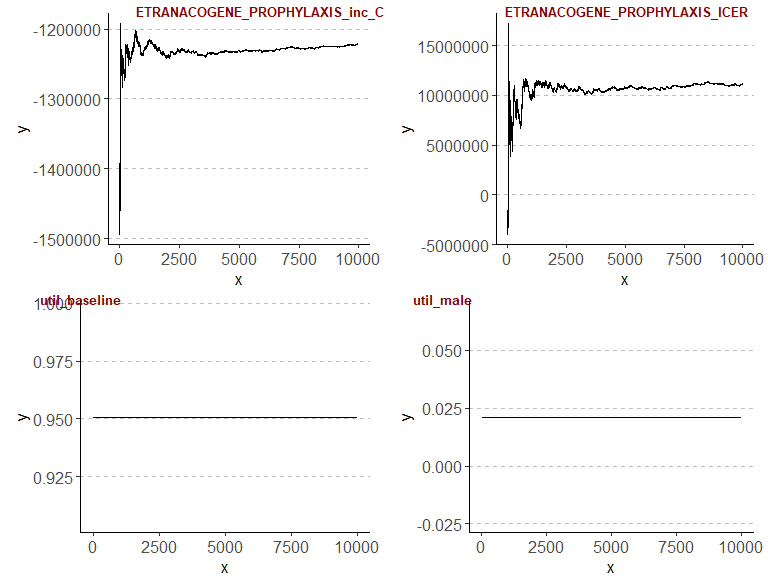
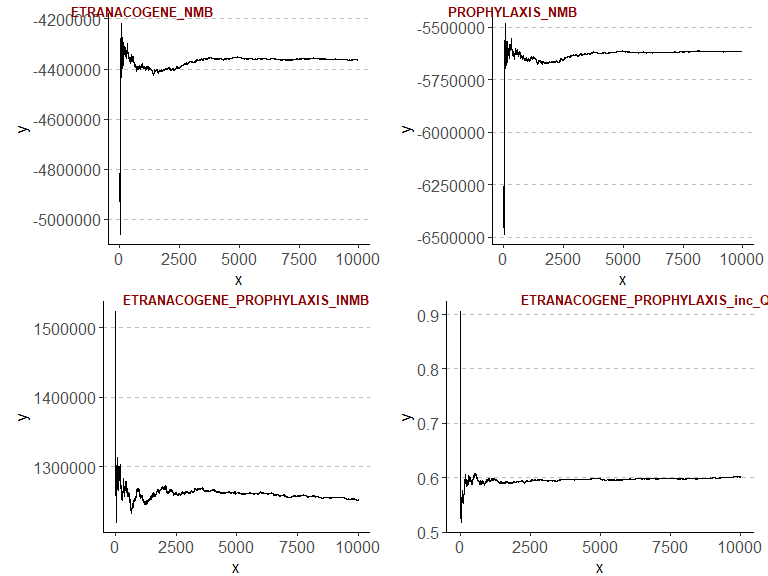
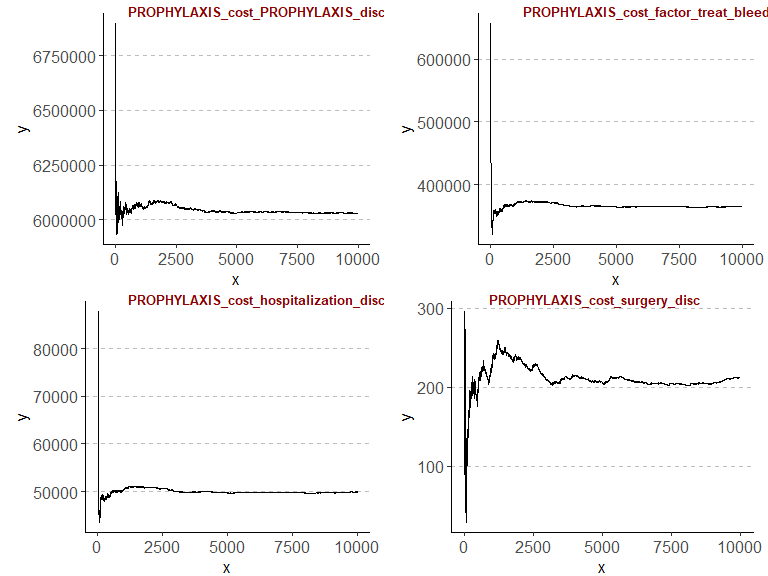
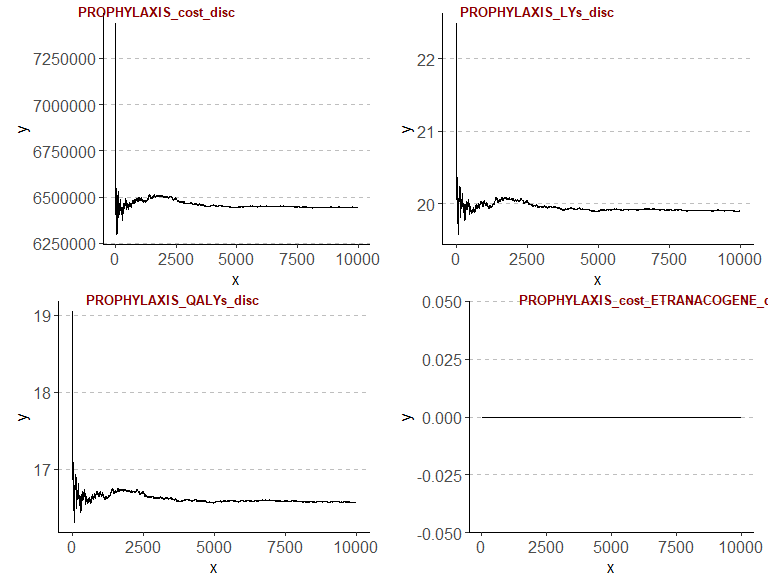
