Isabelle Lundqvist | isabelle.lundqvist@novartis.com

PCR27

Utility estimates for health-states associated with chronic myeloid leukemia: A systematic literature review

Isabelle Lundqvist[1], Shaun Walsh[2], Pauline Gilbert[3], Devarshi Bhattacharyya[4], Aditi Kataria[4], Nitin Kaushik[4]

[1]Novartis Sverige AB, Sweden; [2]Novartis Ireland Ltd, Dublin, Ireland; [3]Novartis Pharma AG, Basel, Switzerland; [4]Novartis Healthcare Pvt. Ltd., Hyderabad, India

KEY FINDINGS & CONCLUSIONS

- The utility estimates for CML varied, likely due to differences in assessment methodologies, study populations, geographies and variability in the time of assessment.
- The EQ-5D emerged as the most frequently used tool for utility estimation, consistently reporting higher values for CML-CP health states compared to other tools such as TTO, SG, and VAS.
- This review highlights the worsening QoL associated with disease progression in CML, while showing the associated benefit for patients achieving TFR.
- These findings contribute to a better understanding of the impact of CML on patient's QoL.
- Furthermore, these findings emphasize the importance of considering QoL alongside efficacy outcomes when a decision needs to be made regarding avoidance of treatment switching to later lines.

This research was funded by Novartis Pharma AG
Poster presented at ISPOR Europe 2024, held in-person on 17-20 November 2024

INTRODUCTION

- Chronic myeloid leukemia (CML) is a myeloproliferative neoplasm having worldwide prevalence of 3.25 per 100,000 individuals and contributes to approximately 30% of adult leukemia cases worldwide.
- To assess new therapies in CML, it is imperative to define their value using utility estimates in economic models submitted to reimbursement agencies.
- A systematic review was conducted to identify studies and health technology assessment (HTA) reports reporting health state utilities for patients with CML.

