## On the role of parametrisation in models with a misspecified nuisance component

Heather Battey Department of Mathematics, Imperial College London

ISNPS, June 26, 2024

#### Statistical models

Provisional base for scientific understanding.

Aspects of core scientific interest are often relatively securely specified in terms of a small number of interpretable parameters.

Other aspects may be chosen somewhat arbitrarily, typically on the basis of mathematical convenience.

Prospect of misspecification in the nuisance component is high.

A motivating example

## Treatment effects and block effects

Matched pairs. One individual from each pair chosen at random to receive a treatment, the other is the control.

Outcomes  $(Y_{i1}, Y_{i0})$  exponentially distributed of rates  $\gamma_i \psi$  and  $\gamma_i / \psi$ .

Treatment param.  $\psi$ ; *n* nuisance parameters  $\gamma_1, \ldots, \gamma_n$  encapsulate arbitrary dependence on (perhaps unmeasured) covariates. Semipara.

## Treating pair effects as fixed

If  $\gamma_1, \ldots, \gamma_n$  are treated as fixed arbitrary constants they can be eliminated from the analysis by taking ratios  $Z_i = Y_{i1}/Y_{i0}$ .

Density function: 
$$f_Z(z;\psi) = rac{\psi^2}{(1+\psi^2 z)^2}$$
 no dependence on  $\gamma_i$ .

This is what I would do, but ...

### Treating pair effects as random

An alternative approach treats pair-specific parameters as random. Convenient choice of distribution in previous example, e.g. gamma. A lengthy calculation (Battey and Cox, 2020) shows that  $\hat{\psi}$  is consistent in spite of arbitrary misspecification of the distribution for  $(\gamma_i)_{i=1}^n$ .

## Questions

How sensitive is this conclusion to the formulation of the model?

- Can the response distribution be changed?
- Can the parametrisation be changed?
- Can the assumed distribution on  $\gamma_1, \ldots, \gamma_n$  be changed?

Is there identifiable structure in the model that led to consistency of the MLE for the interest parameter?

Results for general models specialised to particular cases.

#### Motivation and scope

Provide insight into the structure of inference at the population level under misspecification.

Reference to the unsolved nature of the question being addressed, e.g.:

[...] if the model is misspecified there is no guarantee that the estimator will be consistent or even close to the true value.

Evans and Didelez (2024+). Parametrising and simulating from causal models (with discussion). J. R. Statist. Soc. B, to appear.

Misspecification: early history

## Cox (1961, 1962)

Parametric model to be fitted:  $m'(y; \theta), y = (y_1, \dots, y_n)$ True model m(y). MLE  $\hat{\theta} \rightarrow_p \theta_m^0$ , where  $\theta_m^0$  solves

 $\mathbb{E}_m[
abla_ heta \log m'(Y; heta)]_{ heta= heta_m^0}=0.$ 

Equivalently  $\theta_m^0$  minimizes with respect to  $\theta$  the Kullback-Leibler divergence

$$\int m(y) \log \left\{ \frac{m(y)}{m'(y;\theta)} \right\} dy.$$

Asymptotic variance given by the "sandwich formula". Tests of separate families (assumes one is correct).

## Elaboration and development

Huber (1967): regularity conditions etc. White (1982): identifiability of  $\theta_m^0$ ; tests of misspec.

### The inferential target

This talk: the inferential target has a stable interpretation in the true model and the fitted model. Other aspects are needed to complete the specification: these might be misspecified.

## Formalisation

Battey, H. S. and Reid, N. (2024). On the role of parametrisation in models with a misspecified nuisance component. *arXiv:2402.05708* 

#### Misspecified nuisance component

True density function *m* for outcomes parametrised in terms of an interest parameter  $\beta$  with true value  $\beta^*$ .

Assumed model m': same interpretable interest parameter  $\beta$ ; misspecified in other ways; notional nuisance parameter  $\alpha$ .

Log-lik for fitted model:  $\ell(\beta, \alpha) = \log m'(y; \beta, \alpha);$  $(\beta, \alpha) \in \mathcal{B} \times \mathcal{A}.$  Maximisation:  $(\hat{\beta}, \hat{\alpha}) \rightarrow_p (\beta_m^0, \alpha_m^0)$  solves

$$\mathbb{E}_m[\nabla_{(\beta,\alpha)}\ell(\beta_m^0,\alpha_m^0)]=0.$$

Misspecification: no value of  $\alpha \in \mathcal{A}$  gives back *m*.

## The inferential target

Our inferential target is  $\beta^*$ , not  $(\beta_m^0, \alpha_m^0)$  to which the classical literature applies.

What structure implies that  $\beta^* = \beta_m^0$  for any *m*?

Ideally would like to be able to check this without knowledge of m or  $\alpha_m^0$ .

#### Parameter *m*-orthogonality

**Definition.** Let  $\nabla^2_{\beta\alpha}\ell(\beta,\alpha)$  denote the cross-partial derivative of the log-likelihood function. The parameter  $\beta$  is said to be *m*-orthogonal to the notional parameter  $\alpha$  if  $\mathbb{E}_m[\nabla^2_{\beta\alpha}\ell(\beta,\alpha)] = 0$ .

**Notation.**  $\mathcal{B} \perp_m \mathcal{A}$ : global *m*-orthogonality;  $\mathcal{B} \perp_m \alpha$  local *m*-orthogonality at  $\alpha$  for any  $\beta$ ;  $\beta \perp_m \mathcal{A}$  and local *m*-orthogonality at  $\beta$  for any  $\alpha$ .

