



# **Survey calibration and two-phase designs**

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# Outline

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- What are survey calibration estimators
- Relationship to RRZ efficient estimators
- Calibration and ‘estimated weights’ paradox
- Calibration for straightforward AIPW estimators
- Semiparametric models with small codimension

Underlying question: **where does the information come from in semiparametric models with high codimension**

# Estimating a total

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Population size  $N$ , sample size  $n$ , sampling probabilities  $\pi_i$ , sampling indicators  $R_i$ .

Goal: estimate

$$\sum_{i=1}^N y_i$$

Horvitz–Thompson estimator:

$$\hat{T} = \sum_{R_i=1} \frac{1}{\pi_i} y_i$$

To estimate parameters  $\theta$  replace  $y_i$  with loglikelihood  $\ell_i(\theta)$  or estimating functions  $U_i(\theta)$ .

# Auxiliary information

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HT estimator is inefficient when some population data are available.

Suppose  $x_i$  known for all  $i$ .

Fit  $y_i \sim x_i\beta$  by (probability weighted) least squares to get  $\hat{\beta}$ . Let  $r^2$  be the proportion of variation explained.

$$\hat{T}_{reg} = \sum_{R_i=1} \frac{1}{\pi_i} (y_i - x_i\hat{\beta}) + \sum_{i=1}^N x_i\hat{\beta}$$

ie, HT estimator for sum of residuals plus population sum of fitted values.

# Auxiliary information

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For large  $n$ ,  $N$  and with conditions on moments and sampling

$$\text{var} [\hat{T}_{reg}] = r^2 \text{var} [\hat{T}] + O(N/\sqrt{n}) = (r^2 + O(n^{-1/2})) \text{var} [\hat{T}]$$

and the relative bias of  $\hat{T}_{reg}$  is  $O(1/n)$

The lack of bias does not require any assumptions about the distribution of  $Y|X$

$\hat{\beta}$  is consistent for the population least squares  $\beta$ , for which the mean residual is zero by construction.

# Auxiliary information

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Since  $\hat{\beta}$  is linear in  $y_i$  we can write  $\hat{T}_{reg}$  as

$$\hat{T}_{reg} = \sum_{R_i=1} w_i y_i = \sum_{R_i=1} \frac{g_i}{\pi_i} y_i$$

for some (ugly)  $g_i$ .

$\hat{T}_{reg}$  is an IPW estimator using ‘calibrated’ or ‘tuned’ (French: *calage*) weights.

General calibration problem: for a suitable metric  $d(, )$  find  $g_i$  minimizing  $\sum d(g_i, 1)$  and satisfying the calibration equations

$$\sum_{R_i=1} \frac{g_i}{\pi_i} x_i = \sum_{i=1}^N x_i$$

[Deville et al, JASA 1993; JNK Rao et al, Sankhya 2002]

# Two-phase studies

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Sample  $N$  individuals and measure some variables, then subsample  $n$  individuals with probabilities  $\pi_i$  and measure more individuals

Calibration problem: find  $g_i$  minimizing  $\sum d(g_i, 1)$  and satisfying the calibration equations

$$\sum_{R_i=1} \frac{g_i}{\pi_i} x_i = \sum_{i=1}^N x_i$$

as in single-phase sample.

To estimate parameters  $\theta$ , replace  $y_i$  with  $U_i(\theta)$  and solve

$$\sum_{R_i=1} \frac{g_i}{\pi_i} U_i(\theta) = 0$$

# RRZ estimators

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Class of calibration estimators same as augmented IPW estimators of Robins, Rotnitzky, & Zhao.

Class contains the efficient estimator in the non-parametric phase-1 model (efficient design-based estimator) — the most efficient estimator that is consistent for the same limit as if we had complete data.

Typically not fully efficient if model assumptions are imposed at phase 1 — eg, Cox model assumes infinitely many constraints at phase 1, and efficient two-phase estimator is known (Mark & Katki, 2006; Nan 2004) and is more efficient than calibration estimator.



# Estimated weights

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The fact that

$$\sum_{R_i=1} \frac{1}{\pi_i} U_i(\theta) = 0$$

is strictly less efficient than an estimator based on estimated sampling probabilities  $\hat{\pi}_i$

$$\sum_{R_i=1} \frac{1}{\hat{\pi}_i} U_i(\theta) = 0$$

is widely regarded as a paradox.

