SUPPLEMENTARY tables

Supplementary Table S1. PBAC Raw Variables

| Variable | Description | Values |
| --- | --- | --- |
| Unique ID | Unique ID for each unique combination of drug name and disease (indication) | Numbers 1-214 |
| Drug name | Drug name; obtained from Public Summary Document | Text |
| Date of submission | Date of PBAC consideration; obtained from Public Summary Document | Month, year |
| Left censored | Describes whether the drug name and disease were submitted prior to July 2005; obtained from Section 2 (Background) | Yes/No |
| Previous submission | Describes whether drug and disease had a prior submission; obtained from Section 2 (Background) | Many/No/Once |
| Date of re-submission | Date of PBAC consideration of prior submission; obtained from Public Summary Document | Month, Year |
| Disease | Indication; obtained from Section 1 (Purpose of Application) | Text |
| Severity/place of disease | Describes the specific listing of the drug (i.e., severity of disease, patient population, failed treatment, criteria); obtained from Section 4 (Listing Requested and PBAC’s View) | Text |
| Disease category | Derived from review of disease; proxy measure for severity of disease | Oncology/Other |
| Patient population size | Describes the estimated number of patients receiving the drug per year or number of prescriptions per year or number of packs dispensed per year; obtained from Section 11 (Estimated PBS Usage and Financial Implications) | Text (e.g., number of patients)/Number |
| Active comparator | Submission included an active comparator; obtained from Section 6 (Comparator) | Yes/No |
| Comparative clinical evidence | Describes the level of clinical evidence presented in submission; obtained from Section 7 (Clinical Trials) | Text (e.g., randomized controlled trial, indirect comparison, meta-analysis) |
| Primary endpoint | Provides the primary endpoint or key result of clinical trials; obtained from Section 7 (Clinical Trials) and Section 8 (Results of Trials) | Text (e.g., overall survival, time to progression) |
| Surrogate endpoint | Derived based on review of primary endpoint | Yes/No |
| Manufacturer claim | Clinical claim made by the manufacturer; obtained from Section 9 (Clinical Claim) | Text (e.g., definitely superior, similar) |
| Lower bound of incremental cost per QALY | Lower value of range provided for measure; obtained from Section 10 (Economic Analysis); for those entries where cost-neutrality or cost-savings are estimated, 0 will be entered here | Number, Inequality (e.g., < A$30,000) |
| Higher bound of incremental cost per QALY | Higher value of range provided for measure; obtained from Section 10 (Economic Analysis) | Number, Inequality  (e.g., < A$30,000) |
| Timeframe of incremental cost per QALY | If provided, the timeframe used in analysis; obtained from Section 10 (Economic Analysis) | Number |
| Lower bound of financial cost to PBSa | Lower value of range provided for measure; obtained from Section 11 (Estimated PBS Usage and Financial Implications) | Number, Inequality (e.g., < A$10 million) |
| Higher bound of financial cost to PBSa | Higher value of range provided for measure; obtained from Section 11 (Estimated PBS Usage and Financial Implications) | Number, Inequality  (e.g., < A$10 million) |
| Timeframe of financial cost to PBSa | If provided, the timeframe used in analysis; obtained from Section 11 (Estimated PBS Usage and Financial Implications) | Number |
| Recommendation | PBAC’s decision to list drug; obtained from Section 12 (Recommendation and Reasons) | Yes/Partial Yes/No/Defer |

PBAC = Pharmaceutical Benefits Advisory Committee; PBS = Pharmaceutical Benefits Scheme; QALY = quality-adjusted life-year.

a There is a second set of columns for a few records that reported more than one range of values (e.g., different scenarios, timeframes).

