the introduction of imatinib and subsequent second- and third-generation tyrosine kinase inhibitors (TKIs). These agents have dramatically improved patient outcomes, with 10-year overall survival rates now exceeding 85% for patients in chronic phase CML.1 This therapeutic success has shifted the clinical focus from simply extending life to optimising the quality of life for CML survivors. The EHA 2026 discussion on CML survivorship highlighted the critical need to address long-term TKI-related adverse events, the potential for treatment-free remission (TFR), and the psychosocial impact of living with a chronic malignancy.2

Long-term TKI therapy, while effective, is associated with a range of adverse events that can significantly impair a patient's quality of life. Common side effects include fatigue, gastrointestinal disturbances, fluid retention, musculoskeletal pain, and dermatological issues.3 More serious adverse events, such as cardiovascular complications (e.g., hypertension, arterial occlusive events) and pulmonary hypertension, are particularly associated with second- and third-generation TKIs like nilotinib and ponatinib.4 These toxicities often necessitate dose reductions, treatment interruptions, or even discontinuation, which can compromise treatment efficacy. Therefore, proactive management of TKI-related adverse events is paramount to maintaining patient adherence and improving overall well-being.5 The mechanism of action of TKIs involves inhibiting the constitutively active BCR-ABL1 tyrosine kinase, which is the oncogenic driver in CML. While highly effective at targeting the malignant clone, this inhibition can also affect other kinases and cellular pathways, leading to the observed off-target toxicities. The specific TKI used, its selectivity profile, and individual patient comorbidities all influence the spectrum and severity of adverse events experienced.

Achieving Treatment-Free Remission

A major advancement in CML management aimed at improving survivorship is the concept of treatment-free remission (TFR). TFR allows eligible patients to discontinue TKI therapy without molecular relapse, thereby eliminating TKI-related side effects and improving quality of life.6 Eligibility for TFR typically requires a sustained deep molecular response (DMR), often defined as MR4 or MR4.5, for a minimum duration (e.g., 2-3 years) while on TKI therapy.7 Studies like the EURO-SKI and STIM trials have demonstrated that approximately 50% of patients who meet strict eligibility criteria can successfully achieve TFR.8,9 These trials enrolled adult patients with chronic phase CML who had achieved and maintained a deep molecular response for a predefined period. Patients were then carefully monitored for molecular relapse after TKI cessation, typically through quantitative PCR for BCR-ABL1 transcripts.

The process of TKI discontinuation requires careful patient selection and rigorous molecular monitoring. Patients who relapse after TKI cessation typically regain DMR upon re-initiation of the same TKI, suggesting that TFR is a safe and reversible strategy for many.10 However, the psychological impact of discontinuing therapy and the anxiety associated with potential relapse must be addressed through comprehensive patient education and support.11 The decision to attempt TFR should be made collaboratively between the patient and clinician, considering individual patient factors, TKI history, and molecular response kinetics.12 The long-term success of TFR is influenced by factors such as duration of TKI therapy, depth of molecular response prior to discontinuation, and the specific TKI used. Further research continues to refine patient selection criteria and monitoring protocols to optimize TFR success rates and ensure patient safety.

Beyond TKI-related toxicities and TFR, the psychosocial burden of CML survivorship warrants attention. Patients often experience anxiety, depression, and fear of relapse, even when in deep molecular remission.13 Financial toxicity, due to the high cost of TKIs and ongoing medical care, also represents a significant challenge for many patients.14

Comprehensive survivorship care plans should therefore integrate psychological support, financial counselling, and regular assessment of health-related quality of life (HRQoL) to address these multifaceted needs.15 The prevalence of CML is estimated to be approximately 1-2 cases per 100,000 adults per year, with an increasing number of long-term survivors due to effective TKI therapy. This growing population necessitates a greater focus on the long-term implications of living with CML.

While significant progress has been made in extending the lives of CML patients, the focus must now expand to ensuring these extended lives are lived with optimal quality. This involves a proactive approach to managing TKI toxicities, carefully selecting patients for TFR, and providing holistic psychosocial support. Further research is needed to identify biomarkers that predict successful TFR, develop novel strategies to manage refractory TKI side effects, and standardise HRQoL assessments in CML clinical trials and routine practice.16 Limitations in current understanding include the precise mechanisms underlying sustained TFR in some patients and the optimal management strategies for patients who experience persistent, low-grade TKI-related adverse events despite dose adjustments. Addressing these gaps will further enhance the quality of life for CML survivors.

CLINICAL IMPLICATIONS

The shift in CML management from mere survival to quality survivorship presents a compelling challenge for clinicians. The evidence supporting treatment-free remission is robust, yet many eligible patients remain on lifelong TKI therapy, enduring preventable side effects. This suggests a gap in translating trial data into routine clinical practice, possibly due to a lack of confidence in managing TKI discontinuation or insufficient patient education regarding TFR eligibility and monitoring requirements. Clinicians must proactively identify patients who meet the stringent criteria for TFR and engage them in shared decision-making, ensuring they understand both the benefits of TKI cessation and the necessity of close molecular surveillance.

From an industry perspective, the success of TFR poses a unique dilemma. While TKIs have been blockbuster drugs, the increasing emphasis on discontinuing therapy could impact long-term market dynamics. This necessitates a pivot towards demonstrating value beyond indefinite treatment, perhaps through supporting robust TFR monitoring platforms or investing in therapies for patients who fail TFR or experience significant TKI intolerance. Furthermore, the high cost of TKIs remains a barrier to access and contributes to financial toxicity for patients, even in systems with universal healthcare. Pharmaceutical companies, alongside healthcare systems, must address these economic realities to ensure equitable access to optimal CML care, including the option of TFR.

For patients, the prospect of living without daily medication and its associated side effects is transformative. However, the psychological burden of discontinuing a life-saving drug, coupled with the anxiety of potential relapse, cannot be underestimated. Patient advocacy groups and healthcare providers have a responsibility to provide comprehensive support, including psychological counselling and clear, consistent communication about molecular monitoring results. The goal is not just to extend life, but to restore a sense of normalcy and reduce the constant reminder of a cancer diagnosis, thereby truly achieving quality survivorship.

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