**Geometrically.** The stronger property  $\nabla^2_{\beta\alpha}\ell(\beta,\alpha) = 0$  is an absence of torsion; the true model *m* trivially plays no role.  $\mathbb{E}_m[\nabla^2_{\beta\alpha}\ell(\beta,\alpha)] = 0 \Rightarrow$  any torsion is not systematic when data are generated from *m*. Relevance of parameter *m*-orthogonality

Immediate: 
$$\beta^* = \beta_m^0 \iff \underbrace{\mathbb{E}_m[\nabla_\beta \ell(\beta^*, \alpha_m^0)] = 0}_{C0}.$$

But we don't know  $\alpha_m^0$  or *m*.



C1.1 and C1.2 imply C0 and therefore consistency of  $\hat{\beta}$ . In some classes of models C1.1 & C1.2 can be guaranteed for any *m* through suitable parametrisation.

#### A weaker requirement

Suppose C1.2 fails, i.e.  $\beta^* \not\perp_m \mathcal{A}$ .

Second result:  $(i^{\beta\beta}g_{\beta} + i^{\beta\alpha}g_{\alpha} = 0 \ \forall \alpha \in \mathcal{A}) \Longrightarrow \beta^* = \beta_m^0$ . Scalar case:  $(i_{\alpha\alpha}g_{\beta} + i_{\beta\alpha}g_{\alpha} = 0 \ \forall \alpha \in \mathcal{A}) \Longrightarrow \beta^* = \beta_m^0$ 

where  $i = i(\beta^*, \alpha) = \mathbb{E}_m[-\nabla^2_{(\beta, \alpha)}\ell(\beta^*, \alpha)]$ 

$$\left(\begin{array}{cc}i^{\beta\beta}&i^{\beta\alpha}\\i^{\alpha\beta}&i^{\alpha\alpha}\end{array}\right)=\left(\begin{array}{cc}i_{\beta\beta}&i_{\beta\alpha}\\i_{\alpha\beta}&i_{\alpha\alpha}\end{array}\right)^{-1}$$

.

$$egin{aligned} &g_eta &:= g_eta(eta^*, lpha) &= & \mathbb{E}_{m}[
abla_eta \ell(eta^*, lpha)], \ &g_lpha &:= g_lpha(eta^*, lpha) &= & \mathbb{E}_{m}[
abla_lpha \ell(eta^*, lpha)]. \end{aligned}$$

## Examples

A class of natural examples involve misspecified random effects distributions.

There are also many examples where parameter *m*-orthogonality arises due to a parameter cut: these are relatively easy cases, although the conclusion may not have been obvious without having the structure made clear.

Examples of neither form...

#### Matched comparison and balanced two-group problems

Outcomes  $(Y_{i1}, Y_{i0})_{i=1}^n$  on treated and untreated individuals. Distributions parametrised by treatment effect  $\psi$  and pair-effect  $\gamma_i$  (drop subscript) belong to a transformation model under the action of  $g = g_{\psi} \in G$ .

 $\psi$ -symmetric parametrization: density functions  $f_1$ ,  $f_0$  related by

$$f_U(u;\gamma)du = f_1(gu;g\gamma)d(gu) = f_0(g^{-1}u;g^{-1}\gamma)d(g^{-1}u)$$

for some  $g \in G$ . In other words,  $g^{-1}Y_1 \stackrel{d}{=} U$  and  $gY_0 \stackrel{d}{=} U$ , where the distribution of U is of "standardised form".

For any assumed mixing distribution over  $\gamma$  parametrised by  $\alpha \in A$ , and for any true mixing distribution, conditions C1.1 and C1.2 are satisfied.

### Some less abstract applications

 $(Y_{i1}, Y_{i0})_{i=1}^{n}$  exponentially distributed. Symmetric parametrisation  $\gamma_{i}\psi$  and  $\gamma_{i}/\psi$ . Treat  $\gamma_{i}$  as random with any parametric distribution. Regardless of the assumed and true random effects distribution,  $\hat{\psi} \rightarrow_{p} \psi^{*}$ .

Extends to the symmetric parametrisation of Weibull and gamma, with treatment effect multiplicative on the rate.

In location families the symmetric parametrisation is in the means  $\gamma_i + \psi$ , and  $\gamma_i - \psi$ .

(Other relevant groups? Theory OK for rotation models but this class of examples seems a bit contrived (??))

#### Unbalanced two-group problems

Let  $(Y_{ij1})_{i=1}^{r_{j1}}$  and  $(Y_{ij0})_{i=1}^{r_{j0}}$  be observations within the *j*th stratum for treated and untreated individuals respectively. Unbalanced  $r_{j1} \neq r_{j0}$ .

Reduce by sufficiency within treatment groups and strata:  $S_{j1}$  and  $S_{j0}$ .

#### Stratified two-group Poisson problem with unbalanced strata

Conditionally on  $\gamma_j$ ,  $Y_{ij1}$  and  $Y_{ij0}$  are Poisson distributed counts of rates  $\gamma_j \psi^*$  and  $\gamma_j / \psi^*$ , then  $S_{j1}$  and  $S_{j0}$  are sums of these counts and are Poisson distributed of rates  $r_{j1}\gamma_j\psi^*$  and  $r_{j0}\gamma_j/\psi^*$ .

 $r_{j1}$  and  $r_{j0}$  reflect the number of patients at risk in each group;  $(\gamma_j)_{j=1}^m$  is a stratum-specific nuisance parameter.

Fitted model treats  $(\gamma_j)_{j=1}^m$  as gamma distributed:  $\hat{\psi} \to_p \psi^*$  under any random effects distribution with the same mean.

#### Evans and Didelez (2024) JRSSB discussion paper

Marginal structural model in a 'frugal parametrisation'.

Nuisance parameters enter through the propensity score.

E&D model has a parameter cut, implying parameter *m*-orthogonality when the propensity score is misspecified.

The proof of E&D's main theorem implicitly establishes the remaining condition. See HB discussion of E&D.

# The end