

**Utilising real-world evidence for health technology assessment**

**Development of a cancer survival use case**

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**INTRODUCTION**

- In cancer health technology assessments (HTA), extrapolation techniques are used to estimate overall survival in people who receive a treatment, beyond observed trial data.
- This is important to inform economic evaluations used for HTA, which assess cost effectiveness over a lifetime.
- However, this is a key source of decision uncertainty because it involves forecasting the future based on shorter-term observed data.
- Real-world evidence can help address this uncertainty. For example, country-specific, real-world survival data for patients receiving the current standard of care could be used to validate the trial-based survival extrapolations typically used in HTA processes.
- Here, we describe an EHDEN use case that is in development to demonstrate how real-world data from the OMOP-CDM could help address this priority issue for HTA.

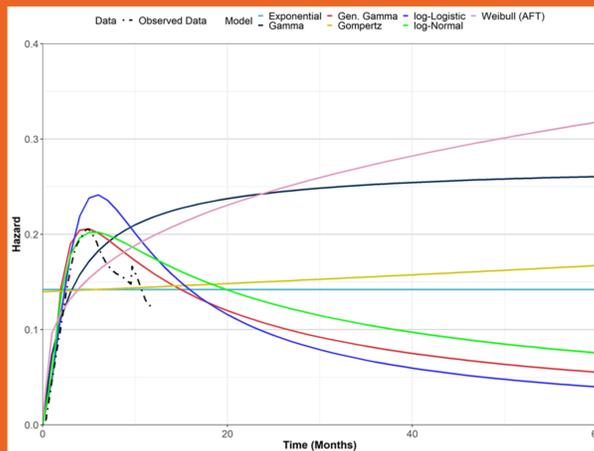
**AIMS**

Using CPRD data in the first instance, we aim to:

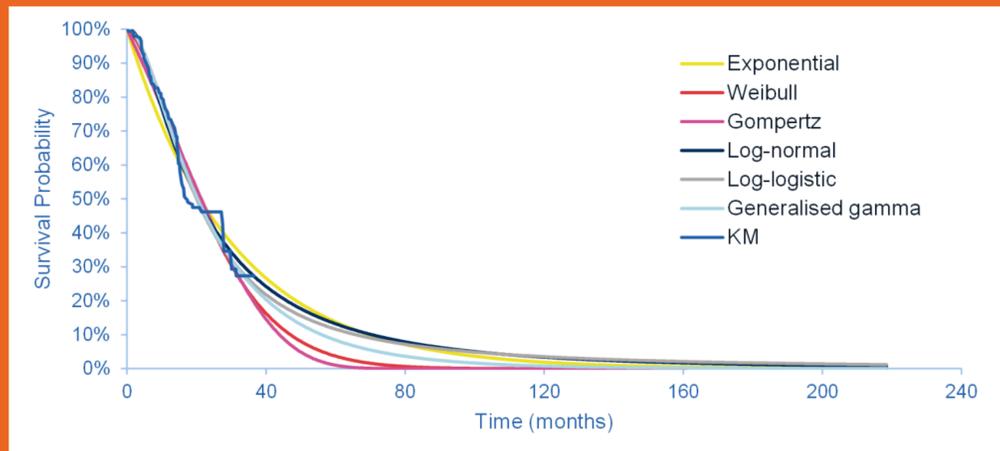
- Develop and assess data quality of phenotypes for the identification of breast, colorectal, head and neck, lung, liver, prostate, and stomach cancer.
- Estimate overall survival of the studied cancers, stratifying by key demographic variables and comorbidity.
- Fit standard parametric survival functions to the data to extrapolate long-term natural history of the studied cancers, including visual and statistical goodness of fit.
- Include these outputs in a user-friendly, interactive 'EHDEN Cancer Survival Data Dashboard'.

# We aim to develop an **EHDEN Cancer Survival Dashboard**, allowing users to quickly examine **survival data** and explore **long-term projections**.

**Smoothed hazard plots**



**Extrapolated survival curves**



**Goodness of fit**

Parametric Model	Akaike Information Criterion	Bayesian Information Criterion	3-year survival estimate	5-year survival estimate
Exponential	346.4	349.1	31.4%	14.6%
Generalised Gamma	342.5	350.6	25.5%	9.3%
Gompertz	345.0	350.3	21.9%	1.5%
Log-logistic	<b>340.3</b>	<b>345.6</b>	25.8%	11.9%
Log-normal	341.3	346.7	28.4%	13.6%
Weibull	341.6	347.0	22.0%	4.0%

Potential survival outputs from the EHDEN Cancer Data Dashboard. Please note, these figures are samples and have been intentionally de-labelled for illustration purposes. Data from NICE technology appraisal 722: Pemigatinib for treating relapsed or refractory advanced cholangiocarcinoma with FGFR2 alterations.

**RESULTS**

- We engaged with potential end users of the Dashboard—academic experts, HTA decision makers, industry experts and potential EHDEN data partners—to understand what information would be most useful to them.
- Some of the potential outputs identified from this consultation can be found in Figure 1.

**DISCUSSION**

- The development of this survival analysis use case will demonstrate a potential benefit of EHDEN and OHDSI tools to address priority areas of HTA agencies and industry stakeholders.
- The main strengths of this use case are likely to be a large sample size and the observational nature of the data, meaning its outputs will be representative of real-world clinical practice and outcomes.
- The development of the EHDEN Cancer Survival Data Dashboard will encourage clear and transparent reporting.
- From an HTA perspective, the dashboard will allow alternative parametric functions for survival extrapolation to be overlaid on observed Kaplan-Meier curves.
- This could have real benefits to healthcare reimbursement decisions based on HTA—for example, by informing comparator survival estimates where there is only a single-arm study, or by validating survival estimates from a clinical trial in the most relevant, real-world population.
- Other identified priority areas for HTA will be considered in future EHDEN HTA use cases in other disease areas.

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