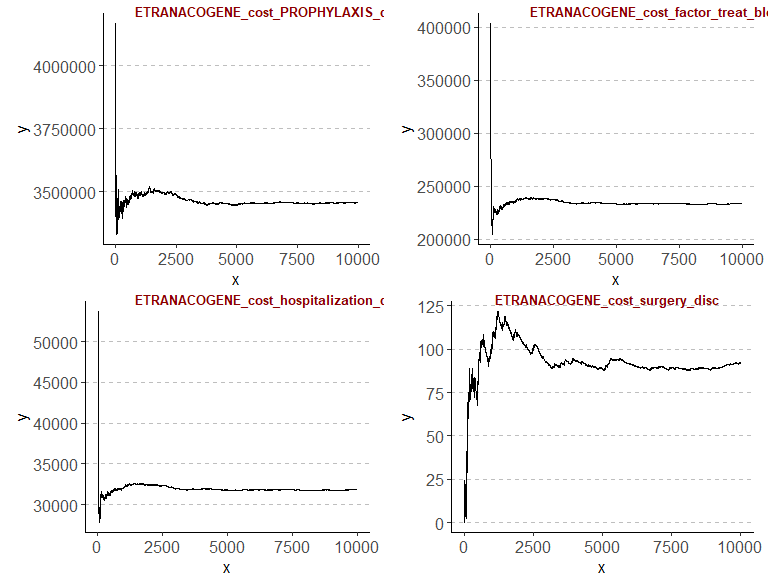
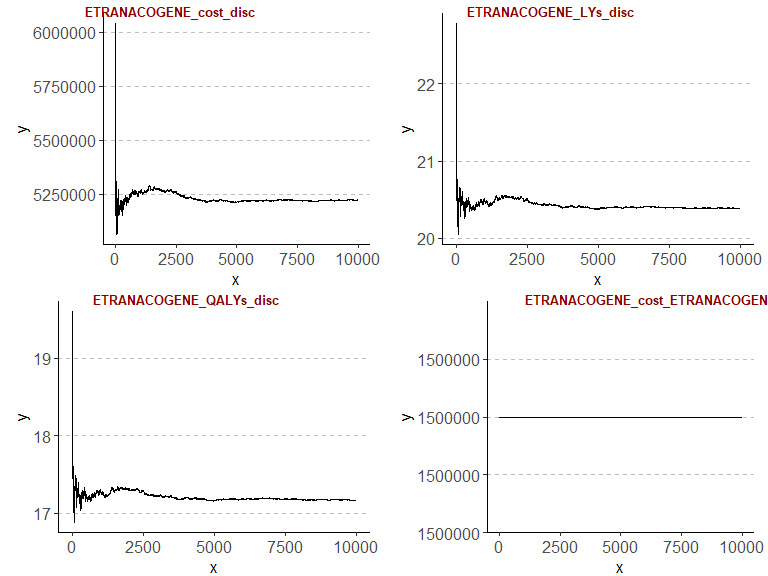
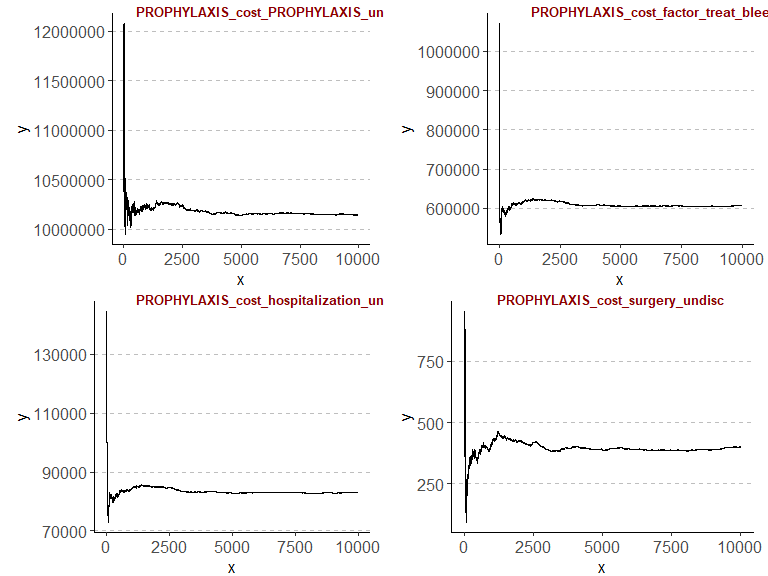
