Real-world evidence from a federated data network to reduce uncertainty in the long-term extrapolations of oncology treatment effects

Jeremy Dietz[1], Ravinder Claire[1], James Love-Koh[1], Jamie Elvidge[1], Danielle Newby[2], Edward Burn[2], Dalia Dawoud[1]

[1] Science, Evidence & Analytics (SEA), National Institute for Health and Care Excellence

[2] Nuffield Department of Orthopaedics Rheumatology and Musculoskeletal Sciences (NDORMS), University of Oxford

Background

The National Institute for Health and Care Excellence (NICE) participates in international research projects, such as the European Health Data and Evidence Network (EHDEN), to collaborate on developing innovative methods and new ways of working. This supports our transformation plan to focus on what matters most, create advice that is useful and useable, and learn from data and implementation. The European Health Data and Evidence Network (EHDEN) is a federated network of real-world data partners from across Europe, standardized to the Observation Medical Outcomes Partnership Common Data Model (OMOP CDM). We demonstrate how EHDEN can provide real-world evidence (RWE) that can be used not only to inform the selection of long-term overall survival extrapolations but also to inform comparator survival estimates in the case of single-arm trials.

What we've done

Observational data on overall survival for 7 common cancers (breast, colorectal, lung, liver, pancreas, prostate, stomach) and one rarer cancer (head and neck) were obtained from the UK Clinical Practice Research Datalink (CPRD) between 2000 and 2019.

Parametric curves (Gompertz, Weibull, Exponential, Log-logistic, Log-normal, Generalised Gamma, Spline 1 knot, Spline 3 knots) were fitted to extrapolate long-term survival. The corresponding graphs and goodness of statistical fit measures (AIC & BIC) were uploaded to a user-friendly interactive R Shiny dashboard.

Outcomes and impact

The potential impact of these results for health technology assessment (HTA) is two-fold:

1. These results can help reduce uncertainty in determining the most plausible long-term extrapolations. In this instance RWE data often covers a longer time period (~14 years) than data available from a trial. Additionally, RWE offers a larger sample size, thereby increasing power.
2. In the case of single-arm trials, RWE could be used in a naive comparison to inform comparator survival estimates.

