Australia – an industry perspective

Developing HTA guidelines for rare disease therapies in Australia – an industry perspective

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There are unique challenges with assessing rare disease therapies for funding as part of a broader HTA process. Experience from Australia shows industry can partner with government and provide innovative solutions for how these challenges might be recognised as part of the evaluation framework.

Results

• Current challenges identified by the IWG include: obtaining appropriate advice and guidance upfront; communicating the burden of disease; reliance on lower levels of evidence; challenges in demonstrating cost-effectiveness; transparency of decision making (Figure 2).
• The IWG supported a recommendation to establish a fit-for-purpose process for evaluation of rare disease therapies – supported by amended guidelines (Figure 3).
• Through a review of guidelines for rare disease therapies from England, Scotland and Canada – several themes emerged: early engagement via an established pre-submission framework; improved transparency in decision making and funding decisions (Figure 4).
• Results will inform specific recommendations to government.

Conclusion

There are unique challenges with assessing rare disease therapies for funding as part of a broader HTA process. Experience from Australia shows industry can partner with government and provide innovative solutions for how these challenges might be recognised as part of the evaluation framework.