Health-related Quality of Life Measured by EQ-5D Utility Scores among Patients with TGCT: A Trial-based Analysis of EQ-5D Data from Patients in the MOTION Trial

PCR107

Agnes Benedict[1], Balazs Dobi[2], Nicholas Zeringo[3], Paul Hewitt[4], Ulrike Beckert[4], Venediktos Kapetanakis[5], Brooke Harrow[3]

[1]Thermo Fisher Scientific, Vienna, Austria; [2]Thermo Fisher Scientific, Budapest, Hungary; [3]Deciphera Pharmaceuticals, LLC, Waltham, MA, USA; [4]Deciphera Pharmaceuticals, AG, Zug, Switzerland; [5]Thermo Fisher Scientific, London, UK

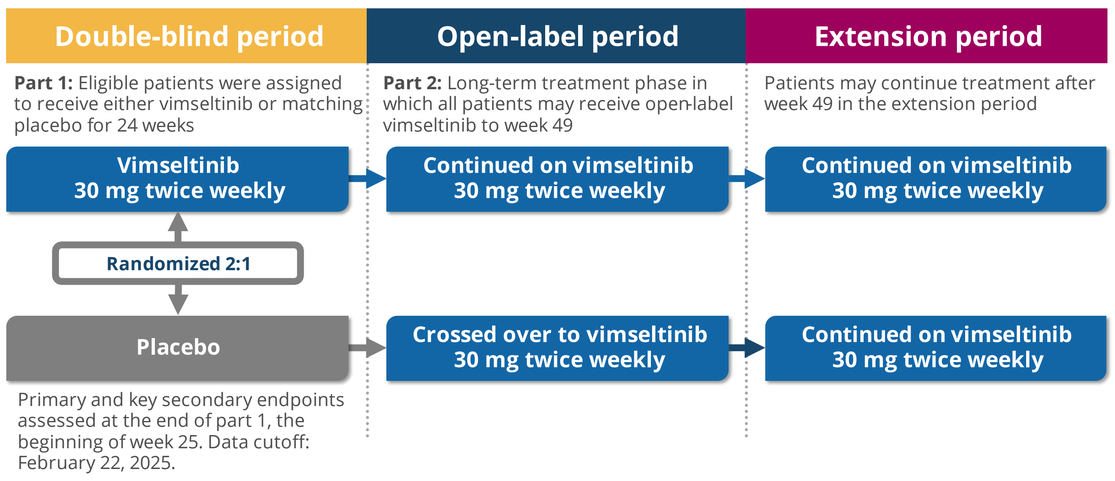
Introduction

- **Vimseltinib showed a significant objective response rate (ORR) per RECIST v1.1 and clinically meaningful functional, symptomatic, and quality of life improvement vs. placebo, providing an effective treatment option for patients with tenosynovial giant cell tumor (TGCT) not amenable to surgery.[1]**
- TGCT is a locally aggressive neoplasm that involves the synovium, bursae, or tendon sheath. Tumor location varies and is often associated with joint destruction, pain, stiffness, and limited range of motion.
- Although surgery is the standard of care in TGCT, some cases are not amenable due to significant risk of morbidity and/or incomplete resection.[2]
- Vimseltinib is an oral, switch-control tyrosine kinase inhibitor designed to selectively and potently inhibit the CSF1 receptor (CSF1R).
  - Vimseltinib was approved in February 2025 by the US Food and Drug Administration.[3]
  - In September 2025, the European Commission approved vimseltinib for the treatment of adult patients with TGCT associated with clinically relevant physical function deterioration and in whom surgical options have been exhausted or would induce unacceptable morbidity or disability.[4]
- MOTION is an international, randomized, double-blind, placebo-controlled, phase 3 trial in adults with symptomatic TGCT whose disease is not amenable to surgery (NCT05059262).[5]
  - The primary outcome in the trial was ORR per RECIST v1.1 by IRR at week 25 and was 40% for the vimseltinib arm and 0% for the placebo arm. Similarly, 67% of patients receiving vimseltinib had an objective response by tumor volume score per IRR compared with 0% in the placebo group.
  - Patients on vimseltinib reported improvement in several important measures, including range of motion, physical function, stiffness, pain, and overall health.

OBJECTIVE

- This study aimed to characterize health-related quality of life (HRQoL), longitudinally, among patients with TGCT in terms of EQ-5D utility values and examine the impact of vimseltinib on HRQoL, based on the MOTION trial data.