Supplementary Table S2. Logistic Models Parameter Estimates and Odds Ratios

| Variable | Logistic Model 1a  (n = 204) | | Logistic Model 2a  (n = 238) | |
| --- | --- | --- | --- | --- |
| Parameter Estimate (SE) | Odds Ratio (95% CI) | Parameter Estimate (SE) | Odds Ratio (95% CI) |
| Financial impact  (millions A$) | Reference category: ≤0 | | | |
| >30 | –1.40  (0.86) | 0.25  (0.05-1.34) | –1.61  (0.83) | 0.20  (0.04-1.02) |
| ≥10 to ≤30 | –2.16\*  (0.76) | 0.12  (0.03-0.51) | –2.77\*  (0.77) | 0.06  (0.01-0.29) |
| >0 to <10 | –1.81\*  (0.66) | 0.16  (0.04-0.60) | –2.04\*  (0.67) | 0.13  (0.04-0.48) |
| Cost per quality-adjusted life-year (thousands A$) | Reference category: none | | | |
| >75 | –2.85\*  (0.99) | 0.06  (0.01-0.40) | –2.57\*  (0.86) | 0.08  (0.01-0.41) |
| >45 to ≤75 | –1.24  (0.73) | 0.29  (0.07-1.20) | –1.34  (0.70) | 0.26  (0.07-1.04) |
| >0 to ≤45 | –0.59  (0.60) | 0.55  (0.17-1.77) | –0.61  (0.59) | 0.54  (0.17-1.72) |
| Manufacturer claim | Reference category: noninferior or equivalent | | | |
| Superior or advantages | 0.44  (0.57) | 1.55  (0.51-4.74) | 0.35  (0.56) | 1.42  (0.47-4.30) |
| Comparative clinical evidence | Reference category: randomized control trial + meta-analysis or indirect comparison analysis | | | |
| Randomized controlled trial | 0.22  (0.34) | 1.25  (0.64-2.44) | 0.63  (0.33) | 1.89  (0.98-3.62) |
| Active comparator | Reference category: no | | | |
| Yes | 0.91\*  (0.44) | 2.49  (1.06-5.85) | 0.93\*  (0.41) | 2.54  (1.14-5.69) |
| Disease category | Reference category: other | | | |
| Oncology | –0.36  (0.45) | 0.70  (0.29-1.69) | –0.36  (0.42) | 0.7  (0.31-1.59) |
| Total submission count | Reference category: 1 | | | |
| 3 |  |  | 2.26\*  (0.69) | 9.62  (2.47-37.42) |
| 2 |  |  | 1.30\*  (0.51) | 3.66  (1.35-9.96) |
| Time since previous submission |  |  | –0.19  (0.1) | 0.83  (0.69-1.00) |

CI = confidence interval; SE = standard error.

a Logistic model 1 included only the first observed submission of each unique drug and indication combination within our database; logistic model 2 included all observed submissions of each unique drug and indication combination within our database.

\* Statistically significant.

