

Country vignettes

HTA appraisal/reimbursement processes for rare disease treatments (January 2020)

There is increasing recognition that conventional health technology assessment (HTA) appraisal and reimbursement processes may be unsuitable for rare disease treatments, particularly in those diseases that require highly specialized care and that are very rare. They often fail to demonstrate added benefit or meet cost-effectiveness thresholds because of uncertainties in the evidence and high prices. Work Package 10 on appraisal of rare disease treatments in the EU-funded IMPACT-HTA project is developing guidance on novel approaches for the appraisal of rare disease treatments that will help overcome these limitations and ensure improved access to these treatments through more consistent, transparent and robust decision-making.

The first step was to gain a good understanding of how countries are dealing with rare disease treatments in their appraisal and reimbursement processes. It was thought that some HTA bodies were implementing special processes for rare disease treatments or ultra-rare disease treatments, but these were not well documented or consistent across countries. A comprehensive overview of European countries was missing. The aim of creating these country vignettes was to document HTA appraisal/reimbursement processes for rare disease treatments in all EU and EEA Member States, Canada and New Zealand, in a concise, consistent and clear manner.

This was done in several stages: (1) identification of key experts (when possible, those involved in these processes for rare disease treatments), (2) administration of survey to key experts, (3) creation of country vignette on the basis of the information provided in the surveys and additional desk research, and (4) additional questions to, and final validation of vignette with key experts.

This work has been ongoing since mid-2018 and we are now delighted to make these vignettes publicly available through the IMPACT-HTA website. The strength of these vignettes is that a lot of the information comes from key experts, and what is more, that some of the information provided is not available in public resources. Nonetheless, some of the information may be incomplete or contain inaccuracies. If you identify any issues, do not hesitate to contact the IMPACT-HTA team.

IMPACT HTA

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