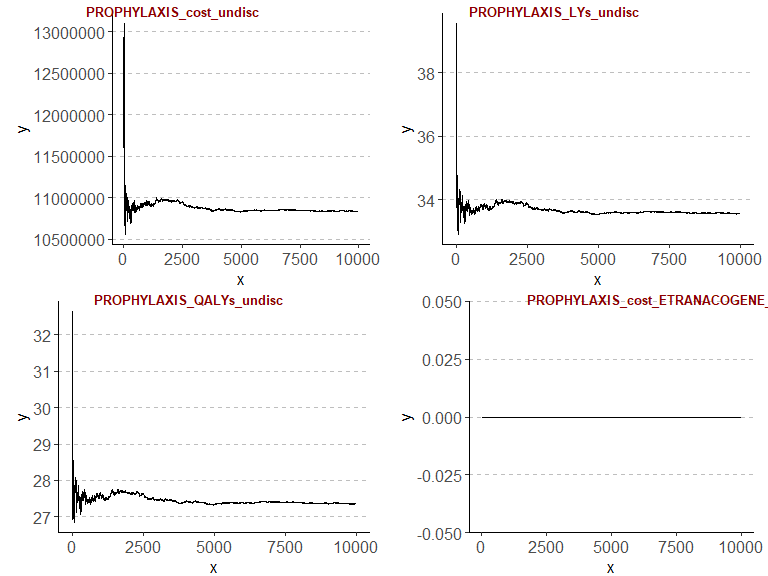
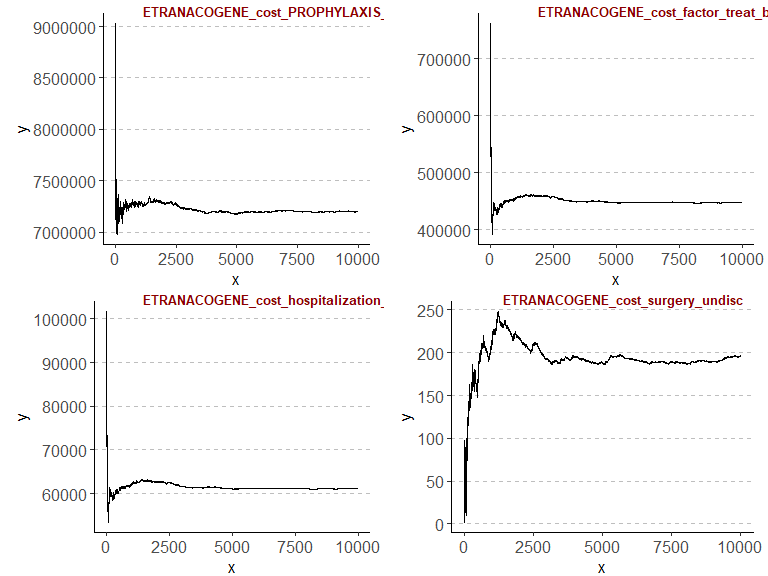
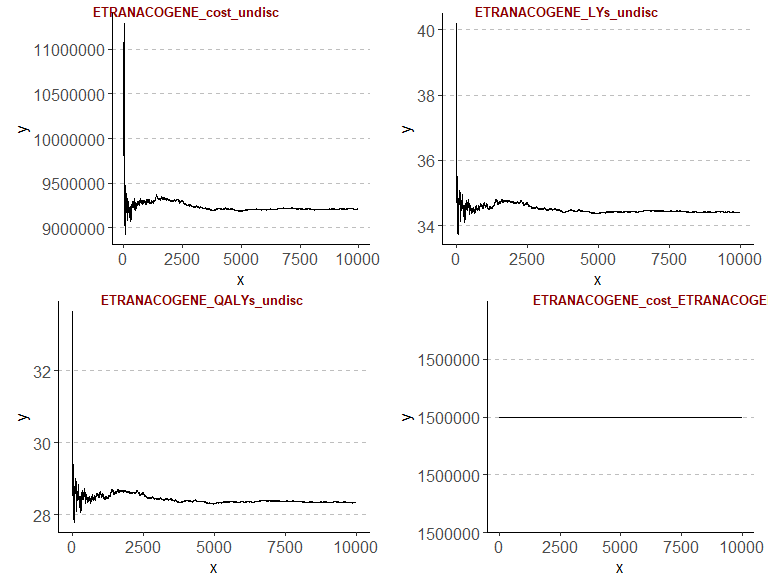
Analysis of HB CE deterministic model