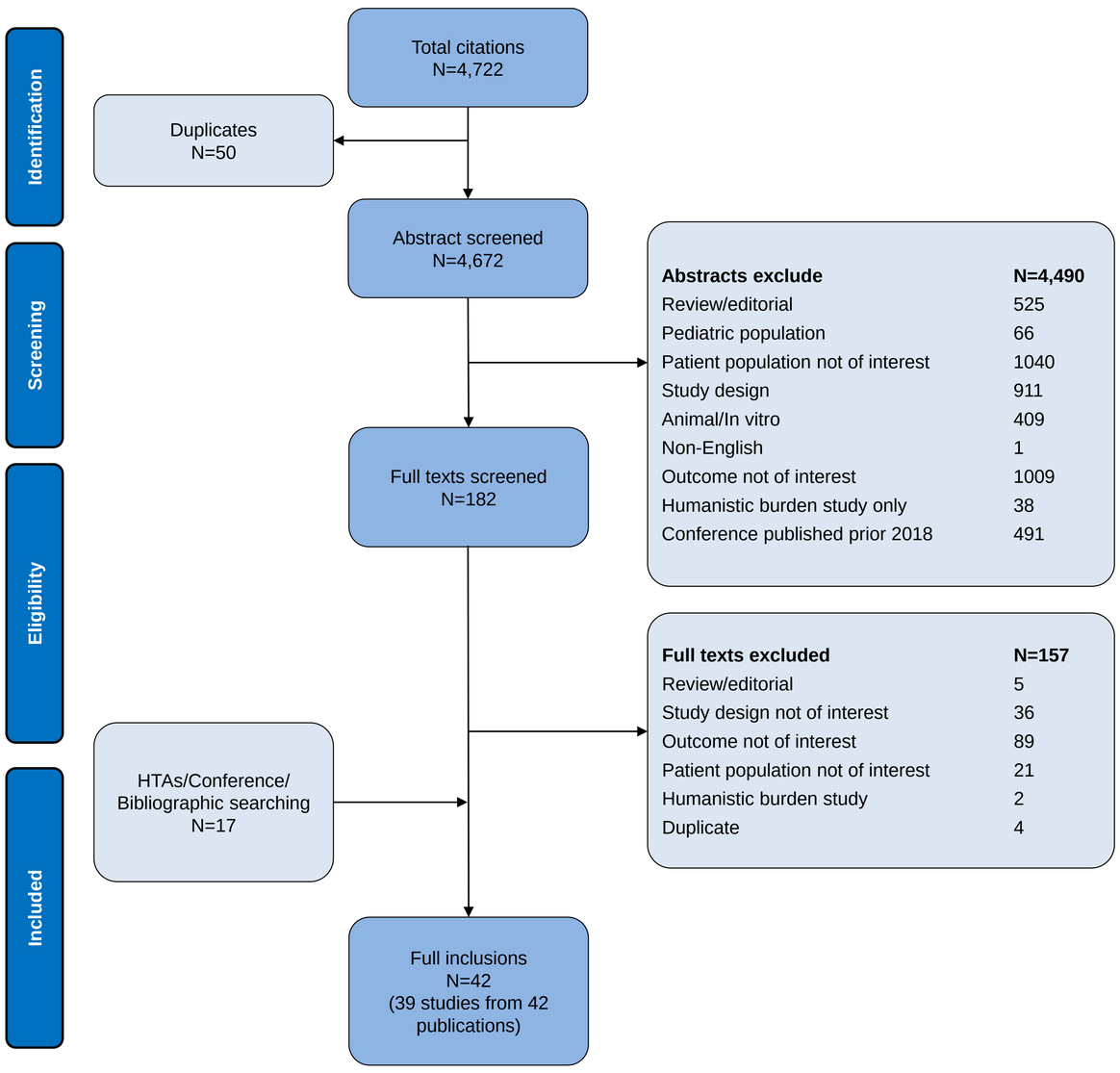
METHODS

- Key biomedical databases (MEDLINE®, EMBASE® and Cochrane) and health technology assessment (HTA) reports of key countries published between 2000 to 8-Feb-2024 were searched for English language studies.
- Studies providing utility or disutility data for adults with CML, disutility data for patients with blood cancers other than CML were considered.
- Screening of studies and data extraction were conducted by two independent reviewers.
- The methodological quality and bias of included studies were evaluated using the National institute for health and clinical excellence-Health state utility-values (NICE-HSUV).

RESULTS

- Thirty-nine studies including HTAs met the inclusion criteria (**Figure 1**) and were extracted.
- Most of the identified studies were predominantly conducted in the UK or multinational.

Figure 1: PRISMA flow diagram



Patient characteristics

- Patients were either in chronic phase, advanced phase (accelerated phase (AP), and blast phase (BP)) or mixed phase (both chronic and advanced) of CML.
- In the majority of studies, mean age was ≥40 years with a male predominance.

Utilities of CML

- Twenty-two studies reported data on health utility for CML across different health states and responses.
- The tools used to estimate the utilities in the identified studies were the EuroQol-5D (EQ-5D), time trade off (TTO), visual analog scale (VAS), and standard gamble (SG).
- Average utility-values for CML health states varied widely, depending on factors such as the method of elicitation, geographic location, and study population.
- Overall, utility-values across studies demonstrated a declining trend as patients transitioned from their initial-line of therapy and disease-progression.
- Different disease phases (chronic phase (CP), accelerated phase (AP), and blast phase (BP)) were associated with distinct utility-values, reflecting the severity of CML progression.
  - The range of utility-values measured using EQ-5D for CML-CP patients was 0.72-0.89, while for patients with CML-AP it was 0.53-0.78, and for patients with CML-BP it ranged between 0.29-0.59.
  - These lower utility-values highlight the impact of disease progression on patients' quality of life (QoL).
- The utility estimation for CML-CP health states using different tools yielded the following values: 0.72-0.89 using EQ-5D, 0.72 using TTO, 0.39 using SG, and 0.29 using VAS. The data for TTO, SG, and VAS comes from Guest, 2012 study.
- Notably, Foulon, 2021 is the only study that provided insights into utility-values across different health states and line of treatments.
  - According to this study, the mean utility-value among CML-CP patients was measured at 0.72, with a slightly higher value (0.76) observed for patients receiving first-line treatment compared to those receiving second or third-line treatment (0.68).
  - Additionally, patients achieving treatment-free remission (TFR) reported a higher mean utility-value of 0.84, indicating the positive impact of remission on patients' quality of life.
- The utility-values for different health states are presented in **Table 1**.

