

The Health Technology Appraisal Process: Advocating For Patients

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Introduction

As a health journey partner, Sanofi UK appreciates the important work that patient organisations do to benefit the lives of patients. As outlined in our Patient Charter, we recognise the importance of working collaboratively with patient organisations and strive to adopt an inclusive and supportive approach to everything we do.

This booklet is designed to help those advocating on behalf of patients to understand how the Health Technology Appraisal (HTA) process works. It outlines how patient organisations can get involved in that process to ensure that the patient voice is heard by those making decisions.



The HTA Process in England

THE NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE (NICE): AN OVERVIEW

- NICE was founded in 1999 to **reduce variation in the availability and quality of NHS treatments and care**.¹ Formerly known as the National Institute for Clinical Excellence, its name and remit changed in 2012 when it took on responsibility for **social care guidance**.²
- NICE guidance technically only covers England, but NICE has agreements to provide certain NICE products and services to Wales, Scotland and Northern Ireland. Decisions on how NICE guidance applies in these countries are made by the devolved administrations. They are often involved and consulted with in the development of NICE guidance.²
- NICE is **accountable to the Secretary of State at the Department of Health and Social Care** but remains independent from Government.²

NICE National Institute for
Health and Care Excellence

The Role of NICE

NICE's main role is to produce evidence-based guidance and advice for health, public health and social care practitioners.³ It includes:

1. Technology Appraisals Guidance:

recommendations on specific treatments, procedures, devices and diagnostics

2. NICE guidelines:

recommendations on preventing and managing specific conditions, improving health and managing medicines

3. Care pathways:

NICE also produces care pathways and briefings on other issues of relevance to the health service

NICE also creates quality standards and performance metrics for those delivering and commissioning services.

Health Technology Appraisals

Health Technology Appraisals (HTAs) are recommendations on the use of new and existing treatments within the NHS, such as:

- Medicines
- Medical devices
- Diagnostic techniques
- Screening tools
- Surgical procedures
- Health promotion activities

NICE base its recommendations on a review of clinical and economic evidence:

Clinical evidence:

how well the treatment works

Economic evidence:

how well the treatment works in relation to how much it costs the NHS

There are three main types of HTA:^{4,7}

1. Single Technology Appraisal (STA)

Where **one treatment**, described by NICE as a 'technology' is **assessed in one of its licensed indications**.⁴

This could be assessing a treatment for one broad disease area, (e.g. advanced breast cancer), or assessing a treatment for the management of a more specific type of disease (e.g. neoadjuvant treatment of HER2-positive breast cancer).

An STA process from start to finish usually takes 35 - 43 weeks.⁵

2. Multiple Technology Appraisal (MTA)

This is a less common appraisal.

It could cover **multiple technologies in the same indication, or one technology covering multiple disease areas**.⁴

For instance, in 2016 an MTA recommended seven new drugs for rheumatoid arthritis at the same time.

An MTA process takes longer, at around 54 weeks.⁶

3. Highly Specialised Technologies (HST)

This covers recommendations on the use of new and existing highly specialised medicines and treatments within the NHS in England.⁷

HSTs only evaluate **technologies for very rare conditions**.⁷

A single HST evaluation can only cover **one technology for one indication**.⁷

This booklet discusses recent affordability rules that have been introduced to the technology appraisal process and provide an overview of the most common type of HTA – the STA. Within this overview, we set out the key stages of the STA process and provide details on how you can get involved.

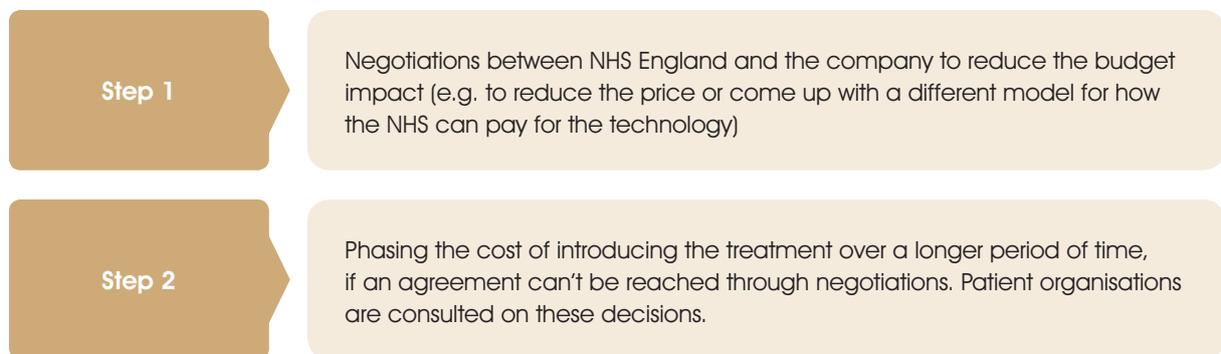
If you would like to find out more about the technical language used by NICE in the appraisal process, please refer to the 'Key Terms' summary at the end of this booklet.

