Differences between regulatory approval, HTA, and patients regarding health technologies.

	Regulatory approval	HTA	Patient
Decision(s) to be made by the stakeholder	• Does the technology do more good than harm for patients with the defined target indication? • Should this technology be marketed?	• Does the technology offer useful, appropriate benefits for all or a select sub-group of patients in this healthcare system compared to what is most commonly used in the disease area? • Are the costs associated with the technology affordable and justified by its benefits?	• Is it effective? • What benefit and/or harm should I expect from taking it? • How does it compare to other treatments available? • How much will it cost me? • How convenient is the treatment?
Type of evidence required	• Safety. • Efficacy. • Quality.	• Safety. • Effectiveness. • Economics and budgetary impact. • Social, ethical, legal, organisational impact.	• Safety. • Effectiveness.
Evidence considered	• (Pre-launch) Randomised controlled trials, with a standard-of-care or placebo comparator • (Post-launch) Safety/pharmacovigilance (always), relative efficacy or effectiveness, when assessing a product's benefit-risk profile in extended/long-term use.	• Randomised controlled trials, observational studies. • Systematic reviews of pertinent literature. • Relative effectiveness and costs, as assembled from trials or through analytic techniques such as meta-analysis, modelling.	• Personal and others' experience. • Results from trials explained in lay language.
Validity	• Internal validity (can a causal conclusion be drawn without systematic bias?).	• External validity (can the results of a study be generalised to other situations and to other people?).	• Internal and external validity.
Outcomes	• Hard clinical endpoint outcomes. • Laboratory findings. • Surrogate outcomes. • Patient-relevant outcomes (increasingly).	• Quality of life. • Long-term clinical outcomes. • Patient-relevant outcomes.	• Outcomes relevant to me.

	Regulatory approval	HTA	Patient
Comparator	• Standard-of-care medicinal product (active control), or • Placebo.	• Active control, ideally reflecting what might be replaced by the new technology.	• The best option available, or • What I am currently taking if switching to new medicine. • No treatment.
Time horizon	• Trial duration. • Post marketing studies. • Pharmacovigilance over the lifetime of a product.	• Life time; or at least the time needed to capture risks and benefits of treatment.	• Time horizon relevant to me.

Adapted from:

1. Tsoi B, Masucci L, Campbell K, Drummond M, O'Reilly D, Goeree R. Harmonization of reimbursement and regulatory approval processes: a systematic review of international experiences. Expert Rev Pharmacoecon Outcomes Res. 2013 Aug;13(4):497–511.
2. Henshall C, Mardhani-Bayne L, Frønsdal KB, Klemp M. Interactions between health technology assessment, coverage, and regulatory processes: emerging issues, goals, and opportunities. Int J Technol Assess Health Care. 2011 Jul;27(3): 253–60.