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Title: Real World Evidence a Tool to Overcome the Lack of a Comparative Arm in Drug Evaluation in the Context of Rare Diseases

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Abstract : Objective: To build a comparative arm for product (X) in specific gene mutated advanced gastrointestinal cancer using real world evidence to fulfill HTA requirements in drug evaluation. Methods: Data for product (X) were collected from phase II clinical trial while real world data for (Y) and (Z) were collected from US database. Real-world (RW) cohorts were matched to clinical trial base line characteristics using weighting by odds method. Outcomes included progression-free survival (PFS) and overall survival (OS) rates. Study location and participants: Internationally (product X, n=80) and from USA (Product Y and Z, n=73) Results: Two comparisons were made: trial cohort 1 (X) versus real-world cohort 1 (Z), trial cohort 2 (X) versus real-world cohort 2 (Y). For first line, the median OS was 9.7 months (95% CI 8.6- 11.5) and the median PFS was 5.2 months (95% CI 4.7- not reached) for real-world cohort 1. For second line, the median OS was 10.6 months (95% CI 4.7- 27.3) for real-world cohort 2 and the median PFS was 5.0 months (95% CI 2.1- 29.3). For OS analysis, results were statistically significant but not for PFS analysis. Conclusion: This study provided the clinical comparative outcomes needed for HTA evaluation.

Keywords : real world evidence, pharmacoeconomics, HTA agencies, oncology
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