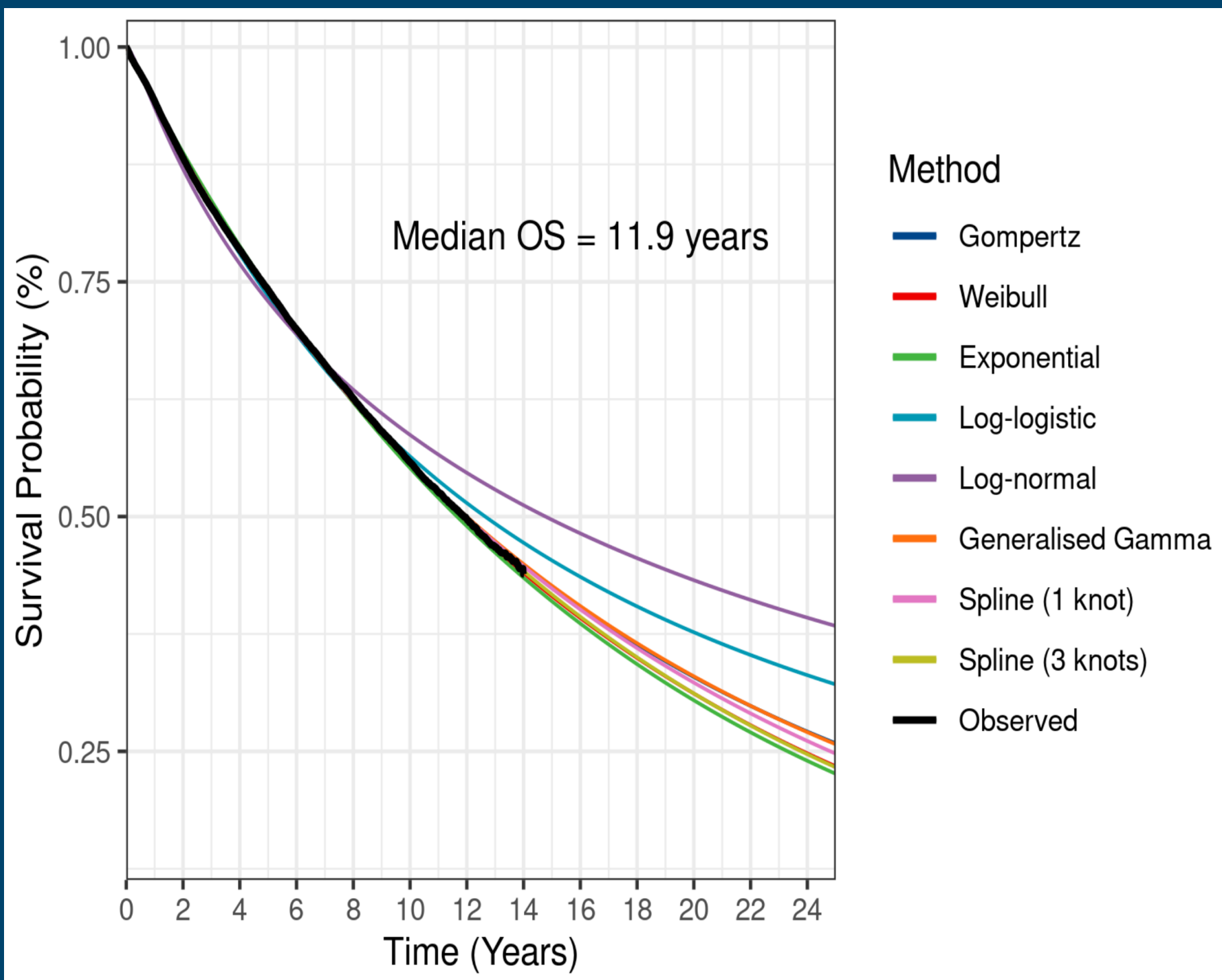Images from the survival dashboard are presented below.

Next steps

Currently, results are only available from the UK CPRD. However, additional EHDEN data partners from across Europe are actively participating in this study.

Data partners will conduct cohort diagnostics to assess the feasibility of running the complete study package on their data. If feasible, they will proceed with the full analysis and then upload their aggregate results to the interactive Shiny dashboard.

A study-a-thon is being organized for January 2024 to gather collaborators for discussion of results and output preparation.

### Prostate cancer survival outputs from the EHDEN Cancer Data Dashboard

Median OS = 11.9 years

Method: Gompertz, Weibull, Exponential, Log-logistic, Log-normal, Generalised Gamma, Spline (1 knot), Spline (3 knots), Observed

0	2	4	6	8	10	12	14
95855	65863	44771	29243	17735	9353	3835	58

Extrapolated survival curves

Method	AIC	BIC
Spline (3 knots)	196482.5	196529.9
Spline (1 knot)	196521.7	196550.1
Generalised Gamma	196526.9	196555.3
Gompertz	196536.9	196555.9
Weibull	196552.2	196571.2
Exponential	196560.8	196570.3
Log-logistic	196621.0	196639.9
Log-normal	197892.0	197910.9

