Access to medicines:

Patient input into HTA processes

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Health Technology* Assessment

* Health Technology = medicine, medical device or diagnostic

HTA

Different from country to country

HTA takes international evidence about a new technology compared with the best standard of care used in the national healthcare system

Purpose of HTA is to inform health care policy-makers about the following decisions:

- If a health technology (e.g. a medicine) should be used
- How best to use it
- Which patients will benefit most from it

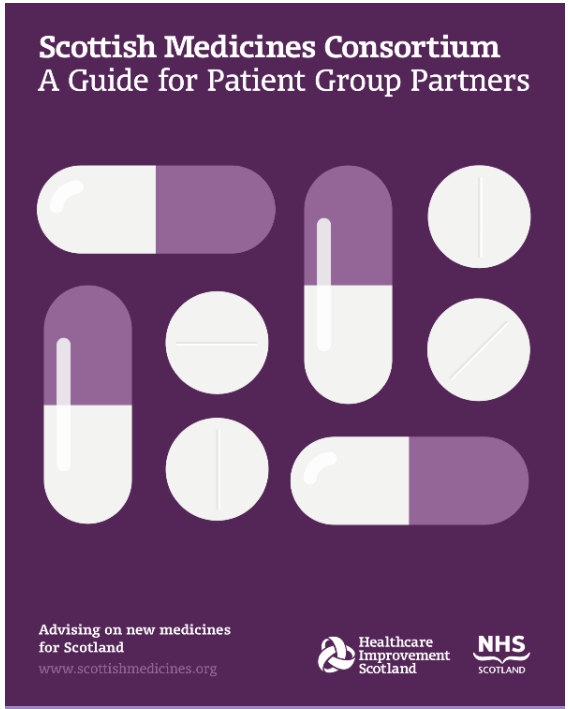
A common approach looks likes this:

Initial assessment → **Consultation** → **Final recommendation**

Scoping phase	Evidence Submission	Committee Meeting 1	Consultation	Committee meeting 2	Final Appraisal Decision / Recommendation
To determine the areas of focus for an appraisal	To collect evidence from affected stakeholders	To make a draft recommendation	To allow input on the draft recommendation	To discuss the comments	The final recommendation
					
Patient input	**Patient input**	**Patient input**	**Patient input**	**Patient input**	**Patient input**
Consultation and workshops	Patient organisations and patient expert written submissions	Patient experts attend and answer questions Public members involved in decision making discussions	Patient organisation and patient experts comment	Patient experts exceptionally invited back Public members in the decision making discussions	Comment on factual accuracies Can appeal

What kind of questions do HTA bodies ask of patient groups?

1. How does this condition affect the day-to-day lives of people living with it?
2. How well do medicines which are currently available help patients manage this condition?
3. Have you (the patient organization) been able to consult with patients who have used this medicine?
4. Would this medicine be expected to improve the patient's quality of life and experience of care, and if so, how?
5. What kind of impact would treating a patient with this medicine have on the patient's family or carers?
6. Are there any disadvantages of the new medicine compared to current standard treatments?



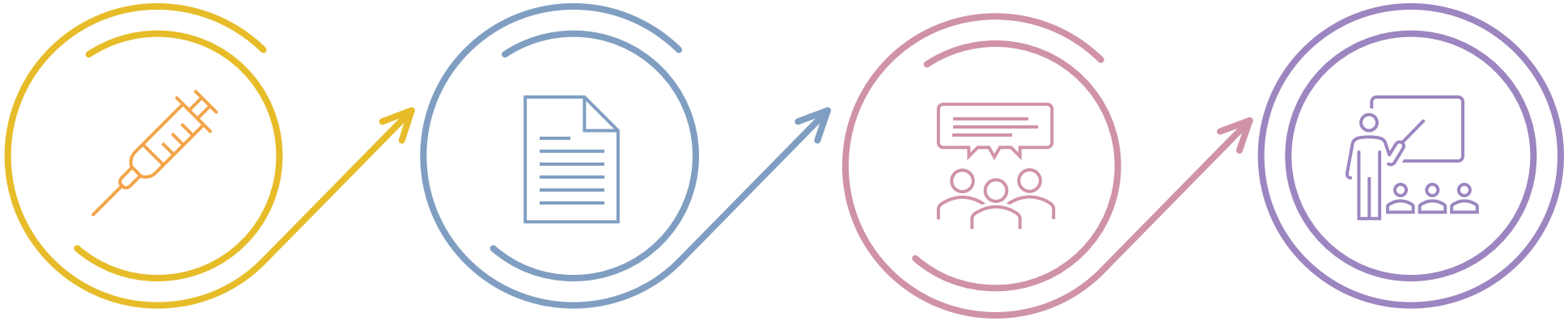
Example from Scotland but most HTA bodies ask these same questions