What new affordability rules have been introduced to the HTA process?

There are a number of financial challenges facing the NHS, which has led to the introduction of new “affordability” rules.

1. BUDGET IMPACT TEST⁸

- NICE and NHS England introduced the budget impact test as an attempt to help the NHS become more sustainable
- It assesses the financial impact a technology will have in the first 3 years of its launch
- If a technology costs the NHS £20 million or more, in that time, one of the following steps need to be taken before the technology can be made available for use on the NHS:⁸



2. CHANGES TO THE HIGHLY SPECIALISED TECHNOLOGIES (HST) GUIDANCE

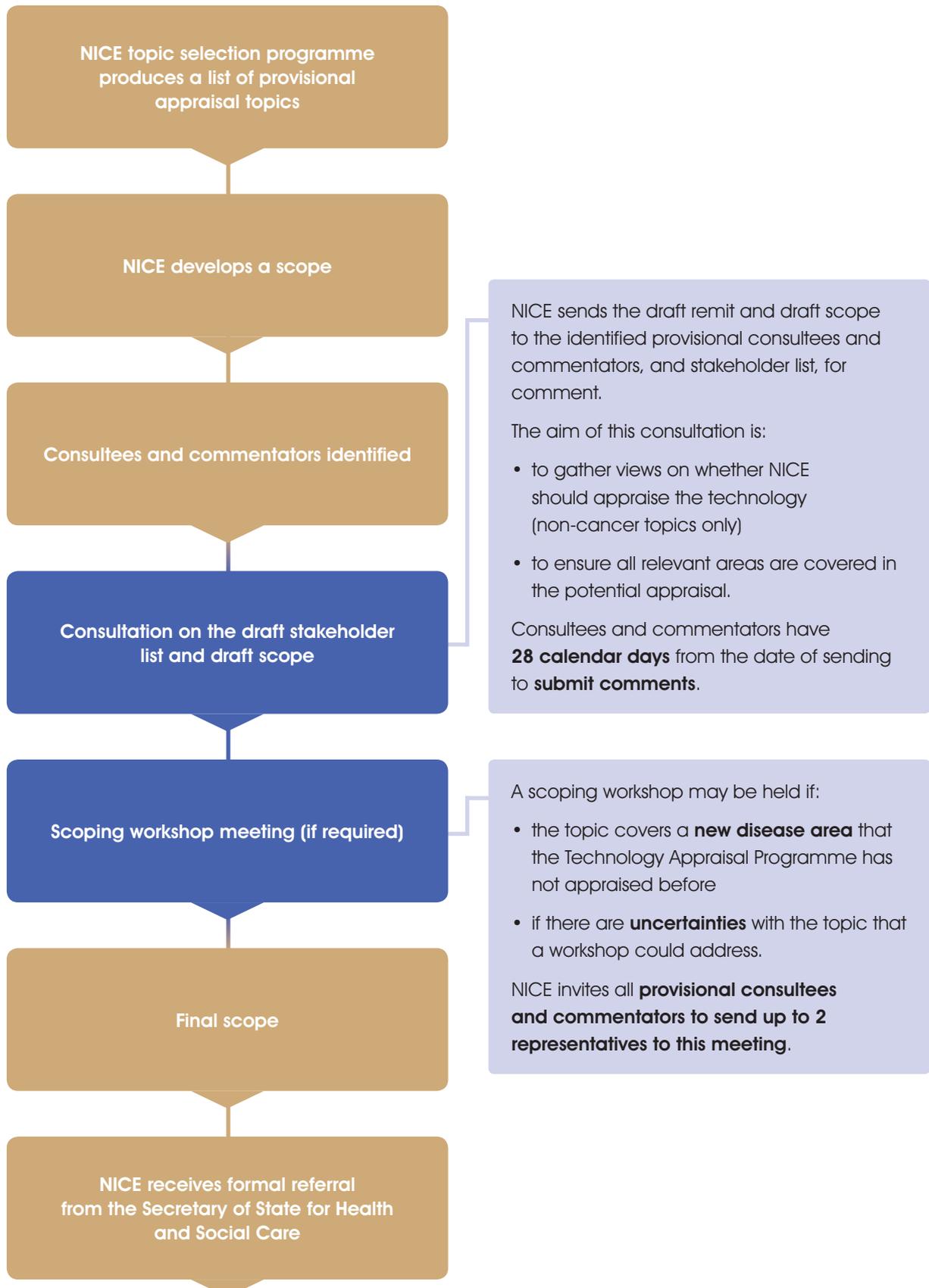
Funding for HST evaluations also changed in 2017. One of the key changes was the **introduction of a quality-adjusted life year (QALY) modifier**. As a result of the reforms, a QALY weighting will now become the key deciding factor within evaluations of rare disease treatments, giving a progressive advantage to those treatments that offer greater QALY gains. Rare disease treatments will now be assessed against a maximum threshold of **£300,000 per QALY** (a threshold ten times higher than the normal limit).⁹

