## Title

# N-of-1 Trials of Expensive Biological Therapies: A Third Way (Appendix A - Decision Tree) 

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Appendix A. Details of the cost analysis for the base case and for the one-way sensitivity analysis.

| Estimated Costs: Base Case |  |  |  |  |  |  |
| :---: | :---: | :---: | :---: | :---: | :---: | :---: |
| Strategy |  | Branch | Description | Prob1 | Prob2 | Cost |
| A | Start M; if response inadequate add E ; if response still inadequate switch to triple therapy | A1 | Mtx | 0.35 |  | \$1,308.84 |
|  |  | A2.1 | Mtx, M+E | 0.65 | 0.5 | \$48,460.23 |
|  |  | A2. 2 | Mtx, M+E, Trip | 0.65 | 0.5 | \$7,169.63 |
|  |  |  |  |  |  |  |
| B | Start E; if response inadequate add M ; if still inadequate switch to Triple Rx | B1 | Etc | 0.4 |  | \$51,437.88 |
|  |  | B2.1 | Etc, E+Mtx | 0.6 | 0.5 | \$52,637.65 |
|  |  | B2.2 | Etc, E+Mtx, Trip | 0.6 | 0.5 | \$11,345.75 |
| C | Do an n-of-1 trial comparing M vs. E; treat with winner for another 112 weeks; if tie use M; if both fail singly try combination M+E for 13 weeks, treating failures with triple Rx | C1 | M wins | 0.267 |  | \$6,499.60 |
|  |  | C2 | E wins | 0.3432 |  | \$42,489.68 |
|  |  | C3 | Tie | 0.238 |  | \$6,499.60 |
|  |  | C4.1 | Both fail individually; combo succeeds | 0.1521 | 0.5 | \$43,416.56 |
|  |  | C4.2 | Both fail individually; combo fails | 0.1521 | 0.5 | \$11,982.08 |


| Estimated Costs: Sensitivity Analyses |  |  |  |  |  |  |
| :---: | :---: | :---: | :---: | :---: | :---: | :---: |
| Sensitivity analysis 1: treat n -of-1 ties with combination therapy (most expensive choice) (affects branch C only) |  |  |  |  |  |  |
| Strategy |  | Branch | Description | Prob1 | Prob2 | Cost |
| C |  | C1 | M wins | 0.267 |  | \$6,499.60 |
|  |  | C2 | E wins | 0.3432 |  | \$42,489.68 |
|  |  | C3 | Tie | 0.238 |  | \$43,416.56 |
|  |  | C4.1 | Both fail | 0.1521 | 0.5 | \$43,416.56 |
|  |  | C4.2 |  | 0.1521 | 0.5 | \$11,982.08 |
|  |  |  |  |  |  |  |
| Sensitivity analysis 2: use ACR20 as the criterion |  |  |  |  |  |  |
| Strategy |  | Branch | Description | Prob1 | Prob2 | Cost |
| A | Start M; if response inadequate add E ; if response still inadequate switdh to triple therapy | A1 | Mtx | 0.6 |  | \$1,308.84 |
|  |  | A2.1 | Mtx, M + E | 0.4 | 0.75 | \$48,460.23 |
|  |  | A2. 2 | Mtx, M+E, Trip | 0.4 | 0.25 | \$7,169.63 |
| B | Start E; if response inadequate add M ; if still inadequate switch to Triple Rx | B1 | Etc | 0.65 |  | \$51,437.88 |
|  |  | B2.1 | Etc, E+Mıx | 0.4 | 0.75 | \$52,637.65 |
|  |  | B2.2 | Etc, E+Mtx, Trip | 0.4 | 0.25 | \$11,345.75 |
| C | Do an n-of-1 trial comparing M vs. E; treat with winner for another 112 weeks; if tie use M; if both fail singly try combination $\mathrm{M}+\mathrm{E}$ for 13 weeks, treating failures with triple Rx | C1 | M wins | 0.267 |  | \$6,499.60 |
|  |  | C2 | E wins | 0.3264 |  | \$42,489.68 |
|  |  | C3 | Tie | 0.3705 |  | \$6,499.60 |
|  |  | C4.1 | Both fail individually; combo succeeds | 0.0196 | 0.75 | \$43,416.56 |
|  |  | C4.2 | Both fail individually; combo fails | 0.0196 | 0.5 | \$11,982.08 |

