Proportionally quicker or easier? International comparison of NICE's proportionate approach to technology appraisals (PATT) with global peers

HTA53

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Background

In 2022 the National Institute for Health and Care Excellence (NICE) introduced its proportionate approach to technology appraisals (PATT) to increase appraisal capacity and enable faster guidance development.[1, 2]

PATT is considered appropriate where a full Single Technology Appraisal (STA) isn't required, such as for (1) therapies with similar clinical effectiveness to an existing therapy that is routinely funded in England and Wales, and (2) therapies considered 'low risk' e.g. have already undergone the STA process for another indication.[1, 2]

As of October 2023, five treatments have been recommended.[3–7] According to NICE, PATT has delivered final recommendations up to 20 weeks faster than the standard process.[1, 2]

Objective

To explore assessment length and speed of patient access to therapies assessed by NICE during the PATT pilot compared with matched appraisal processes for international peers in France, Germany, Australia, and Canada.

Methods

Drugs that had been assessed under the PATT pilot as of October 2023 were identified via the NICE website.

Comparator countries were selected on the basis of having publicly available information on assessment timings and representing a selection of international peer agencies with similar rigour of decision making to NICE.

Marketing authorisation dates were determined for the regulatory bodies specified in **Table 1**. Appraisal timings and reimbursement populations were extracted, or estimated, from publicly available submission documents from the relevant health technology assessment (HTA) body websites. All timings were checked by an independent reviewer.

Nintedanib had undergone assessment by NICE for idiopathic pulmonary fibrosis (IPF) prior to being reviewed under PATT. This review (TA379) was also assessed, to provide a comparison of PATT vs. a standard STA for the same therapy.

Table 1: Timings used to determine start of the appraisal process, submission date and end of process

HTA body	Marketing authorisation	Start of the appraisal process	Date of dossier submission	End of the appraisal process
NICE	MHRA*	Draft scope	From committee papers	FAD published
HAS	EMA	Date initiale (procédure centralisée)	Not available	Adopté par la Commission de la transparence
G-BA	EMA	Start of proceedings date	From 'module' cover page	Resolution date
PBAC	Australian Register of Therapeutic Goods	25 weeks prior to PBAC meeting†	17 weeks prior to PBAC meeting†	6 weeks following PBAC meeting†
CADTH	Health Canada NOC	Patient/clinician input open	Submission received	Final recommendation posted

* Except for nintedanib, where the 2015 EMA approval date was used as this pre-dated Brexit and transfer of regulatory approvals to the MHRA.
† Except for nintedanib as no PBAC meeting dates were reported.

Abbreviations: CADTH, Canada's Drug and Health Technology Agency; EMA, European Medicines Agency; FAD, final appraisal document; G-BA, Federal Joint Committee; HAS, Haute Autorité de Santé; HTA, health technology assessment; MHRA, Medicines and Healthcare products Regulatory Agency; NICE, National Institute for Health and Care Excellence; NOC, notice of compliance; PBAC, Pharmaceutical Benefits Advisory Committee.

Results

- As of October 2023, five appraisals had positive recommendations through PATT. A further 20 relevant HTAs (18 complete and two in progress, **Table 2**) were identified and assessed.

Comparability of appraisals

- To ensure a like-for-like comparison, assessment target populations were compared across drugs and HTA bodies. While there were some minor differences in final reimbursed population, these were considered not to have impacted on appraisal timings.
- The exception was for nintedanib, which NICE initially reimbursed for a restricted population in 2016 before aligning with the same population as the other HTA bodies in the 2023 PATT appraisal.

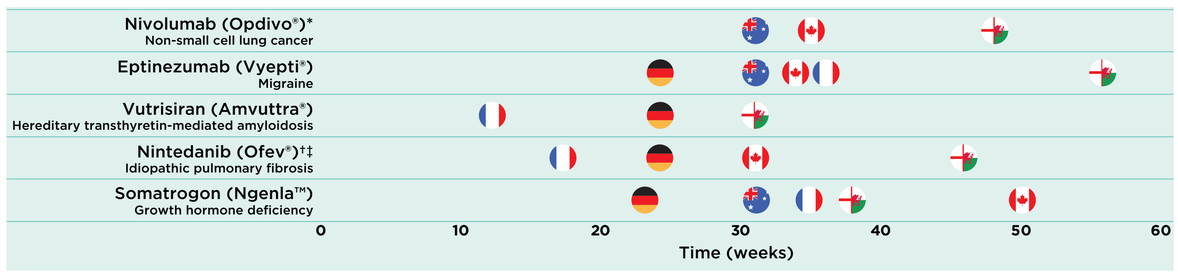
From start of proceedings to resolution

- Using the 'start of the appraisal process' to the 'end of the appraisal process' cut off's for each HTA body, NICE had either the longest or second longest appraisal lengths vs. peers (see **Figure 1**): somatrogon (38 weeks [range 23–50]), nintedanib (46 weeks [range: 17–46]), vutrisiran (31 weeks [range: 12–31]), eptinezumab (56 weeks [range: 24–56]) and nivolumab (48 weeks [range: 31–48]).