Demonstrating significant QALY benefits for patients with very rare diseases can be challenging, however, particularly with usually small groups of patients participating in clinical trials. A number of patient organisations (as part of an Alliance) have raised concerns about changes to the HST programme, arguing that the QALY threshold is an arbitrary selection that could exclude a significant number of rare disease patients from accessing innovative therapies.¹⁰ These patient organisations have also voiced their concerns that the QALY modifier will be over-reliant on health economic calculations that do not adequately reflect the value of treatments to families affected by rare diseases.¹⁰

The illustration on the next page sets out the key stages of the NICE STA process in addition to details of how you can get involved at each stage.

STAGES OF THE NICE STA PROCESS - SCOPING¹¹

HOW YOU CAN GET INVOLVED



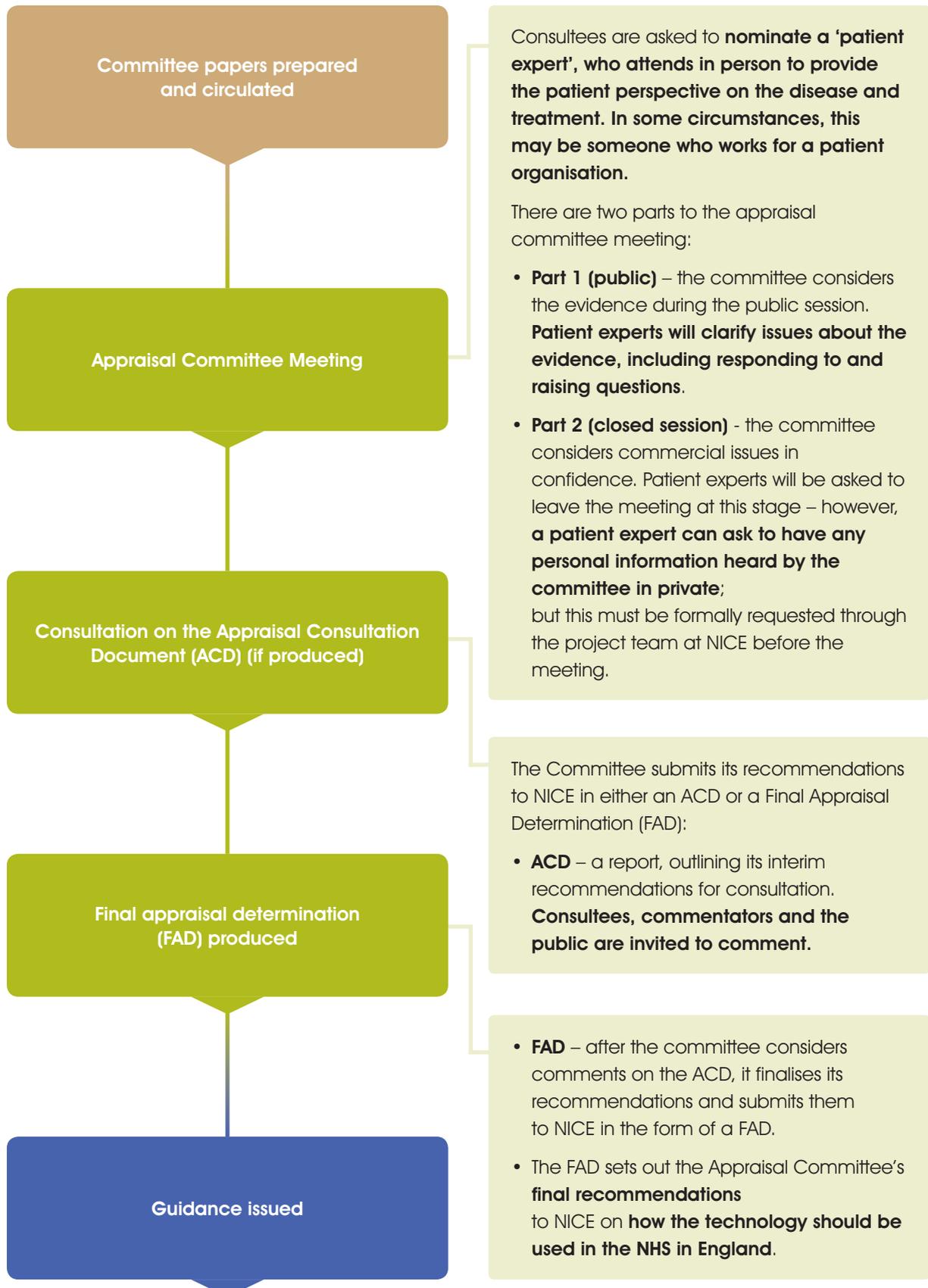
STAGES OF THE NICE STA PROCESS - APPRAISAL¹¹

HOW YOU CAN GET INVOLVED



STAGES OF THE NICE STA PROCESS - APPRAISAL¹¹

HOW YOU CAN GET INVOLVED



How can you engage in the NICE HTA process?

Patient organisations have a unique role in the HTA process. They can get involved, acting as a voice for patients, carers and families, from scoping stages through to final guidance:

- You can **facilitate patient and carer input** to ensure their views are heard (e.g. through written evidence submissions to NICE)
- **Generating meaningful data** (e.g. via surveys) is one way you can represent patients. Surveys can improve understanding of the impact of a disease on patients' lives. Outputs could help explain the difference that access to a given treatment may make for them.