Source: https://www.scottishmedicines.org.uk/media/2724/patient-group-partner-guide.pdf

HTA Review

A look at recent HTA decisions on a biologic treatment for atopic eczema

Methodology



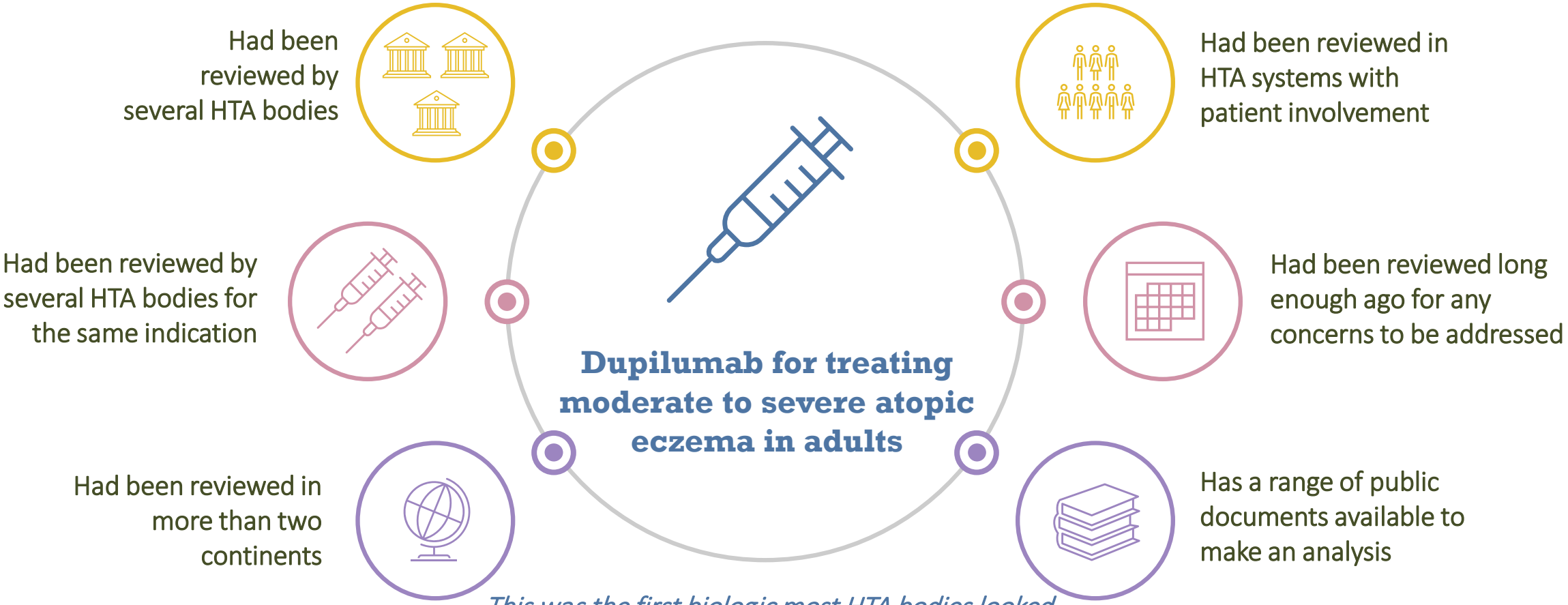
Focus on one biologic and indication for a consistent analysis

Identify HTA reports in the public domain to analyse

Review concerns from HTA bodies and patient input to address concerns

Identify key learnings for the patient advocate community in future HTA processes

To review HTA reports, a biologic was selected that had the following attributes



This was the first biologic most HTA bodies looked at and has the most public documentation

HTA reports from initial submissions in 2017/18 (1)

