OBJECTIVES: To compare expected costs per AML patient in remission using decision tree and cost-in-use analysis based on a comparative 10-country phase III clinical trial. METHODS: Using a five-year time horizon, costs were estimated from the payor’s perspective for patients in their first complete remission from AML. Clinical endpoints were remission (Leukemia Free), relapse and death. Resources consumed were derived from decision trial data and supplemented with cost information from the literature and advisors (for patients in relapse). Comparators were histamine dihydrochloride + low dose interleukin-2 (n = 129) vs. standard of care (n = 132). Unit costs were taken from UK sources including NHS reference cost, British National Formulary 56, National Blood Services and the literature for concomitant medications, blood products, emergency room visits, physician visits and relapses. Cost for interleukin was included; however, the investigated drug cost was not included in the analysis as no price has been set to date. We computed the expected cost by treatment for each method, using a 5% discount rate. RESULTS: Overall 5 year Leukemia Free Survival for treatment vs. standard of care was 2.23 years vs. 1.75 (P = 0.02), respectively. Expected costs per treatment arm for the tree method, treatment vs. standard of care, were £40,725 vs. £39,371, respectively, while for the cost-in-use method, treatment vs. standard of care, £40,209 vs. £41,725, respectively. We estimated the expected cost for the treatment arm by 1.3%, and underestimated the cost for standard of care by 5.6%. CONCLUSIONS: The two methods estimated similar values. However, the cost-in-use method yields a more accurate estimate compared to the tree method because the tree method does not adjust for events that take place between nodes, thus possibly introducing error. The cost-in-use method captures resources with known time points, minimizing over- or under-reporting of resources consumed.

BACKGROUND: Mixed treatment comparisons (MTC) is useful in comparing treatments when head-to-head comparisons are not possible. MTC can also be used to compare the effectiveness of treatments that are not directly comparable. For instance, we might be interested in comparing the effectiveness of two treatments, but only one has been studied in a head-to-head trial. The other treatment has been studied in a different trial, and we are interested in comparing the two treatments based on the results of the different trials. We used a mixed treatment comparison (MTC) approach to compare the effectiveness of two treatments, A and B, based on the results of two different trials, trial 1 and trial 2. The trials compared the effectiveness of treatments A and B against a common control, C. The trials were conducted in different settings, and the patients in the trials were not directly comparable. We used a mixed treatment comparison (MTC) approach to compare the effectiveness of treatments A and B based on the results of the two different trials. The trials compared the effectiveness of treatments A and B against a common control, C. The trials were conducted in different settings, and the patients in the trials were not directly comparable. We used a mixed treatment comparison (MTC) approach to compare the effectiveness of treatments A and B based on the results of the two different trials. The trials compared the effectiveness of treatments A and B against a common control, C. The trials were conducted in different settings, and the patients in the trials were not directly comparable.

METHODS: Proportions of HBeAg(+) CHB patients with undetectable HBV DNA at Year 1 were from published trials referenced in D&J. Bayesian MTC analyses were conducted using models and assumptions proposed in Higgins and Whitehead (H&W, 1996) and Lu and Ades (L&A-Unconstrained and L&A-Constrained, 2004). Analyses were implemented in WinBUGS v1.4. Model performance was evaluated by how well it fit observed proportions. RESULTS: The dataset was small relative to the number of comparisons evaluated. Only 10 randomized controlled trials satisfied D&J inclusion/exclusion criteria, yielding data on only 12 of 28 possible head-to-head comparisons among the 8 treatments considered (LAM-lamivudine, ADV-adenosine, ETV-entecavir, TEL-telbivudine, TDF-tenofovir, ADV+LAM, TEL-LAM, and PLB-placebo). The H&W estimates were very similar to D&J results. The estimated proportions from L&A-C are most consistent with observed data (see Table). Results could not distinguish among the four most efficacious treatments at Year 1 (ETV, TDF, TEL-LAM). CONCLUSIONS: Compared with observed data, the L&A-C model is better able to predict observed results than either the D&J, H&W or L&A-U. With limited data, MTC results can vary across models and model performance should be evaluated against observed data. Proportion of patients achieving undetectable HBV DNA at Year 1: The treatments (No. Trials) for TDF(1): 74.8% (19.6%, 72.6%), ADV(1): 75.6% (19.6%, 72.6%), ETV(1): 70.1% (58.6%, 74.5%), TDF(1): 75.6% (58.6%, 74.5%). The model performance was evaluated by how well it fit observed proportions. RESULTS: The dataset was small relative to the number of comparisons evaluated. 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