Niklaus Meier

November 14, 2023

## [1] "These plots show the convergence of various outcomes as more patients are added to generate the running mean."



Diagnostics of convergence of running incremental outcomes over all patients in simulation

| ETRA vs. PROPH inc. QALYs | ETRA vs. PROPH inc. Costs | ETRA vs. PROPH inc. NMB |
| --- | --- | --- |
| 0 | 0 | 0 |
| 0 | 0 | 0 |
| 0 | 0 | 0 |
| 0 | 0 | 0 |
| 0 | 0 | 0 |
| 0 | 0 | 0 |
| 0 | 0 | 0 |
| 0 | 0 | 0 |
| 0 | 0 | 0 |
| 0 | 0 | 0 |

## [1] "We used intervals of 1000 patients, and checked whether the running mean of the incremental outcomes change by more than 1%."

## [1] "A 0 means that we have reached a stable value, i.e. we have achieved convergence and have a sufficient sample size, using this interval and this percentage change as a measure of sufficient stability."

Comparison undiscounted outcomes between ETRANACOGENE and Prophylaxis

| Treatment | LYs | QALYs | Costs | NMB | ICER |
| --- | --- | --- | --- | --- | --- |
| ETRANACOGENE | 34.40 | 28.34 | 9211920 | 0 | 0 |
| Prophylaxis | 33.56 | 27.36 | 10838802 | 0 | 0 |
| Increment | 0.84 | 0.98 | -1626882 | 0 | 0 |

Comparison discounted outcomes between ETRANACOGENE and Prophylaxis

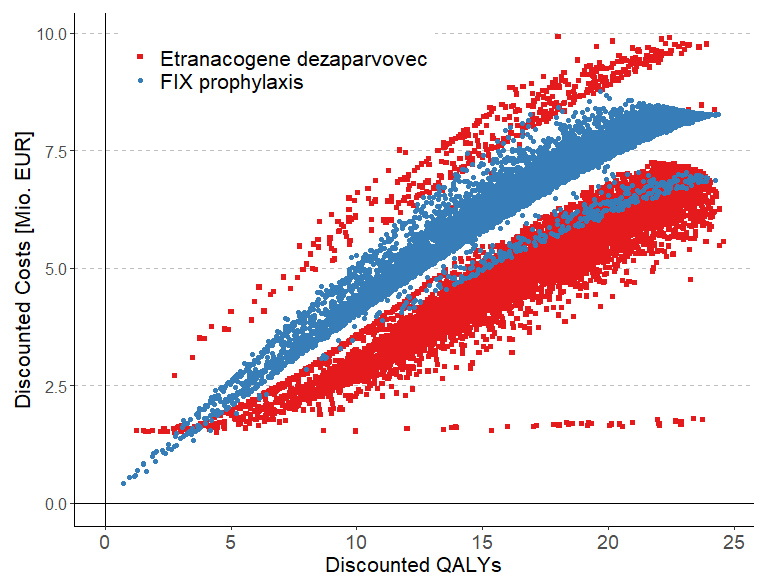
| Treatment | LYs | QALYs | Costs | NMB | ICER |
| --- | --- | --- | --- | --- | --- |
| ETRANACOGENE | 20.39 | 17.17 | 5223105 | -4364641 | 0 |
| Prophylaxis | 19.90 | 16.57 | 6444970 | -5616597 | 0 |
| Increment | 0.49 | 0.60 | -1221865 | 1251956 | -2030303 |

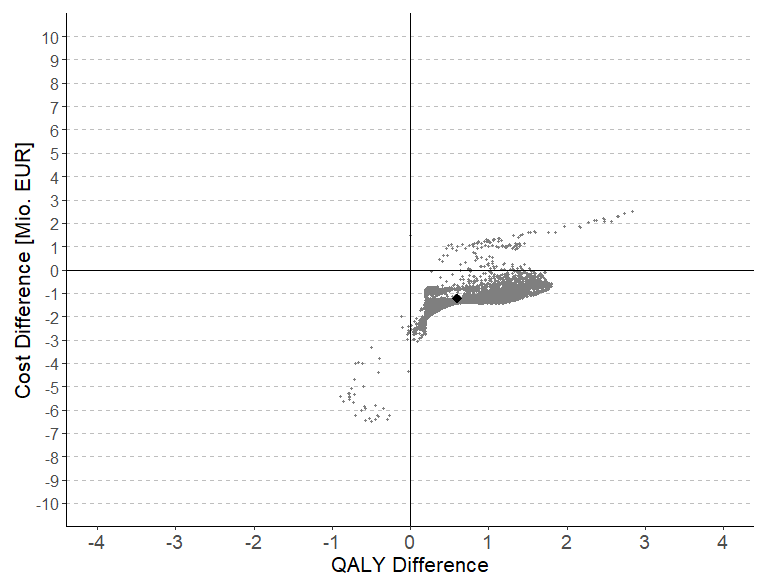
Undiscounted costs broken down by category