From submission date to resolution

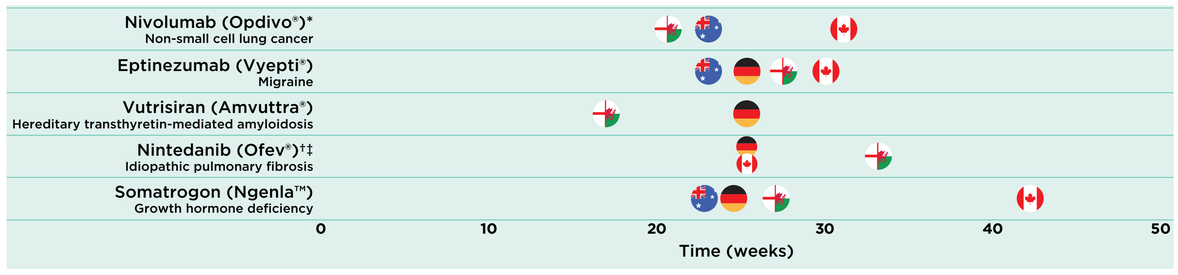
- Using the 'date of dossier submission' to the 'end of the appraisal process' cut offs resulted in less variation between HTA bodies, with NICE generally falling towards the shorter end of the range except for nintedanib (see **Figure 2**): somatrogon (27 weeks [range 23–42]), nintedanib (33 weeks [range: 25–33]), vutrisiran (17 weeks [range: 17–25]), eptinezumab (27 weeks [range: 23–30]) and nivolumab (21 weeks [range: 21–31]). Submission dates were not available for Haute Autorité de Santé (HAS).

Figure 1: Time elapsed from start of proceedings to resolution



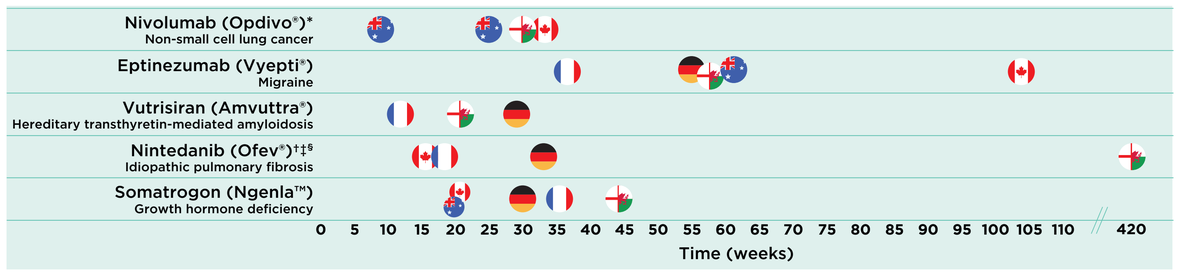
* PBAC assessed nivolumab (Opdivo®) twice (July 2022 and November 2022) after it was initially 'not recommended'.
† There was an assessment by PBAC for nintedanib (Ofev®); but public information on timings was not available and therefore it was excluded from the analysis.
‡ G-BA re-assessed nintedanib (Ofev®) as the costs exceeded the €50 million threshold. As patients had access after the first assessment we did not include the second in further analysis.
Abbreviations: G-BA, Federal Joint Committee; PBAC, Pharmaceutical Benefits Advisory Committee.

Figure 2: Time elapsed from submission date to resolution of proceedings



There were no submission dates for HAS.
* PBAC assessed nivolumab (Opdivo®) twice (July 2022 and November 2022) after it was initially 'not recommended'.
† There was an assessment by PBAC for nintedanib (Ofev®); but public information on timings was not available and therefore it was excluded from the analysis.
‡ G-BA re-assessed nintedanib (Ofev®) as the costs exceeded the €50 million threshold. As patients had access after the first assessment we did not include the second in further analysis.
Abbreviations: G-BA, Federal Joint Committee; HAS, Haute Autorité de Santé; PBAC, Pharmaceutical Benefits Advisory Committee.

