Report ROMVIMZA® - Vimseltinib

Product & Mechanism of action

Substance: Vimseltinib

Brand Name: Romvimza

Originator/licensee: Deciphera Pharmaceuticals (Netherlands) B.V.

Classification: NCE

ATC code: L01FX15

Orphan Status:
Eu: Yes
Us: No

Mechanism of action: Vimseltinib, an antineoplastic agent, is a protein kinase inhibitor that targets CSF1R. The CSF1/CSF1R signalling axis has a critical role in the development of TGCT. Vimseltinib exerts its antineoplastic activity by inhibiting CSFR1 expressing cells and blocking downstream signalling pathways that promote tumour growth and macrophage proliferation [1].

Authorized indications Licensing status

Authorized Indication:
EMA: Vimseltinib is indicated for treatment of adults with symptomatic TGCT associated with clinically relevant physical function deterioration and in whom surgical options have been exhausted or would induce unacceptable morbidity or disability [1].

FDA: Vimseltinib is indicated for treatment of adults with symptomatic TGCT for which surgical resection will potentially cause worsening functional limitation or severe morbidity [2].

Route of administration: OS

Licensing status
EU CHMP P.O. date: 24/07/2025
FDA M.A. date: 14/02/2025

EU Speed Approval Pathway: No
FDA Speed Approval Pathway: Yes

ABBREVIATIONS:
AE: Adverse Event
CHMP: Committee for Medicinal Products for Human Use
CI: Confidential Interval
CSF1: Colony stimulating factor 1
CSF1R: Colony stimulating factor 1 receptor
HR: Hazard Ratio
M.A.: Marketing Authorization
ORR: Objective response rate
OS: Oral administration
PFS: Progression-Free Survival
P.O.: Positive Opinion
PS: Performance Status
Pts: Patients
RECIST: Response Evaluation Criteria in Solid Tumours
TGCT: Tenosynovial giant cell tumour
TEAE: Treatment-emergent related AEs
WHO: World Health Organization

Essential therapeutic features

Summary of clinical EFFICACY:
MOTION (NCT05059262) was a multicentre, randomised, double-blind, placebo-controlled, phase 3 trial carried out in 35 specialised hospitals in 13 countries.
Pts. aged ≥18 years with a histologically confirmed TGCT for which surgical resection might worsen functional limitation or cause severe morbidity and ≥1 lesion measuring minimum 2 cm in size according to RECIST v.1.1 were eligible. Previous systemic therapy targeting CSF1 or
CSF1R, including vimseltinib, was not permitted.
Pts. (N=123) were randomly assigned in a 2:1 ratio to receive vimseltinib 30 mg orally twice weekly (N=83) or placebo (N=40). Treatments were administered in 28-cycles for 24 weeks. Randomization was stratified by tumour site (lower limb vs all other) and region (USA vs non-USA).

The primary endpoint was ORR by independent radiological review using RECIST, v.1.1, at week 25 in the ITT population.

Results are shown in table 1 [3].

Table 1. Efficacy results assessed at week 25

Efficacy parameter	Vimseltinib (n=83)	Placebo (n= 40)
ORR per RECIST (95% CI)	33 (40%)	0
Complete response	4 (5%)	0
Partial response	29 (35%)	33 (83%)
Stable disease	42 (51%)	7 (18%)
Not evaluable	8 (10%)	0
Difference (95% CI)	(40%, CI 29 to 51)	
p-value	<0·0001	

Summary of clinical SAFETY:
The safety population included all pts. who received at least one dose of study treatment. The only grade 3-4 TEAE that occurred in more than 5% of pts. treated with vimseltinib was increased blood creatine phosphokinase, which is consistent with the known mechanism of action of CSF1R inhibitors. Five dose interruptions occurred in approximately half of pts. receiving vimseltinib (43 of 83 pts.) but were short in duration

Ongoing studies:
- ***For the same indication:*** Yes
- ***For other indications:*** No

Discontinued studies (for the same indication): No

References:
[1] https://www.ema.europa.eu/en/medicines/human/EPAR/romvimza
[2] https://www.accessdata.fda.gov/drugsatfda_docs/label/2025/219304s000lbl.pdf
[3] https://www.thelancet.com/journals/lancet/article/PIIS0140-6736(24)00885-7/fulltext
[4] https://www.jrheum.org/content/44/10/1476.long
[5] https://www.sciencedirect.com/science/article/pii/S0305737222001608

NHS impact

Cost of therapy:
Not available yet.

Epidemiology:
The epidemiology of TGCT, including localised and diffuse forms, shows an estimated prevalence of 3.4 cases per 10,000 people in the European Union. This figure includes an annual prevalence of 90.2% of cases successfully treated surgically and a recurrence rate of 9.8%, with an average lifetime prevalence of 41 years for the localised form (with an incidence rate of 0.45 per 10,000 people). For the diffuse form, the overall prevalence is 1.15 cases per 10,000 people [4].

POSSIBLE PLACE IN THERAPY:
When surgery is not possible or involves excessive risk for symptomatic TGCT, the main treatment option is represented by CSF1R inhibitors.
Radiotherapy is generally not recommended but may be considered in selected cases where there are no alternatives.
For asymptomatic pts. or those with manageable symptoms, active surveillance is the first option, while symptom management (with analgesics and physiotherapy) is essential to improve quality of life. Conventional chemotherapy is reserved for rare cases of advanced malignant TGCT [5].

OTHER INDICATIONS IN DEVELOPMENT: -

SAME INDICATION IN EARLIER LINE(S) OF TREATMENT: -

OTHER DRUGS IN DEVELOPMENT for the SAME INDICATION: Emactuzumab (NCT05417789); Pexidartinib (NCT04488822).

*Service reorganization: No
*Possible off label use: Yes

Issued in July 2025