NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Health Technology Evaluation

Pimicotinib for treating tenosynovial giant cell tumours when systemic treatment is needed ID6647

Draft scope

Draft remit/evaluation objective

To appraise the clinical and cost effectiveness of pimicotinib within its anticipated marketing authorisation for treating tenosynovial giant cell tumours in adults who require systemic therapy.

Background

Tenosynovial giant cell tumour (TGCT) is a group of rare, benign tumours that involve the synovium (connective tissue in joints), bursae (fluid-filled sac around a joint) and tendon sheath (synovial membrane around a tendon). The tumours cause the synovium, bursae and tendon sheaths to grow and thicken. This can cause damage to the surrounding tissues of the body. The condition is progressive, and symptoms include pain, swelling and restricted movement of the joint. It has a substantial impact on quality of life, including affecting physical function and activities of daily living. Many people with TGCT have difficulty walking. The tumours can affect large or small joints. The World Health Organization categorises the tumours into 2 distinct types:[1]

- Localised TGCT (80-90% of cases), can be within or outside the joint, usually affecting smaller joints such as the hands and feet, and
- Diffuse TGCT (10-20% of cases; previously know as pigmented villonodular synovitis [PVNS]), which usually affects large joints such as the knee or hip.

TGCT mainly affects adults between 20 and 50 years. Registry data from Denmark reported annual incidence of 30.3 cases per million person-years for adults with localised TGCT and 8.4 cases per million person-years for adults with diffuse TGCT.[2] Prevalence per 100,000 people was 44.3 for localised TGCT and 11.5 for diffuse TGCT. Applying this prevalence to the adult population in England and Wales equates to a prevalent population of approximately 21,000 people with localised TGCT and 5,000 people with diffuse TGCT.

The main treatment for TGCT is surgery to remove some or all of the synovium. This is often curative, although the tumour can recur, particularly in diffuse TGCT.[3] Surgery can be repeated, but also comes with the risk of complications. Radiation therapy may be used, either alone or as an adjunct to surgery. Some people have TGCT that is not eligible for surgery. For example, if surgery could lead to dysfunction or complications, or the lesions cannot be completely resected. If surgery is not appropriate, imatinib or nilotinib may be options, although these treatments do not have marketing authorisations for this indication.

The technology

Pimicotinib (branded name unknown, Merck Serono) does not currently have a marketing authorisation in the UK for treating TGCT. Pimicotinib has been studied in clinical trials for people with TGCT who require systemic treatment.

Intervention(s)	Pimicotinib
Population(s)	Adults with tenosynovial giant cell tumour who need systemic treatment
Subgroups	If the evidence allows, the following subgroups will be considered: • Type of tenosynovial giant cell tumour (localised vs. diffuse)
Comparators	Established clinical management without pimicotinib, which may include: • Off-label tyrosine kinase inhibitors (such as imatinib or nilotinib)
Outcomes	The outcome measures to be considered include: • response rate • pain • stiffness • physical function • adverse effects of treatment • health-related quality of life.
Economic analysis	The reference case stipulates that the cost effectiveness of treatments should be expressed in terms of incremental cost per quality-adjusted life year. The reference case stipulates that the time horizon for estimating clinical and cost effectiveness should be sufficiently long to reflect any differences in costs or outcomes between the technologies being compared. Costs will be considered from an NHS and Personal Social Services perspective.
Other considerations	Guidance will only be issued in accordance with the marketing authorisation. Where the wording of the therapeutic indication does not include specific treatment combinations, guidance will be issued only in the context of the evidence that has underpinned the marketing authorisation granted by the regulator.
Related NICE recommendations	None

Questions for consultation

Is the population listed appropriate? In what circumstances would surgery be considered inappropriate and would systemic treatment be needed to treat TGCT?

Have all relevant comparators for pimicotinib been included in the scope? Which treatments are considered to be established clinical practice in the NHS for TGCT when surgery is not appropriate? Does this include imatinib or nilotinib?

Are the outcomes listed appropriate?

Are the subgroups suggested appropriate? Are there any other subgroups of people in whom pimicotinib is expected to be more clinically effective and cost effective or other groups that should be examined separately?

Where do you consider pimicotinib will fit into the existing care pathway for tenosynovial giant cell tumour?

Please select from the following, will pimicotinib be:
A. Prescribed in primary care with routine follow-up in primary care
B. Prescribed in secondary care with routine follow-up in primary care
C. Prescribed in secondary care with routine follow-up in secondary care
D. Other (please give details):
For comparators and subsequent treatments, please detail if the setting for prescribing and routine follow-up differs from the intervention.

Would pimicotinib be a candidate for managed access?

Do you consider that the use of pimicotinib can result in any potential substantial health-related benefits that are unlikely to be included in the QALY calculation?

Please identify the nature of the data which you understand to be available to enable the committee to take account of these benefits.

Please indicate if any of the treatments in the scope are used in NHS practice differently than advised in their Summary of Product Characteristics. For example, if the dose or dosing schedule for a treatment is different in clinical practice. If so, please indicate the reasons for different usage of the treatment(s) in NHS practice. If stakeholders consider this a relevant issue, please provide references for data on the efficacy of any treatments in the pathway used differently than advised in the Summary of Product Characteristics.

NICE is committed to promoting equality of opportunity, eliminating unlawful discrimination and fostering good relations between people with particular protected characteristics and others. Please let us know if you think that the proposed remit and scope may need changing in order to meet these aims. In particular, please tell us if the proposed remit and scope:

- could exclude from full consideration any people protected by the equality legislation who fall within the patient population for which pimicotinib will be licensed;

- could lead to recommendations that have a different impact on people protected by the equality legislation than on the wider population, e.g. by making it more difficult in practice for a specific group to access the technology;

- could have any adverse impact on people with a particular disability or disabilities.

Please tell us what evidence should be obtained to enable the committee to identify and consider such impacts.

NICE intends to evaluate this technology through its Single Technology Appraisal process. (Information on NICE's health technology evaluation processes is available at https://www.nice.org.uk/about/what-we-do/our-programmes/nice-guidance/nice-technology-appraisal-guidance/changes-to-health-technology-evaluation).

References

1. De Saint Aubain Somerhausen N, van de Rijn M (2020) Tenosynovial Giant Cell Tumor. In: Soft Tissue and Bone Tumours, World Health Organization (WHO) Classification of Tumours. Fifth Edition, Volume 3.

2. Ehrenstein V, Andersen SL, Qazi I, et al. (2017) Tenosynovial Giant Cell Tumor: Incidence, Prevalence, Patient Characteristics, and Recurrence. A Registry-based Cohort Study in Denmark. The Journal of Rheumatology 44(10):1476-1483.

3. National Organization for Rare Disorders (NORD) (2023) Tenosynovial Giant Cell Tumor. Available from: https://rarediseases.org/rare-diseases/tenosynovial-giant-cell-tumor/ [Accessed 15 December 2025]