The Effective Estimand*

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Abstract

In empirical scientific investigations, investigators often employ statistical estimation procedures to obtain parameter estimates that they will use as a basis to generalize their findings to new settings. Unfortunately, the current theory of statistical estimation has little to say about the validity of this generalization when the estimation procedure is either a) derived from a misspecified model, or b) chosen before the parameterization of the problem has been fixed. In this paper, we address this gap by providing a formal necessary condition for a statistical procedure to be considered useful for a scientific investigation. This condition revolves around a canonical notion of the parameter that an estimation procedure is actually estimating, which we call the effective estimand. Using this idea, we say a procedure is a valid basis for generalization (a necessary condition for scientific usefulness) if and only if its effective estimand is invariant across the contexts to which the investigator hopes to generalize their conclusions.

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Contents

1 Introduction

1.1 Summary

George Box's aphorism that all models are wrong, but some are useful, is all but a mantra in applied statistics, but the notion of the usefulness of a model, or, more broadly, of a statistical procedure has not been defined in any formal sense. The goal of this paper is to define a necessary condition for a statistical procedure to be useful for drawing scientific conclusions. We define this condition in two stages. First, we state a necessary condition for a statistical *parameter* to be useful for a scientific investigation. This first stage, is only novel in its simplicity; our condition is largely in line with qualitative statements made about the theory of statistical modeling (Cox, 1990; Lehmann, 1990), and is subsumed by the more formal requirements suggested in (McCullagh, 2002). Second, we provide a generic definition of the parameter that a statistical procedure, whether it be model-based or purely algorithmic, can be understood to be estimating estimating. We call this quantity the effective estimand, and its definition is the main focus of this paper.

This work is motivated by recent trends in statistical practice that diverge from Fisher's original characterization of estimation problems in as following a Parameterize–Estimate–Analyze sequence in which an investigator specifies superpopulation characteristics of interest, estimates those parameters, and then analyzes the variability of the estimator (Fisher, 1922; Cook, 2007). Instead, modern statistical practice often allows the estimation procedure to dictate the characteristics of interest, with investigators obtaining a dataset, then applying a wide range of procedures drawn from a "reservoir of models" (Lehmann, 1990), which often imply completely different parameter spaces. We call this procedure-first estimation. Importantly, drawing inferences from a misspecified model, even when the investigator intends to follow the classical Fisherian protocol, is a special case of procedure-first estimation. The usefulness of data summaries yielded by procedure-first estimation depends critically on what superpopulation characteristics they target, and whether these characteristics can serve as a valid basis of generalization across the contexts in which the investigator wishes to draw conclusions.

To map statistical procedures to parameters, we invert the standard argument made in the statistical estimation literature: instead of defining a parameter and designing and characterizing an estimator to estimate it, we fix an estimation procedure and ask what parameter the procedure should be understood to be estimating. Formally, our approach follows the plug-in principle in reverse. Instead of designing an estimator by plugging an empirical distribution into an operator that summarizes the superpopulation, here, we say an estimator estimates the quantity recovered by plugging the superpopulation into an operator defined by the estimator. Figure 1 summarizes our approach, with components to be defined in Section 2 and Section 4.

$$
\begin{tikzcd}
 & \text{Context} & \text{Parameter} \\
\text{Superpopulation} & \mathbb{P}_{0,W} \arrow[r, "\Phi_{\hat{\theta}}(\cdot,W)"] \arrow[d, "\Sigma"'] & \bar{\theta}(W) \\
\text{Sample} & \hat{\mathbb{P}}_{Y_W} \arrow[r, "\Phi_{\hat{\theta}}(\cdot,W)"] & \hat{\theta}(Y_W, W) \arrow[u, "\text{“Estimates”}"]
\end{tikzcd}
$$

Figure 1: Relationship between probabilistic objects that define the estimator-estimand relationship when applying an estimator $\hat{\theta}(\cdot)$ to a sample Y_W. $\mathbb{P}_{0,W}$ is the contextual superpopulation, or replication distribution of the sample Y_W. $\hat{\mathbb{P}}_{Y_W}$ is the empirical distribution obtained by drawing a sample Y_W, represented here by applying the sampling operator Σ on the contextual superpopulation $\mathbb{P}_{0,W}$. Computing the estimator $\hat{\theta}(Y_W)$ can be represented as applying a statistical functional $\Phi_{\hat{\theta}}$ on the empirical distribution $\hat{\mathbb{P}}_{Y_W}$; we say that this estimates the effective estimand $\bar{\theta}_W$, or the quantity returned by plugging the contextual superpopulation $\mathbb{P}_{0,W}$ into the estimating functional $\Phi_{\hat{\theta}}$.