- As organisations providing support to patients and with a strong understanding of their needs, you can also play a crucial role in letting them know about decisions that are being made on treatments. This might involve suggesting suitable patients to provide evidence at NICE appraisal committee meetings and supporting them before they give evidence.



Registering to take part in the NICE process is straightforward. You just need to go onto the NICE website and register your details.¹² NICE will then provide you with details of relevant consultations. The Public Involvement Programme (PIP) team at NICE can also help. More information on the PIP team can be found at: <https://www.nice.org.uk/about/nice-communities/nice-and-the-public/public-involvement>.

KEY TERMS – THE NICE PROCESS¹¹

Consultee: Can comment on the draft scope and the Appraisal Consultation Document (ACD), nominate experts to the Appraisal Committee and can appeal against the Final Appraisal Determination (FAD).

Commentator: Engages in the process but doesn't submit evidence. Cannot nominate experts or appeal the FAD, but can comment on the draft scope and ACD.

Company: Also called the manufacturer – the company that produces the drug being assessed or the drug it is being compared against.

Cost-Effectiveness: A measure of the clinical effectiveness of a drug against its cost.

Appraisal Consultation Document (ACD):

A report from the Appraisal Committee, outlining the interim recommendations for consultation.

Evidence Review Group (ERG): A group commissioned to produce an independent assessment of the evidence submitted by the company with a technology being appraised within the STA process.

Technical team: Consists of the chair or vice chair of the committee along with the NICE team. The technical team will be responsible for considering the company evidence submission, ERG critique and submissions from other consultees and commentators.

Final Appraisal Determination: The final guidance issued by NICE during the appraisal.