Table 1: Utility-values for different CML health states

Health state	Scale	Study	Utility
CML-CP	EQ-5D	**Foulon, 2021**	**0.72**
		Kuo, 2014	0.79
		NICE_TA425	0.85
		Rochau, 2012	0.89
	TTO	Guest, 2012	0.72
	SG	Guest, 2012	0.39
	VAS	Guest, 2012	0.29
CML-AP	EQ-5D	NICE_TA451	0.53
		Whiteley, 2013	0.78
		NICE_TA425	0.59
CML-BP	EQ-5D	NICE_TA451	0.29
		NICE_TA813	0.43
		Whiteley, 2013	0.66
		NICE_TA425	0.59
TFR	EQ-5D	**Foulon, 2021**	**0.84**
	TTO	Guest, 2014	0.97
	SG	Guest, 2014	0.87
MR	TTO	Guest, 2014	0.94
	SG	Guest, 2014	0.8
	VAS	Guest, 2012	0.74
CyR	TTO	Guest, 2012	0.89
	VAS	Guest, 2012	0.58
	SG	Guest, 2012	0.61
CCyR	EQ-5D	NICE_TA451	0.91
No CCyR	EQ-5D	NICE_TA451	0.73
CMR	TTO	Guest, 2014	0.96
	SG	Guest, 2014	0.85
CML-CP with 1L treatment	EQ-5D	**Foulon, 2021**	**0.76**
		Kuo, 2014	0.83
CML-CP with 2L treatment	EQ-5D	**Foulon, 2021**	**0.68**
		Whiteley, 2013	0.83
CML-CP with 3L treatment	EQ-5D	**Foulon, 2021**	**0.68**
		Whiteley, 2013	0.8
		NICE_TA813	0.84

AP: Accelerated phase; BP: Blast phase; CML: Chronic myeloid leukemia; CMR: Complete molecular response; CP: Chronic phase; CCyR: Complete cytogenetic response; CyR: Cytogenetic response; EQ-5D: EuroQol group 5D; MR: Molecular response; SG: Standard gamble; TFR: Treatment free remission; TTO: Time trade off; VAS: Visual analog scale

Disutility due to CML

- The disutility-value was worse for advanced phase CML than CML-CP (-0.22 versus -0.09).
- Disutility-values for adverse-events in CML patients receiving different TKIs ranged from -0.049 to -0.01.
- These adverse events include anaemia, neutropenia, diarrhoea, and thrombocytopenia.
- The disutility-values associated with CML are presented in **Table 2**.

Table 2: Health disutility for CML

Study	Population	Disutility-value
Bonifacio, 2022	CML-CP with 2L TKI	-0.09
	Advanced phase CML	-0.22
NICE_TA70	Blast phase CML	-0.09
NICE_TA401	Low risk population (sokal score <0.8) received SCT	-0.041
	High risk population (sokal score >1.2) received SCT	-0.079
NICE_TA425	Grade ≥3 AEs related to imatinib	-0.027
	Grade ≥3 AEs related to nilotinib	-0.049
	Grade ≥3 AEs related to SCT	-0.079
	Grade ≥3 AEs related to hydroxyurea	0
NICE_TA426	Patients received SCT	-0.079
	AEs related to nilotinib	-0.01
	AEs related to imatinib	-0.016
	AEs related to dasatinib	-0.019
	AEs related to hydroxyurea	0
	AEs	Anaemia: -0.073 Diarrhoea: -0.048 Neutropenia: -0.16 Thrombocytopenia: -0.05
NICE_TA813	CML-CP patients with AEs	Anaemia: -0.09 Headache: -0.18 Lipase increased: -0.07 Platelet count decreased: -0.02 Pruritis: -0.09 Hypocalcaemia: -0.06 Superficial oedema: -0.09 Alanine aminotransferase increased: -0.05 Neutropenia: -0.05 Neutrophil count decreased: -0.05 Thrombocytopenia: -0.05

AE: Adverse event; CML: Chronic myeloid leukemia; CP: Chronic phase; SCT: Stem cell transplant; TKI: Tyrosine kinase inhibitors

References

1. Foulon S, et al. Qual Life Res. 2021;30(7):2021-32.
2. NICE 2016 (TA425).
3. NICE 2017 (TA451).
4. Whiteley J, et al. Value Health. 2013;16(7):A387.
5. NICE 2022 (TA813).
6. Guest JF, et al. Leuk Lymphoma. 2012;53(5):928-33.
7. Guest JF, et al. Leuk Lymphoma. 2014;55(8):1870-5.
8. Kuo K, et al. Value Health. 2014;17(3):A92-A3.
9. Rochau U, et al. Value Health. 2012;15(7):A429.
10. NICE 2016 (TA426).
11. NICE 2003 (TA70).
12. NICE 2013 (TA401).
13. Bonifacio M, et al. Pharmacoecon Open. 2022;6(1):95-104.

Acknowledgements

The authors acknowledge Nitin Kaushik (Novartis, Hyderabad) for providing medical writing assistance with this poster.

Conflict of Interest

Isabelle Lundqvist, Shaun Walsh, Pauline Gilbert, Devarshi Bhattacharyya, Aditi Kataria and Nitin Kaushik are employees of Novartis.

Scan to obtain:

- Poster

https://www.medicalcongressposters.com//Default.aspx?doc=a8f8b