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COMPARISON OF PHARMACOECONOMIC (PE) EVALUATIONS FOR DRUGS FOR RARE DISEASES (DRDS) EVALUATED BY CADTH AND NICE.

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OBJECTIVES: The processes by which DRDs are evaluated varies across health technology assessment (HTA) agencies. The objective of this analysis was to determine trends in how the Canadian Agency for Drugs and Technologies (CADTH) and the National Institute for Health and Care Excellence (NICE) evaluated DRDs, including factors influencing the recommendations issued, to support decision-making and inform best PE practices for DRDs.

METHODS: CADTH DRD recommendations issued between March 2016 and June 2018 were reviewed, and equivalent NICE recommendations were identified. DRDs were excluded where a recommendation was not issued by both agencies. The recommendation(s) made, incremental cost effectiveness ratios (ICERs), and PE methods used within the submissions were then extracted and compared to identify key trends in how the evidence was appraised.

RESULTS: Nine DRD recommendations were issued by CADTH and NICE. The mean time from submission to recommendation was approximately 6.3 months for CADTH, and 7.8 months for NICE. NICE applied three different processes for the evaluation of the identified DRDs: highly specialized technology appraisal (n=4), single technology appraisal (n=4), and multiple technology appraisal (n=1). No differential processes were applied for DRDs by CADTH. Despite this, final recommendations were similar (positive: n=7 (NICE), n=8 (CADTH); ongoing/negative: n=2 (NICE), n=1 (CADTH)). Similar PE methods were used in the manufacturer-submitted PE evaluations. In all cases the HTA reanalysis ICERs were higher than the manufacturer-submitted ICERs, with the magnitude of difference being greater in the CADTH submissions.

CONCLUSIONS: While different processes are used by NICE and CADTH, and the reasons for the recommendations vary, the methods used, and final recommendations made were broadly consistent. Differences in the HTA re-analysis ICERs when compared with the manufacturers submission are indicative of the challenges associated with conducting economic evaluations for DRDs.