		Why was this chosen?
NICE (England)	Aug 2018: Recommended with restrictions for adults with moderate to severe atopic eczema[1]	• Initial rejection which was later overturned • Lots of input from the patient advocate community • The innovation of biologics recognized
CADTH (Canada)	July 2018: Was not recommended for reimbursement[2] (note in 2020 this was overturned[3])	• Not recommended for reimbursement • This was despite patient input • A later review reversed this decision
PBAC (Australia)	July 2018: Was rejected (and again in 2019), but was finally approved for reimbursement in 2020[4]	• Original submission and resubmission were both rejected for reimbursement • A refocused submission with restrictions approved

1: https://www.nice.org.uk/guidance/ta534/documents/committee-papers-2
2: https://www.cadth.ca/sites/default/files/cdr/complete/SR0533_cdr_complete_Dupixent_July_9_2018.pdf
3: https://www.cadth.ca/sites/default/files/cdr/complete/SR0636%20Dupixent%20-%20CDEC%20Final%20%20Recommendation%20April%2024%2C%202020%20for%20posting.pdf
4: https://www.pbs.gov.au/industry/listing/elements/pbac-meetings/psd/2020-03/files/dupilimumab-psd-march-2020.docx

Summary of key lessons (1)

Patient population for reimbursement is a critical driver

A key driver of the decision was the definition of the patient population and therefore the potential numbers to be treated. This led to the therapy being available but for a restricted group of patients

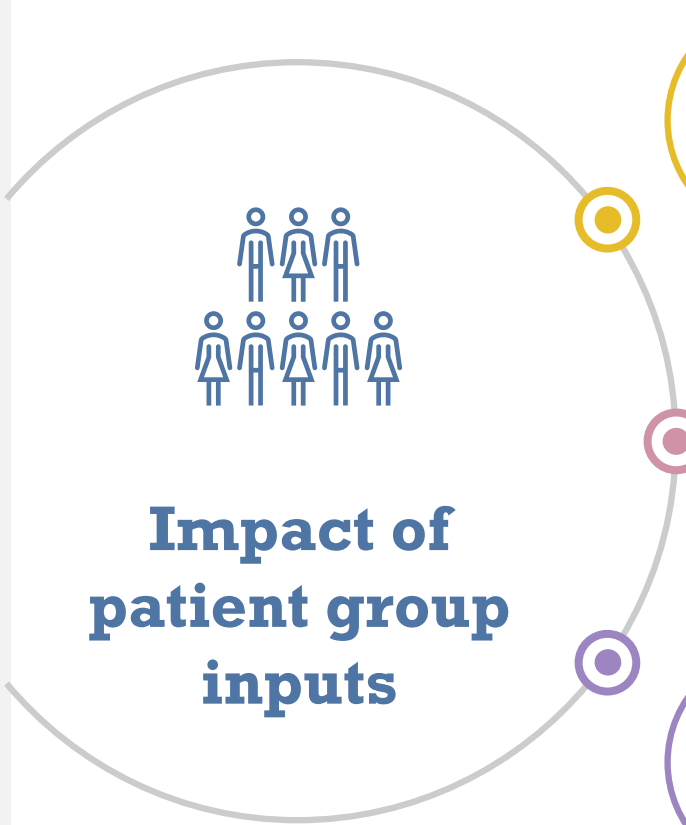
Patient impacts were clearly defined by patient group input

The patient group submissions were recognized as providing important context on the impacts of atopic eczema. These severe impacts were called out in the final reports (even when the decision was negative)

Evidence challenges were cited as the reason for some rejections

The choice of comparator used in the clinical trials or the assumptions in economic models were challenged. This often led to rejections followed by a positive recommendation when new data & models were submitted

Summary of key lessons (2)



Adding context
particularly around unmet needs of patients

Patient group inputs were very focused and clear on the impact of the condition and on the failures of current treatment options to manage atopic eczema for many people. These insights were not questioned or challenged by the committees.

Other factors
drove the negative decisions, outside of patient group control

All cases had an initial negative decision before new data was provided or models were adjusted. In some cases, new patient access schemes or risk share agreements were negotiated. These actions fall outside the scope of patient group input.

Setting a precedent
for atopic eczema and dermatology treatment access

By clearly articulating the severe burden and unmet need facing people with atopic eczema, and the need for access to innovative medicines, the eventual positive recommendations set a precedent for access to reimbursed biologics in dermatology conditions.

Panel Discussion