Supplementary Table S3. Listing of Variables for those with Multiple Submissions

| ID | # Sub. | Rec. | FI. - mill | Cost per QALY | Pop. size | Claim | Design | Comp. | Onc. | Surr. End. |
| --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- |
| 6 | 3 | No | 25 | 45,000 | Med | Sup | RCT+ | PL | No | Yes |
|  | 4 | No | 30 | 75,000 | Med | Sup | RCT+ | PL | No | Yes |
|  | 5 | Yes | 10 | 45,000 | Med | Sup | RCT+ | PL | No |  |
| 7 | 1 | No | 100 | 200,000 | Med | Sup | RCT | PL | Yes | Yes |
|  | 2 | Yes | 100 | 105,000 | Med | Sup | RCT | PL | Yes | Yes |
| 15 | 2 | No | 17 | 45,000 | Low | Sup | RCT+ | PL | No | Yes |
|  | 3 | Yes | 30 | 45,000 | Low | Sup | RCT | PL | No | Yes |
| 21 | 2 | No | 60 | 105,000 | Low | Sup | RCT | Act | Yes | No |
|  | 3 | Yes | 60 | 105,000 | Low | Sup | RCT | Act | Yes | No |
| 24 | 1 | No | 30 | 45,000 | High | Sup | RCT | Act | No | Yes |
|  | 2 | Yes | 10 | 45,000 | High | Sup | RCT+ | Act | No | Yes |
| 29 | 1 | No | 60 | 75,000 | Low | Sup | RCT | PL | No | Yes |
|  | 2 | Yes | 30 | 45,000 | Low | Sup | RCT+ | PL | No | Yes |
| 55 | 2 | No | 30 | 75,000 | Low | Sup | RCT | PL | Yes | Yes |
|  | 3 | Yes | 10 | 45,000 | Low | Equiv | RCT+ | Act | Yes | Yes |
|  | 4 | Yes | 5 | 45,000 | Low | Sup | RCT | PL | Yes | Yes |
| 57 | 1 | No | 10 | 45,000 | Low | Sup | RCT | PL | No | Yes |
|  | 2 | Yes | 10 | 75,000 | Low | Sup | RCT | PL | No |  |
| 61 | 1 | No | 40 | 85,000 | Low | Sup | RCT+ | Act | No | No |
|  | 2 | Yes | 60 | 75,000 | Low | Sup | RCT+ | Act | No | No |
| 65 | 1 | No | 30 | 45,000 | Med | Equiv | RCT+ | PL | No | No |
|  | 2 | No | 10 | 15,000 | Med | Sup | RCT+ | PL | No | Yes |
|  | 3 | Yes | 10 | 15,000 | Med | Sup | RCT+ | PL | No | Yes |
| 72 | 1 | No | 10 | 45,000 | Low | Sup | RCT | PL | No | Yes |
|  | 2 | Yes | 10 | 45,000 | Med | Sup | RCT | PL | No | Yes |
| 84 | 1 | No | 30 | 75,000 | High | Sup | RCT | PL | No | No |
|  | 2 | Yes | 60 | 15,000 | High | Sup | RCT | PL | No | No |
| 92 | 2 | No | 10 | 200,000 | Low | Sup | RCT | PL | Yes | Yes |
|  | 3 | Yes | n/a | 75,000 | Low | Sup | RCT | PL | Yes | Yes |
| 93 | 2 | No | 30 | 105,000 | Low | Sup | RCT+ | Pl | Yes | Yes |
|  | 3 | Yes | 30 | 75,000 | Low | Sup | RCT+ | PL | Yes | Yes |
| 96 | 3 | No | 30 | 75,000 | Low | Sup | RCT | PL | No | No |
|  | 4 | No | 10 | 105,000 | Low | Sup | RCT+ | Act | No | No |
|  | 5 | No | 20 | 45,000 | Low | Sup | RCT+ | Act | No | No |
|  | 6 | Yes | 10 | 75,000 | Low | Sup | RCT+ | Act | No | No |
| 98 | 1 | No | 10 | 28,101 | Low | Sup | RCT | Act | Yes | Yes |
|  | 2 | Yes | 10 | 45,000 | Low | Sup | RCT | Act | Yes | No |
| 99 | 1 | No | 10 | 45,000 | Med | Sup | RCT | PL | No | No |
|  | 2 | Yes | 30 | 45,000 | Med | Sup | RCT | PL | No | No |
| 106 | 1 | No | 100 | 200,000 | High | Sup | RCT | Act | No | No |
|  | 2 | Yes | 100 | 75,000 | High | Sup | RCT | Act | No | No |
| 125 | 1 | No | 10 | n/a | Med | Equiv | RCT | Act | No | No |
|  | 2 | YES | –10 | n/a | Med | Equiv | RCT | Act | No | No |
| 129 | 1 | No | –10 | n/a | High | Equiv | RCT+ | Act | No | Yes |
|  | 2 | Yes | –30 | n/a | High | Equiv | RCT+ | Act | No | Yes |
| 145 | 1 | No | 10 | n/a | Low | Equiv | RCT+ | Act | Yes | No |
|  | 2 | Yes | 10 | n/a | Low | Equiv | RCT+ | Act | Yes | No |
| 146 | 1 | No | 1 | n/a | High | Equiv | RCT+ | Act | No | No |
|  | 2 | Yes | 10 | n/a | High | Equiv | RCT+ | Act | No | No |
| 153 | 1 | No | 10 | n/a | Med | Equiv | RCT | Act | No | Yes |
|  | 2 | Yes | 10 | n/a | Med | Equiv | RCT+ | Act | No | Yes |
| 159 | 1 | No | 10 | n/a | Low | Equiv | RCT+ | Act | No | Yes |
|  | 2 | Yes | 10 | n/a | Low | Equiv | RCT+ | Act | No |  |
| 174 | 1 | No | 10 | n/a | Low | Equiv | RCT+ | Act | No | No |
|  | 2 | Yes | –1 | n/a | Low | Equiv | RCT+ | Act | No | Yes |
| 175 | 1 | No | 30 | n/a | Med | Equiv | RCT+ | Act | No | Yes |
|  | 2 | Yes | 10 | n/a | Med | Equiv | RCT | Act | No | Yes |
| 196 | 1 | Yes | –10 | n/a | Low | Equiv | RCT+ | Act | No | Yes |
|  | 2 | Yes | n/a | n/a | Low | Equiv | RCT | Act | No | Yes |
| 204 | 1 | No | 10 | n/a | Med | Sup | RCT | Act | No | Yes |
|  | 2 | Yes | 10 | n/a | Med | Equiv | RCT | Act | No | Yes |
| 205 | 1 | No | –0.1 | n/a | Med | Equiv | RCT | Act | No | Yes |
|  | 2 | Yes | 10 | n/a | Med | Equiv | RCT | Act | No | Yes |

Act = active comparator in at least one pivotal trial; Comp = comparator; Equiv = equivalent; FI mill = financial impact in A$ millions; Med = medium; n/a = not applicable; Onc = oncology; PL = placebo comparator in all pivotal trials; Pop = population; QALY = quality-adjusted life-years gained; RCT = randomized controlled trials; RCT+ = randomized controlled trials plus meta-analysis or indirect comparison analysis; Rec = PBAC reimbursement recommendation; Surr. End. = surrogate endpoint; Sub = submissions; Sup = superior.

**Supplementary Table S4: Regression Tolerance Estimates to Check for Multicollinarity**

|  |  |
| --- | --- |
| Variable | Tolerance |
| Budget impact | 0.66 |
| Cost per quality-adjusted life-year | 0.44 |
| Active control | 0.72 |
| Oncology | 0.92 |
| Efficacy claim | 0.45 |
| Efficacy basis | 0.92 |