THE HTA PROCESS ACROSS THE DEVOLVED NATIONS



About Sanofi in the UK

Sanofi is a global life sciences company committed to discover, develop and distribute therapeutic solutions focused on patients' needs. Sanofi in the UK partners with a number of different patient organisations to pursue goals which benefit patients. Our Patient Charter outlines our pledges for working with patients and patient organisations. To view the Patient Charter and learn more about our work with patient organisations, please visit www.sanofi.co.uk.

Life is a health journey, with ups and downs, which can be big or small, lifelong or momentary. We, at Sanofi, are there for those challenges, **as a health journey partner**. Many patients are depending on us. We aim to protect, enable and support people facing health challenges, so they can live life to its full potential.

References

- ¹ National Institute for Health and Care Excellence (NICE), 'History of NICE', available at: <https://www.nice.org.uk/about/who-we-are/history-of-nice> (accessed: March 2019)
- ² National Institute for Health and Care Excellence (NICE), 'Who we are', available at: <https://www.nice.org.uk/about/who-we-are> (accessed: March 2019)
- ³ National Institute for Health and Care Excellence (NICE), 'What we do', available at: <https://www.nice.org.uk/about/what-we-do> (accessed: March 2019)
- ⁴ National Institute for Health and Care Excellence (NICE), 'Technology appraisal guidance', available at: <https://www.nice.org.uk/about/what-we-do/our-programmes/nice-guidance/nice-technology-appraisal-guidance> (accessed: March 2018)
- ⁵ National Institute for Health and Care Excellence (NICE), 'STA process timeline', available at: <https://www.nice.org.uk/about/what-we-do/our-programmes/nice-guidance/nice-technology-appraisal-guidance/process/sta-timeline-pre-april-2018> (accessed: March 2019)
- ⁶ National Institute for Health and Care Excellence (NICE), 'MTA timeline', available at: <https://www.nice.org.uk/about/what-we-do/our-programmes/nice-guidance/nice-technology-appraisal-guidance/process/mta-timeline> (accessed: March 2019)
- ⁷ National Institute for Health and Care Excellence (NICE), 'Highly specialised technologies guidance', available at: <https://www.nice.org.uk/About/What-we-do/Our-Programmes/NICE-guidance/NICE-highly-specialised-technologies-guidance> (accessed: March 2019)
- ⁸ National Institute for Health and Care Excellence (NICE) (4 April 2017), 'Changes to NICE drug appraisals: what you need to know', available at: <https://www.nice.org.uk/news/feature/changes-to-nice-drug-appraisals-what-you-need-to-know> (accessed: March 2019)
- ⁹ National Institute for Health and Care Excellence (NICE) (15 March 2017), 'NICE gets go-ahead to fast-track more drug approvals', available at: <https://www.nice.org.uk/news/article/nice-gets-go-ahead-to-fast-track-more-drug-approvals> (accessed: March 2019)
- ¹⁰ Meade, N., Genetic Alliance UK (15th March 2017) 'Changes to the HST Programme'. Available at: <http://www.geneticalliance.org.uk/news-events/news/changes-to-the-hst-programme/> (accessed: March 2019)
- ¹¹ National Institute for Health and Care Excellence (NICE) (April 2018), 'Guide to the processes of technology appraisal', available at: <https://www.nice.org.uk/Media/Default/About/what-we-do/NICE-guidance/NICE-technology-appraisals/technology-appraisal-processes-guide-apr-2018.pdf> (accessed: March 2019)
- ¹² National Institute for Health and Care Excellence (NICE), 'Register as a stakeholder', available at: <https://www.nice.org.uk/get-involved/stakeholder-registration> (accessed: March 2019)
- ¹³ Scottish Medicines Consortium (SMC), 'A Guide to the Scottish Medicines Consortium', available at: https://www.scottishmedicines.org.uk/About_SMC/What_we_do/SMC_Guide__web__final_.pdf, (accessed: March 2019)
- ¹⁴ Scottish Medicines Consortium (SMC), 'PACE Factsheet', available here: https://www.scottishmedicines.org.uk/files/PACE/PACE_factsheet_0417.pdf (accessed: March 2019)
- ¹⁵ Varnava, A, et al (March 2018), 'New Medicines in Wales: The All Wales Medicines Strategy Group (AWMSG) Appraisal Process and Outcomes', available here: <https://link.springer.com/article/10.1007/s40273-018-0632-7> (accessed: March 2019)



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