Figure 3: Time elapsed from marketing authorisation to resolution of proceedings



* PBAC assessed nivolumab (Opdivo®) twice (July 2022 and November 2022) after it was initially 'not recommended'.
† There was an assessment by PBAC for nintedanib (Ofev®); but public information on timings was not available and therefore it was excluded from the analysis.
‡ G-BA re-assessed nintedanib (Ofev®) as the costs exceeded the €50 million threshold. As patients had access after the first assessment we did not include the second in further analysis.
§ NICE restricted its original assessment to only those with forced vital capacity (FVC)<80% whereas other countries applied no such restrictions.
Abbreviations: FVC, forced vital capacity; G-BA, Federal Joint Committee; NICE, National Institute for Health and Care Excellence; PBAC, Pharmaceutical Benefits Advisory Committee.

Table 2: HTAs identified across the five HTA agencies for the five drugs recommended via the NICE proportionate approach

		Somatrogon (Ngenla™)	Nintedanib (Ofev®)	Vutrisiran (Amvuttra®)	Eptinezumab (Vyepti®)	Nivolumab (Opdivo®)
NICE	TA	863	864*	868	871	876
	Population	Treating growth disturbance caused by growth hormone deficiency in children and young people aged ≥3 years	Treating IPF in adults if they have a FVC of >80% predicted	Treating hereditary transthyretin-related amyloidosis in adults with stage 1 or stage 2 polyneuropathy	Preventing migraine in adults, only if they have ≥4 migraine days a month and ≥3 preventive drug treatments have failed	With chemotherapy for neoadjuvant treatment of resectable (tumours ≥4 cm or node positive) NSCLC in adults
HAS		Yes	Yes	Yes	Yes	No
G-BA		Yes	Yes – 2015 and reassessment 2019†	Yes	Yes	In progress
PBAC		Yes	Yes‡	No	Yes	Yes – two assessments after first received a 'not recommended'
CADTH		Yes	Yes	In progress	Yes	Yes

* NICE has appraised nintedanib for IPF twice; once in 2016 [TA379] for treating IPF if the person has a FVC between 50% and 80% of predicted and once in 2023 [TA864] for treating IPF when FVC is >80% predicted. Only the latter appraisal was conducted under the PATT process.
† G-BA re-assessed nintedanib (Ofev®) as the costs exceeded the 50-million-euro threshold. As patients had access after the first assessment we have not included the second assessment in further analysis.
‡ PBAC considered nintedanib for the treatment of patients with IPF in March 2015, November 2015, and November 2016; these three committee meetings have been categorised as a single assessment. Public information on the timings was not available and therefore it was excluded from further analysis.
Abbreviations: CADTH, Canada's Drug and Health Technology Agency; FVC, forced vital capacity; G-BA, Federal Joint Committee; HAS, Haute Autorité de Santé; HTA, health technology assessment; IPF, idiopathic pulmonary fibrosis; NICE, National Institute for Health and Care Excellence; NSCLC, non-small-cell lung cancer; PATT, proportionate approach to technology appraisals; PBAC, Pharmaceutical Benefits Advisory Committee; TA, technology appraisal.

From marketing authorisation to resolution

- Following marketing authorisation, NICE was never fastest in reaching an approval decision across the five treatments (see **Figure 3**). HAS was fastest for two (vutrisiran and eptinezumab), Pharmaceutical Benefits Advisory Committee (PBAC) for two (somatrogon and nivolumab) and Canada's Drug and Health Technology Agency (CADTH) for one (nintedanib).
- NICE was slowest in reaching an approval decision following marketing authorisation for somatrogon (44 weeks [range 20–44]) and nintedanib (420 weeks [range 16–420]).
- NICE has reached a decision on all five of the medicines analysed. HAS is yet to reach a decision for nivolumab, Federal Joint Committee (G-BA) for nivolumab, PBAC for vutrisiran, and CADTH for vutrisiran.

Nintedanib: a case study

- Nintedanib provides an opportunity to compare NICE assessments for a similar indication and drug under both PATT and the STA process. The PATT appraisal was conducted 12 weeks faster than the STA appraisal.
- Of note, NICE was the only body to restrict its first reimbursement recommendation to a subset of patients with IPF. The second PATT appraisal brought the recommended population in line with the recommended populations in the peer agencies.

Limitations

- Differences among HTA body assessment timings and availability of publicly available information makes precise determination of appraisal lengths challenging.
- Where separate pricing negotiation is required after clinical benefit assessment, for example in Germany and France, this was not included in assessment timings due to lack of published data. As such the 'end of the appraisal process' dates may not necessarily reflect when therapies were made available to patients.

Conclusions

- While only five drugs have been assessed under PATT, early analysis shows a positive impact on appraisal length compared with the standard STA route. Overall review appraisal timings, from scoping to final guidance, have shortened, but calendar approval dates are mixed compared with international peers.
- As the PATT process continues to evolve, further data on speed of access to new therapies for patients, and on the burden of the submission process for NICE and for manufacturers, should be explored to evaluate whether proportionate appraisals are meeting the needs of relevant stakeholders effectively.

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Disclosures
No disclosures

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