Goodness of fit
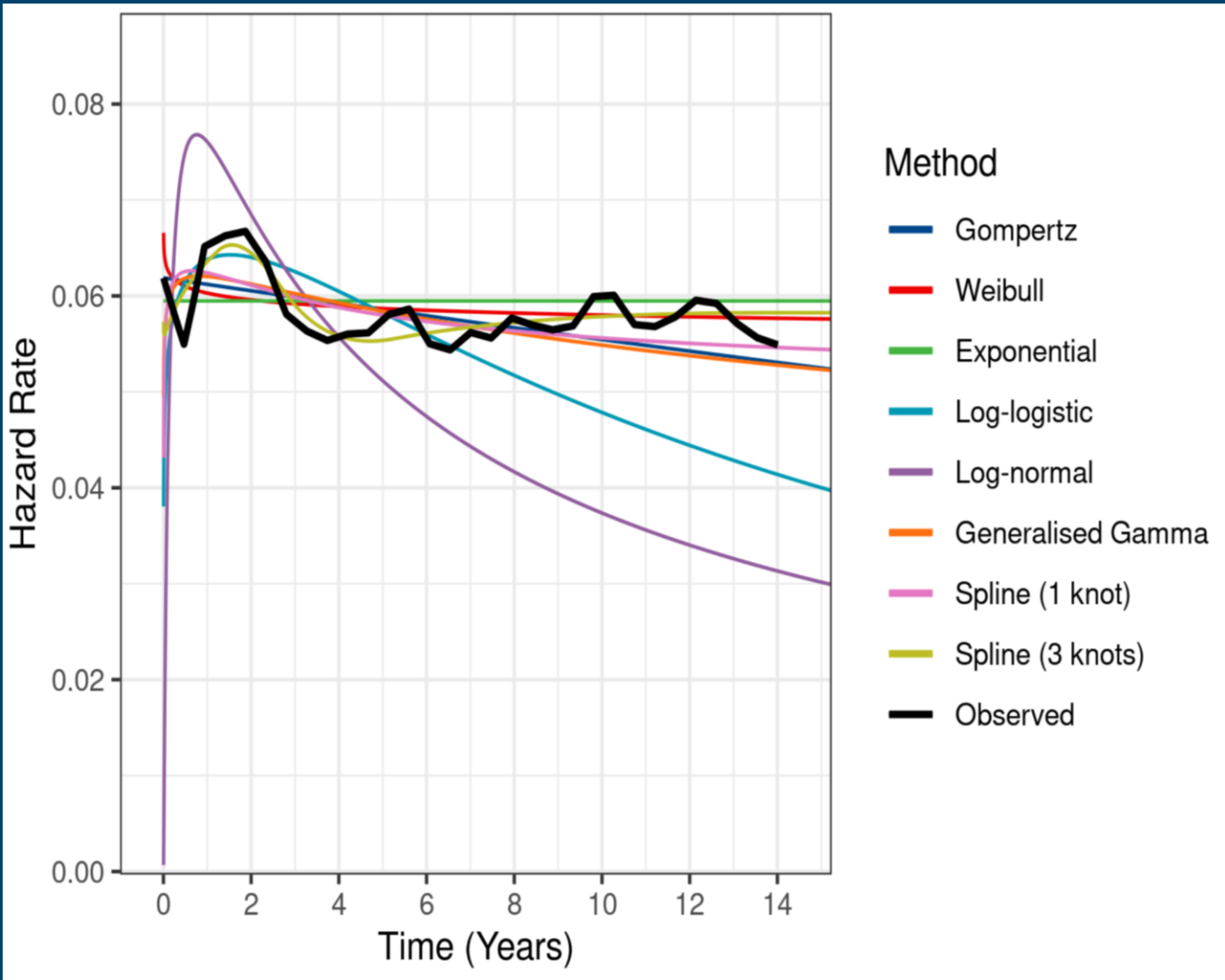
Smoothed hazard plots
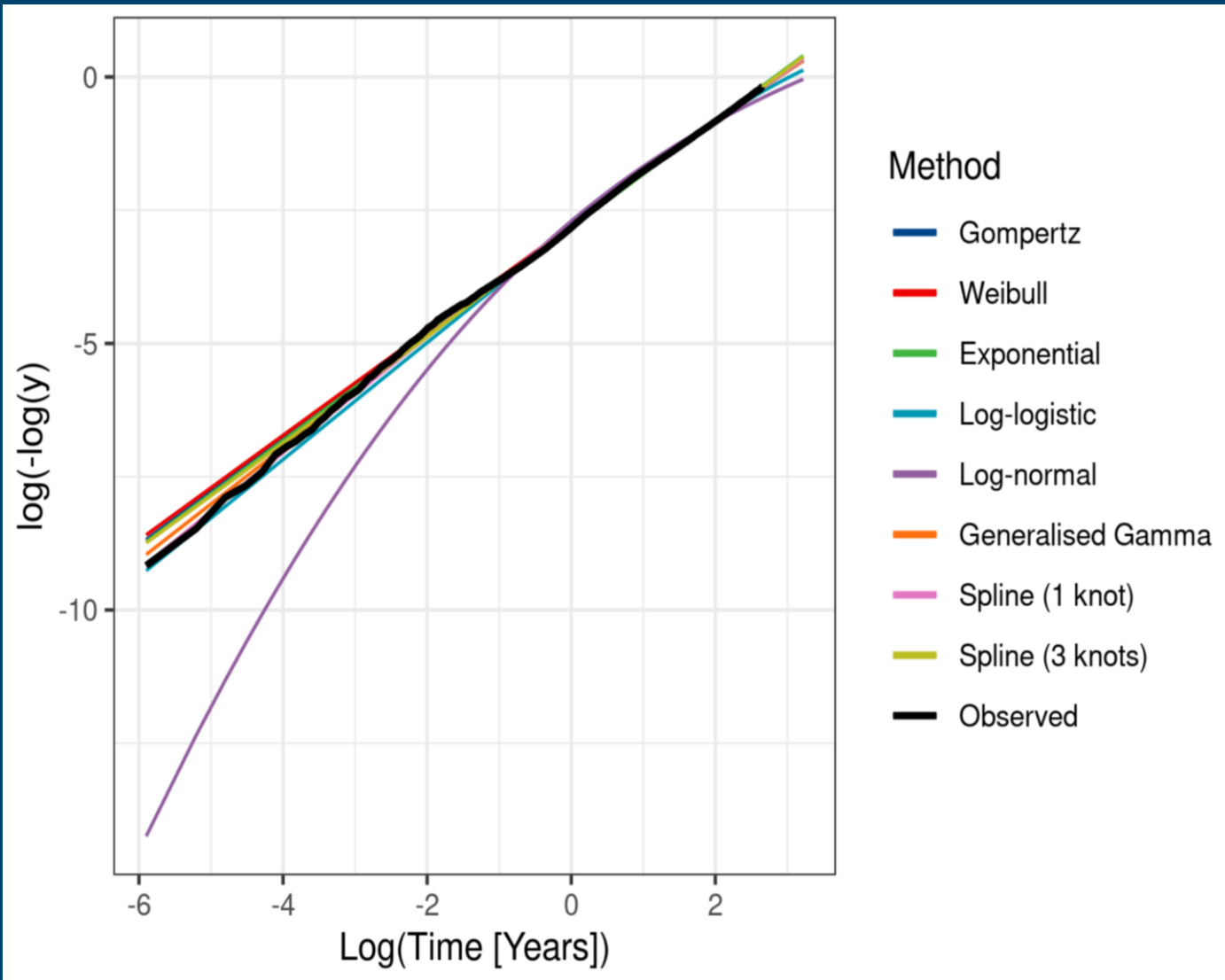
Log cumulative hazard plots

EHDEN Academy
To learn more about EHDEN and the basics behind HTA, consider signing up to the EHDEN academy. A free learning resource for anyone interested in RWE.

Jeremy Dietz (he/him)
Scientific Adviser
National Institute for Health and Care Excellence
Email: Jeremy.Dietz@nice.org.uk
Telephone: (+44) 020 7045 2146