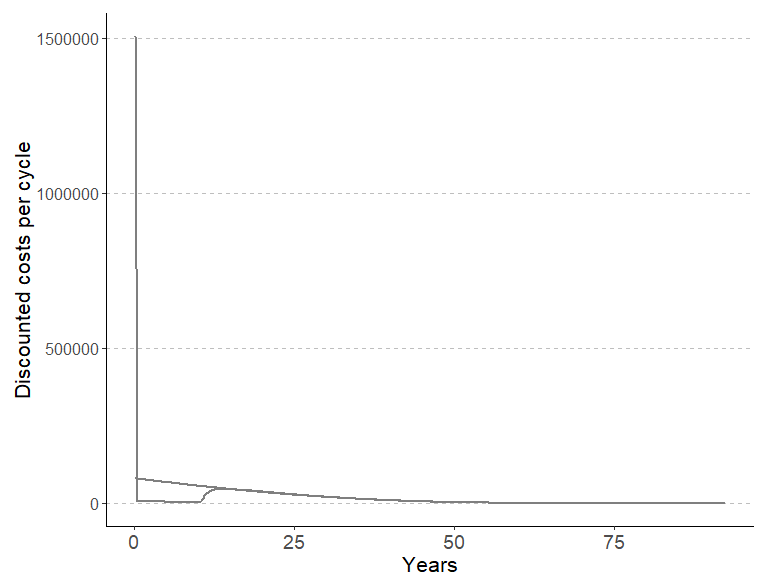
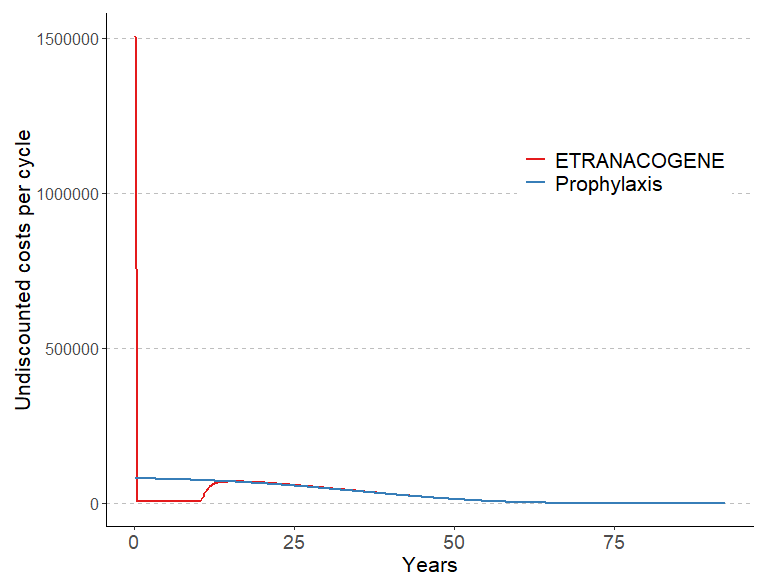
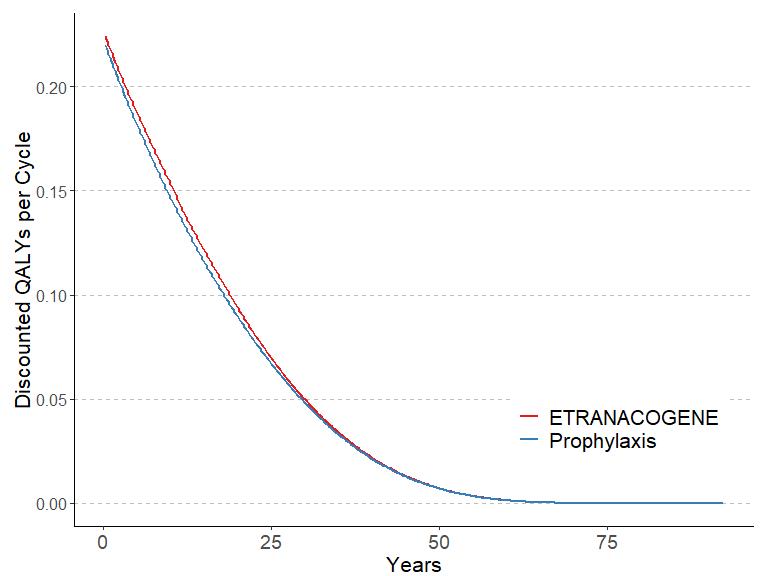
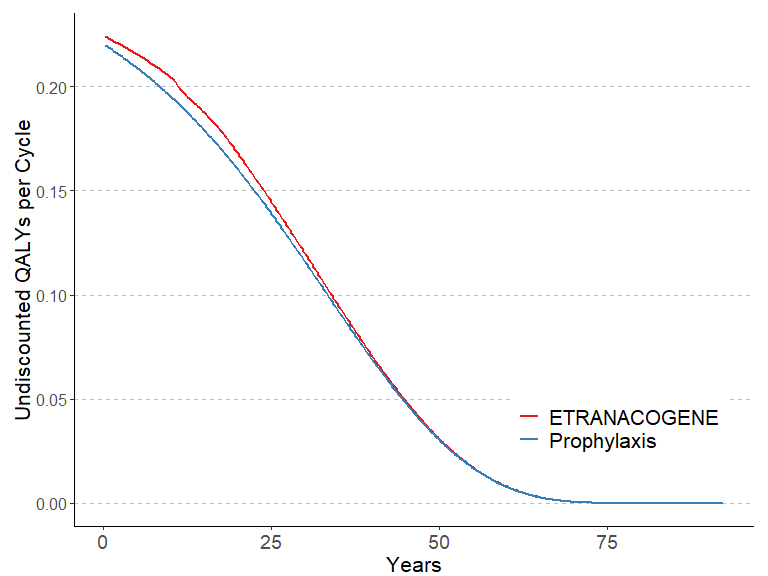
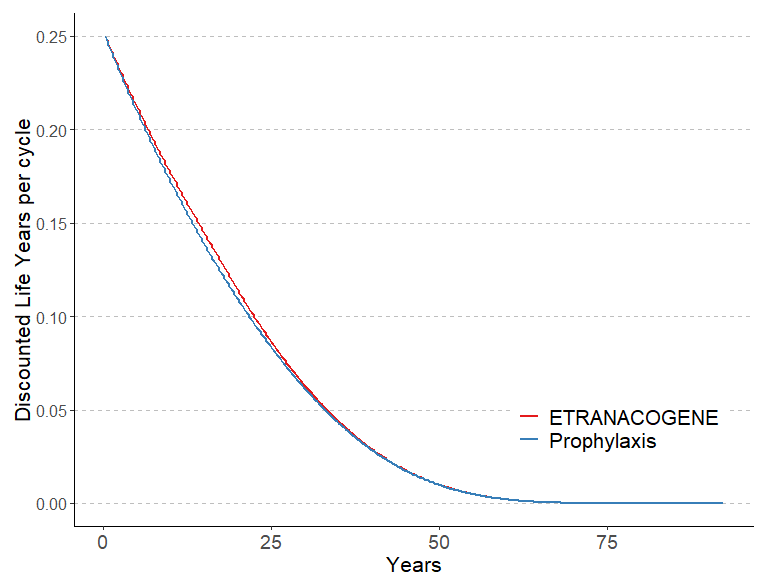
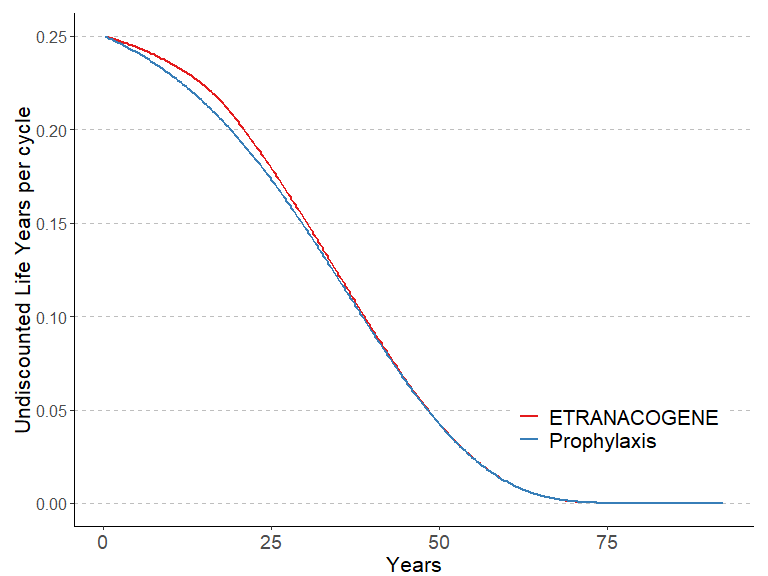
| Treatment | ETRANACOGENE | FIX Prophylaxis | FIX for treatment of bleeds | Hospitalization | Surgery | Total |
| --- | --- | --- | --- | --- | --- | --- |
| ETRANACOGENE | 1500000 | 7202197 | 448284.3 | 61242.11 | 196.19 | 9211920 |
| FIX Prophylaxis | 0 | 10148127 | 607092.5 | 83181.57 | 400.44 | 10838802 |