But as calibration estimators the gain in efficiency is intuitively clear: it is the same as the gain in efficiency from adjusting for pre-randomization variables in a randomized trial

# Estimated weights

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Issue is not 'estimated weights' vs 'known weights' but 'known totals' vs 'estimated totals' for the auxiliary variables by imposing the calibration equations.

Information is gained to the extent that  $U_i(\theta)$  is predictable from auxiliary variables, and this gain can be large.

# Choice of auxiliaries

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The other heuristic gain from the calibration viewpoint is in choosing predictors for estimating  $\pi$ .

The regression formulation shows that the predictors should have strong linear relationships with  $U_i(\theta)$ .

If  $U_i(\theta)$  is of a form such as

$$z_i w_i (y_i - \mu_i(\theta))$$

then  $z_i$  is approximately uncorrelated with  $U_i$

So, don't use a variable correlated with a phase-2 predictor as a calibration variable, use a variable correlated with the phase-2 score function.

# Survival example

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Kulich & Lin (JASA, 2004) gave an example of a (pretend) two-phase study of Wilm's Tumor

- Phase 1: age at onset, tumor diameter, stage, histology (from local lab), time to relapse
- Phase 2: histology from gold-standard central lab.

Phase 2 is a stratified subsample base on relapse and on local-lab histology report.

[In fact, gold-standard histology available for everyone, so we can compare efficiency to full-cohort analysis] Kulich & Lin (following

# Survival example

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RRZ) used local-lab histology and other phase-1 variables in logistic regression to estimate  $\hat{\pi}$  (in fact, separately for each time point).

Effectively the same as calibration using phase-1 variables as auxiliary variables

Alternative:

- estimate misclassification in local-lab histology using phase two.
- Fit a measurement-error model to phase one using Prentice (1982) first-order approximation.
- Use the individual estimating functions  $U_i^*$  as calibration variables.

$U_i^*$  and  $U_i$  should be more strongly correlated than local-lab histology and  $U_i$ .