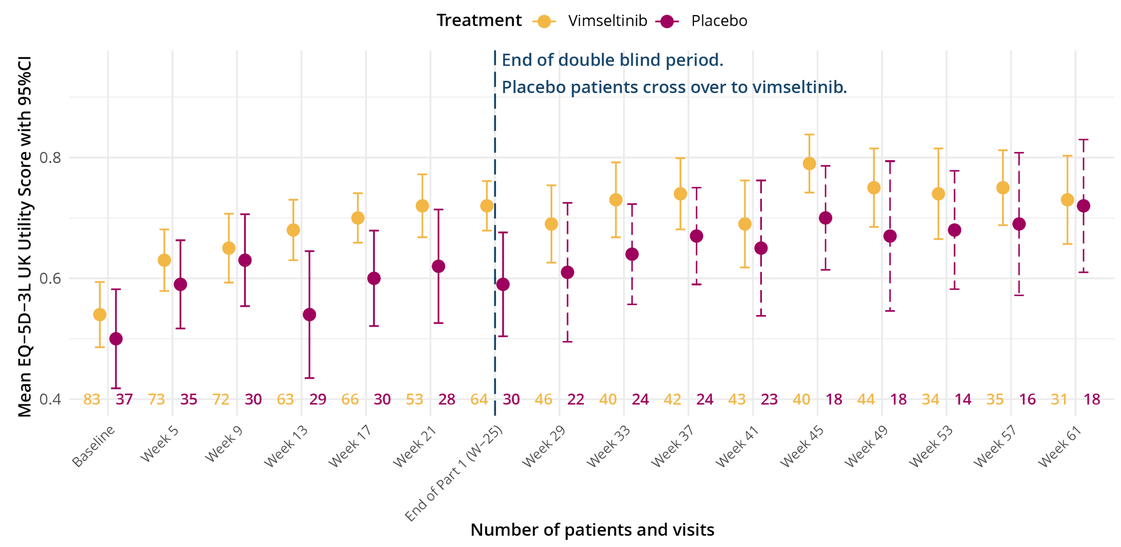
Figure 1. MOTION study design



Methods

- EQ-5D-5L values collected from 123 patients (data cutoff: 22 February 2025) were mapped to age- and sex-adjusted EQ-5D-3L UK utility score values using the National Institute for Health and Care Excellence Decision Support Unit's published mapping algorithm.[6]
- There were three patients on placebo with only baseline EQ-5D observations and no observations at any other visits. These patients were excluded from the regression analysis.
- EQ-5D utility scores using UK tariffs were analyzed using single- and multiple-covariate mixed models for repeated measures (MMRM). Models were compared in terms of goodness-of-fit based on Akaike Information Criteria (AIC)/Bayesian Information Criteria (BIC) and clinical plausibility.
- Predictors of EQ-5D-3L UK utility scores tested included time-varying symptom variables measured in MOTION and also used in clinical practice to characterize disease activity:
  - Mean Brief Pain Inventory (BPI) worst **pain** in the last 24 hours on a numeric rating scale (NRS) (0–10).
  - Worst **stiffness** in the last 24 hours NRS (0–10).
  - **Range of motion**: measures ability to move the affected joint by goniometry compared to a reference standard.
  - Patient-Reported Outcomes Measurement Information System **physical function** (PROMIS-PF; TGCT-specific), a measure assessing the tumor location specific physical function.
- Patient and disease characteristics, including age, gender, and disease type, and the impact of treatment were tested as predictors of EQ-5D utility scores.

Figure 2. EQ-5D-3L utility score distribution over trial visits



Note: End of Part 1 indicates the end of the double-blind period, when all remaining placebo patients crossed over to vimseltinib. Values shown for placebo after that point reflect patients who started vimseltinib.

Results

- Baseline characteristics are shown in **Table 1**. Mean baseline EQ-5D-3L UK utility score was **0.53** (SD 0.25) vs. **0.88** for age- and gender-adjusted UK general population utility. At the end of the double-blind period (25 weeks), the mean EQ-5D-3L UK utility scores were 0.72 (SD 0.17) on vimseltinib vs. 0.59 (SD 0.24) on placebo (**Table 2**).
- Baseline EQ-5D was available for all 123 patients; at the end of the double-blind period, availability of EQ-5D-3L UK utility scores was 75% and 77% for placebo and vimseltinib patients, respectively.
- The MMRM, including treatment as the only covariate, estimated a higher mean utility for vimseltinib by 0.080 (SE 0.034) over placebo.
- The effect of **age**, **gender**, and disease type on EQ-5D-3L UK utility score was tested in univariate models, multiple regression models with treatment, and multiple regression models with treatment and interaction of the given variable with treatment.
  - None of the above variables had a significant effect on HRQoL.
  - In all cases, the effect of vimseltinib remained significant when investigated together, i.e., the treatment effect was not explained by these variables.
  - The interactions terms between the investigated variables and treatment were never significant, i.e., the treatment effect was consistent within subgroups defined by these variables.