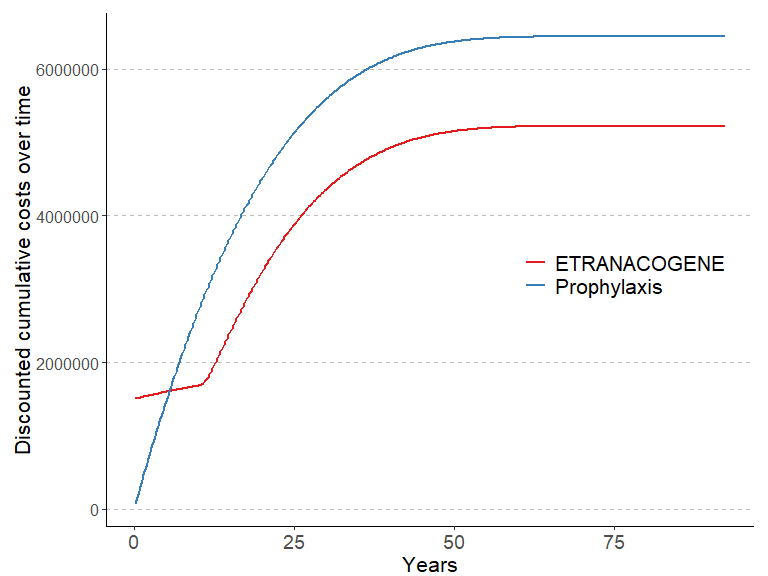
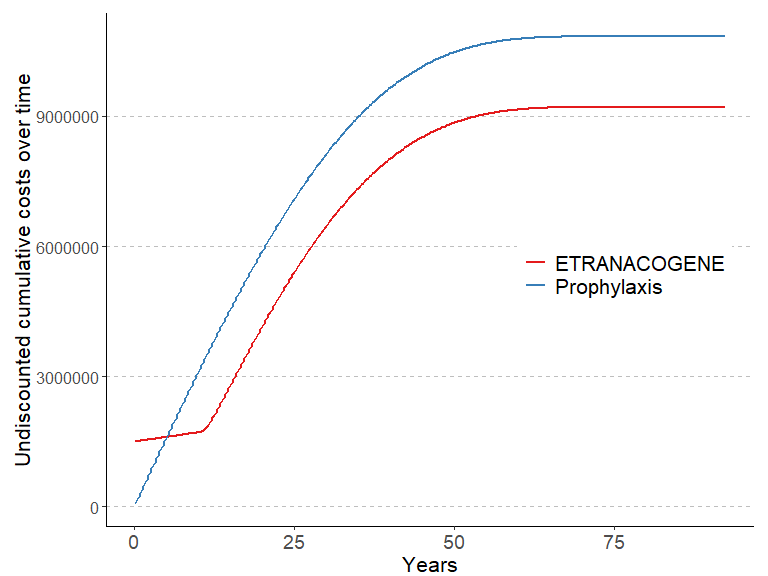
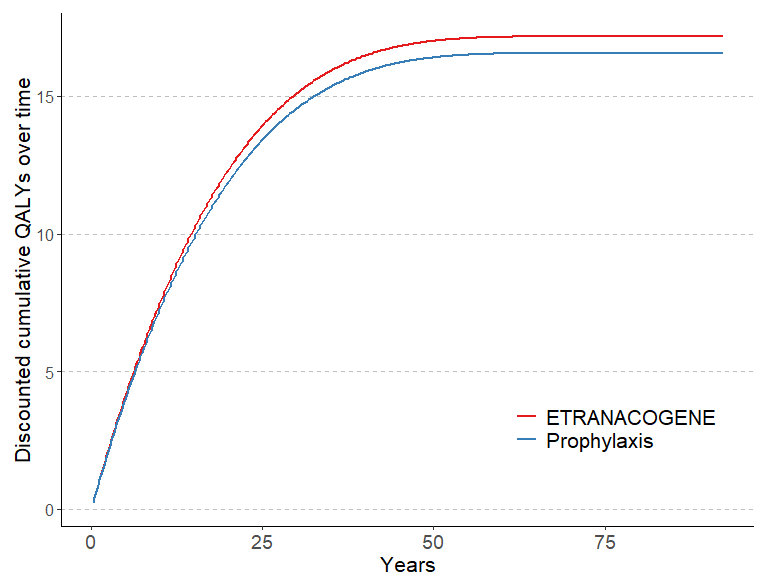
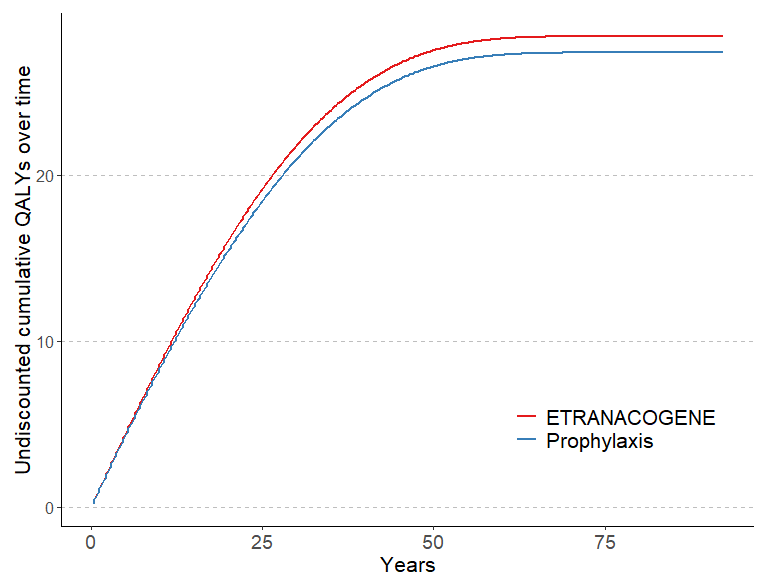
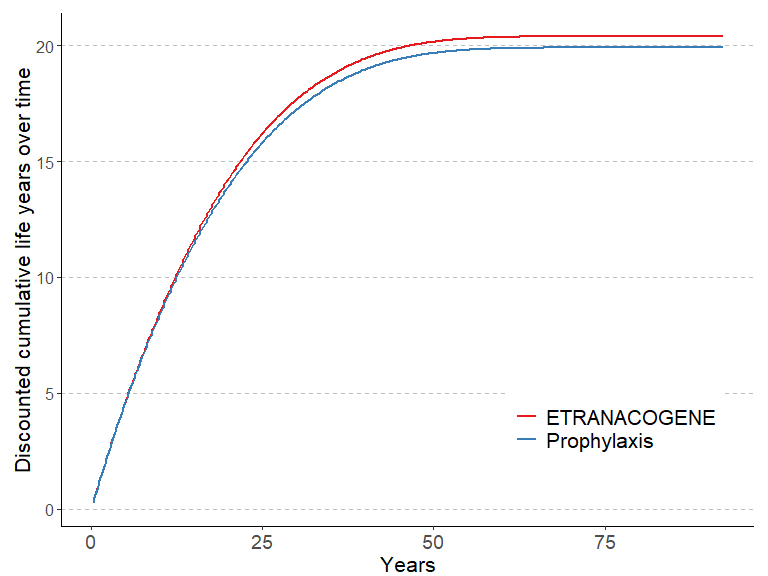
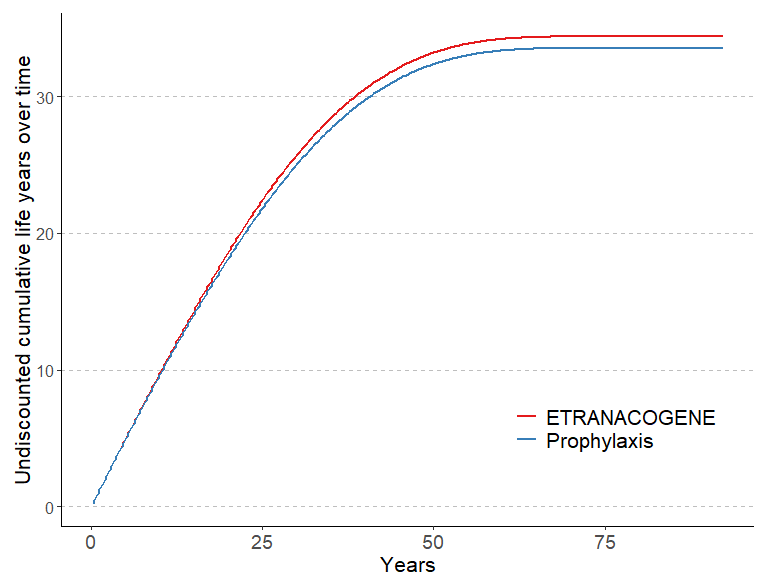
Discounted costs broken down by category