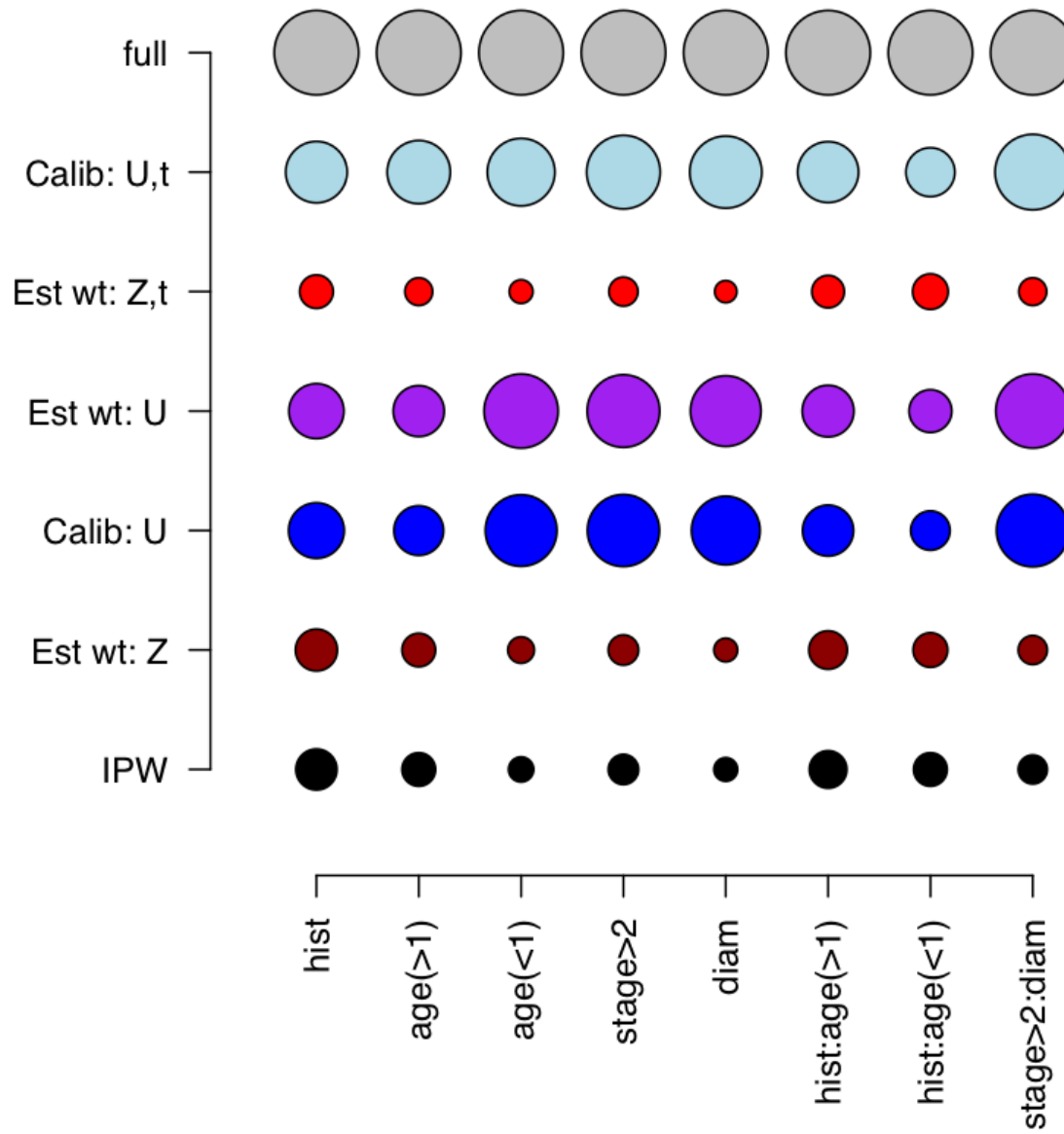
# Results: key

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- **U vs Z**: auxiliary variable is estimating function (U) or raw variable (Z)
- **Calib vs Est wt**: calibration or weights estimated from probabilities from fitted logistic regression.
- **t**: indicates time-dependent weights (3 time periods)
- **IPW**: plain Horvitz-Thompson estimator
- **full**: Full cohort

Area of circle indicated estimated relative efficiency. Rows are estimators, columns are parameters.

# Results



# Gene-environment independence

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Calibration can also be used to fit semiparametric models of finite codimension.

An example is a case-control study of drug-gene interaction

$$\text{logit } P[Y = 1] = \alpha + \beta_g I(\text{gene}) + \beta_d I(\text{drug}) + \gamma I(\text{drug} \cap \text{gene})$$

Often plausible that drug and genetic variant are independent in the population.



# Gene-environment independence

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For  $2 \times 2 \times 2$  table and rare events, case-only estimation exploits the independence [Piegorisch et al, 1994]

		G	
E		0	1
Case	0	a	b
	1	c	d
Control	0	e	f
	1	g	h

case-control estimator:

$$\frac{ad/bc}{eh/gf}$$

case-only estimator:

$$\frac{ad}{bc}$$

# Calibration to independence

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Find  $g_i$  so that

$$\sum_{R_i=1, y=0} \frac{g_i}{\pi_i} (\text{gene} - \mu_g)(\text{drug} - \mu_d) = E[(\text{gene} - \mu_g)(\text{drug} - \mu_d)] = 0$$

This reproduces the case-only estimator exactly. Same approach can be used with more complex models, by calibrating interaction terms to zero.

For discrete data there are only finitely many terms, for continuous data use basis functions such as polynomials or splines.

For rare events calibration to independence is almost fully efficient, implying that no information comes from the structure of the logistic model.

# Calibration to independence

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For non-rare events calibration is slightly more complicated, and is not fully efficient. It captures about half the efficiency gain possible from the NPMLE. (Dai 2007+)

Likelihood-based approaches to this problem exist (?abound): Chatterjee, Carrol, McNeney, Graham.

Calibration is interesting because it is easy and because it tells us about the sources of information.

# Conclusions

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Survey calibration is a useful way to think about RRZ-style estimators: intuition is easier

Calibration is a useful way to construct estimators in models of low co-dimension

Calibration estimators are typically usefully more efficient than the HT estimator (though not always)

Calibration estimators are often less efficient than semiparametric likelihood estimators, but the extra information really does come from the phase-one model assumptions.