Table 1. Baseline Patient Characteristics

Variable	Variable level	Vimseltinib (n=83)	Placebo (n=40)	Total (n=123)
Age (mean [SD])		43.8 (13.9)	42.5 (13.7)	43.4 (13.8)
Sex (%)	**Female**	46 (55)	27 (68)	73 (59)
	Male	37 (45)	13 (33)	50 (41)
EQ-5D-3L UK utility score (mean [SD])		0.54 (0.25)	0.50 (0.25)*	0.53 (0.25)
Worst pain (mean [SD])		5.5 (2.1)	6.0 (1.8)	5.7 (2.0)
PROMIS-PF (mean [SD])		39.0 (6.1)	38.5 (6.0)	38.8 (6.1)
Tumor location (%)	**All other limbs**	10 (12)	4 (10)	14 (11)
	Lower limb	73 (88)	36 (90)	109 (89)

*n=37, as three placebo patients with only baseline EQ-5D observations and no further ones were omitted
Abbreviation: PROMIS-PF = Patient-Reported Outcomes Measurement Information System physical function

Table 2. Patient Numbers and EQ-5D-3L UK Utility Scores in the Double-blind Period

Visit type	Vimseltinib			Placebo		
	N	Mean utility score	SD	N	Mean utility score	SD
Baseline	83	**0.54**	0.25	37	**0.50**	0.25
Week 5	73	**0.63**	0.22	35	**0.59**	0.22
Week 9	72	**0.65**	0.25	30	**0.63**	0.21
Week 13	63	**0.68**	0.20	29	**0.54**	0.29
Week 17	66	**0.70**	0.17	30	**0.60**	0.22
Week 21	53	**0.72**	0.19	28	**0.62**	0.25
End of Part 1 (Week 25)	64	***0.72***	***0.17***	30	***0.59***	***0.24***

Abbreviations: SD = standard deviation

- The final multivariable model included baseline EQ-5D utility, Worst Pain NRS with an increasing linear association, and PROMIS-PF with a decreasing non-linear association.
- The non-linear relationship between PROMIS-PF and the EQ-5D-3L UK utility scores was modeled using a linear and a square root term. Other non-linear models were also investigated and checked by AIC/BIC and clinical plausibility before the square root model was chosen.
- The final regression model can explain the vimseltinib treatment effect on utility using the symptoms of the disease. That is, including treatment into the model with pain NRS and physical function resulted in a positive but non-significant coefficient for it.

Table 3. Final Regression Model on EQ-5D-3L UK Utility Scores

Variable	Estimate (95%CI)	*P*-value
Intercept	-6.327 (-7.147, -5.486)	<0.001
Baseline utility	0.024 (0.007, 0.040)	0.006
Linear term of PROMIS-PF*	-0.127 (-0.145, -0.108)	<0.001
Square root of PROMIS-PF	1.918 (1.667, 2.162)	<0.001
Worst pain NRS	-0.018 (-0.023, -0.014)	<0.001

Abbreviations: CI = confidence interval; NRS = numeric rating scale; PROMIS-PF = Patient-Reported Outcomes Measurement Information System physical function

LIMITATIONS

- Due to the crossover of placebo patients to vimseltinib following the 24-week double-blind period, there are limited data on long-term treatment effect vs. placebo.
- Similarly, the number of patients reduced further by week 57, limiting statistical evidence of long-term trajectories of symptoms and HRQoL.

CONCLUSIONS

- Patients with TGCT not amenable for surgery in the MOTION trial had poor HRQoL at baseline, which is comparable to other chronic diseases such as multiple sclerosis.[7]
- Vimseltinib significantly improved PROMIS-PF and worst pain NRS over time, leading to increases in HRQoL as measured by EQ-5D-3L UK utility score (**mean increase of 0.18 by week 25**). These results are independent of age, gender, and disease type.
- These results fill a gap in describing HRQoL for patients with TGCT not amenable for surgery and will support modeling the changes expected in EQ-5D due to changes in key symptoms of TGCT, facilitating health technology assessment.

PRESENTED AT THE PROFESSIONAL SOCIETY FOR HEALTH ECONOMICS AND OUTCOMES RESEARCH (ISPOR)—EUROPE 9–12 NOVEMBER 2025 | GLASGOW, SCOTLAND, UK

DISCLOSURES: Balazs Dobi and Venediktos Kapetanakis are employees of PPD™ Evidera™ Health Economics & Market Access, Thermo Fisher Scientific. Agnes Benedict was employed by Thermo Fisher Scientific at the time this study was conducted. This study is sponsored by Deciphera Pharmaceuticals, LLC, a member of ONO Pharma. Editorial and graphic design support was provided by Michael Grossi and Kawthar Nakayima of Thermo Fisher Scientific.

REFERENCES: 1. Gelderblom H, et al. *Lancet*. 2024;403(10445): 2709-2719. **2.** Stacchiotti S, et al. *Cancer Treat Rev*. 2023;112:102491. **3.** https://clinicaltrials.gov/study/NCT05059262. **4.** ROMVIMZA™. *Full prescribing information. U.S. FDA.* 2025. **5.** ROMVIMZA™. *EU Summary of Product Characteristics. European Medicines Agency.* 2025. **6.** Hernández Alava M, et al. *PharmacoEconomics*. 2023;41:199–207. **7.** Young CA, et al. *Value Health*. 2025; 1098-3015(25).

PRESENTING AUTHOR: Agnes Benedict | langsisakos@gmail.com