| Treatment | ETRANACOGENE | FIX Prophylaxis | FIX for treatment of bleeds | Hospitalization | Surgery | Total |
| --- | --- | --- | --- | --- | --- | --- |
| ETRANACOGENE | 1500000 | 3457338 | 233834.7 | 31840.27 | 92.39 | 5223105 |
| FIX Prophylaxis | 0 | 6029393 | 365433.5 | 49931.47 | 212.61 | 6444970 |









# System information

Sys.info()

sysname release version nodename machine login user effective\_user   
 "Windows" "10 x64" "build 19045" "M04486" "x86-64" "min1" "min1" "min1"

sessionInfo(package = NULL)

R version 4.3.1 (2023-06-16 ucrt) Platform: x86\_64-w64-mingw32/x64 (64-bit) Running under: Windows 10 x64 (build 19045)

Matrix products: default

locale: [1] LC\_COLLATE=German\_Switzerland.utf8 LC\_CTYPE=German\_Switzerland.utf8 LC\_MONETARY=German\_Switzerland.utf8 LC\_NUMERIC=C LC\_TIME=German\_Switzerland.utf8

time zone: Europe/Zurich tzcode source: internal

attached base packages: [1] stats graphics grDevices utils datasets methods base

other attached packages: [1] rmarkdown\_2.23 ggrepel\_0.9.3 plyr\_1.8.8 ggpubr\_0.6.0 truncnorm\_1.0-9 lookup\_1.0 data.table\_1.14.8 pander\_0.6.5 RColorBrewer\_1.1-3 ggplot2\_3.4.2  
[11] kableExtra\_1.3.4

loaded via a namespace (and not attached): [1] utf8\_1.2.3 generics\_0.1.3 tidyr\_1.3.0 rstatix\_0.7.2 xml2\_1.3.5 stringi\_1.7.12 digest\_0.6.33 magrittr\_2.0.3 evaluate\_0.21 grid\_4.3.1 fastmap\_1.1.1  
[12] backports\_1.4.1 httr\_1.4.6 rvest\_1.0.3 purrr\_1.0.1 fansi\_1.0.4 viridisLite\_0.4.2 scales\_1.2.1 abind\_1.4-5 cli\_3.6.1 crayon\_1.5.2 rlang\_1.1.1  
[23] cowplot\_1.1.1 munsell\_0.5.0 withr\_2.5.0 yaml\_2.3.7 tools\_4.3.1 ggsignif\_0.6.4 dplyr\_1.1.2 colorspace\_2.1-0 webshot\_0.5.5 broom\_1.0.5 vctrs\_0.6.3  
[34] R6\_2.5.1 lifecycle\_1.0.3 stringr\_1.5.0 car\_3.1-2 pkgconfig\_2.0.3 pillar\_1.9.0 gtable\_0.3.3 glue\_1.6.2 Rcpp\_1.0.11 systemfonts\_1.0.4 highr\_0.10  
[45] xfun\_0.39 tibble\_3.2.1 tidyselect\_1.2.0 rstudioapi\_0.15.0 knitr\_1.43 farver\_2.1.1 htmltools\_0.5.5 labeling\_0.4.2 carData\_3.0-5 svglite\_2.1.2 compiler\_4.3.1