1.2 Related work

Thematically, we hope to reconcile the principles that have been advanced in the foundational parametric modeling literature, the pragmatism of the robust estimation literature, and the generality of the nonparametric and semiparametric estimation literatures. McCullagh (2002); Cox (1990); Gelman et al. (2014); Cox & Donnelly (2011) are examples of the first literature; Huber & Ronchetti (2009); Huber (1967, 1964) are primary examples of the second, and Van Der Vaart & Wellner (2000); van der Vaart & Wellner (1996); van der Laan & Rubin (2006) are examples of the third. In addition, two lines of recent research are relevant to our discussion, both of which have referenced a quantity mathematically identical to a special case of the effective estimand, but have used it to make a different sort of argument.

The first line of work, presented in Buja et al. (2016), treats the breakdown of "model-trusting" linear regression modeling assumptions under misspecification, with a focus on quantifying the additional variability in estimation that results from model misspecification. Several notions in these two papers overlap: the representation of parameters as statistical functionals, the notion of the target of a misspecified estimator, and the notion that such a target should, ideally, exhibit a kind of invariance to statistics that are conditioned upon, but that such invariance is often lost under misspecification. In this paper, we consider a larger set of problems where such reasoning can be applied, and, rather than focusing on the variability of a particular estimator, build a framework for understanding whether an estimator can serve as a valid basis for generalization.

The second line of work, presented in Spokoiny (2012), concerns the so-called pseudo-true parameter in the context of maximum likelihood estimation. In the case of maximum likelihood estimators, it happens that the pseudo-true parameter and the effective estimand coincide, so many of Spokoiny's results are directly applicable here, but in this paper, our focus is again on the role that an estimator might play in a scientific argument, and less about the operating characteristics of a fixed estimation procedure. In particular, Spokoiny (2012) begins from the assumption that the pseudo-true parameter is the target of an estimator; here, we provide a justification for this statement.

More generally, this paper delves into foundational relationships between statistics and the philosophy of science. In particular, the notion that we ought to estimate a parameter that remains invariant across contexts has appeared in several placed before: it is one of the requirements encoded by the functor representation of models in McCullagh (2002); it corresponds to the notion of transportability presented in Pearl & Bareinboim (2012); even dating back to Hume's work on the problem of induction, this corresponds to the "principle of uniformity of nature", a necessary assumption for drawing conclusions from inductive reasoning (Hume & Beauchamp, 2000).

2 Preliminaries

2.1 Data Generation, Context, and Estimators

Consider a generic statistical problem with an outcome Y_W observed in a context denoted by W. The context W includes all observable characteristics of the study that the investigator wishes to condition on. This includes design parameters like sample size, or data that are treated as inputs in prediction problems, like covariates. In the most general terms, the investigator's goal is to compute a summary of observed data Y_W that can be used to characterize distinct data $Y_{W'}$ that could be observed across a relevant set of contexts $W' \in \mathcal{W}$. $\mathcal{W}$ is the desired range of situations over which the investigator would like their conclusions to be valid, so we call it the range of validity.

The statistical approach formalizes this problem in terms of probability. Let $(\Omega, \mathcal{F}, \mathcal{M})$ be a probability space, and let Y_W be a random variable that maps the sample space Ω to an outcome space $\mathcal{Y}_W$. Let $\mathbb{P}_{0,W}$ be the distribution that the random variable $Y_W(\omega)$ induces on $\mathcal{Y}_W$. We call $\mathbb{P}_{0,W}$ the contextual superpopulation, because it describes the superpopulation of replications of $Y_W(\omega)$ that could be obtained in the specific context W by drawing new values ω from the sample space Ω. In our formalism, the investigator's goal is to characterize the set of contextual superpopulations $\{\mathbb{P}_{0,W'} : W' \in \mathcal{W}\}$ by computing a function $\hat{\theta}(Y_W, W)$ of the observed data, which we call the estimator. We illustrate the relationship between samples, contexts, and contextual superpopulations in Figure 2. We give some examples of how our formalism maps onto common problems below.

Example 1 (Response Surface)**.** In problems commonly analyzed with linear regression, the context is the sample size and covariates, so $W \in \{(N, X) : N \in \mathbb{N}, X \in \mathbb{R}^{N\times p}\}$, where p is the number of covariates, and the outcome Y_W lives in $\mathcal{Y}_W = \mathbb{R}^N$. Depending on the investigator's application, they may define the range of validity to encompass all sample sizes N and all possible covariate sets X, or, if the linear behavior is believed to only apply in certain contexts, restrict X to a particular region of the covariate space. In this case, for a given context $W = (N, X)$,

	Contexts $\longrightarrow$			
Superpopulations	$\mathbb{P}_{0,W}$	$\mathbb{P}_{0,W_1}$	$\mathbb{P}_{0,W_2}$	$\cdots$
Replicated Samples $\downarrow$	$Y_W(\omega)$	$Y_{W_1}(\omega)$	$Y_{W_2}(\omega)$	$\cdots$
	$Y_W(\omega^{(1)})$	$\ddots$	$\vdots$	$\cdots$
	$Y_W(\omega^{(2)})$	$\cdots$	$Y_{W_2}(\omega^{(2)})$	$\cdots$
	$\vdots$	$\vdots$	$\vdots$	

Figure 2: Replications and contextual superpopulations. As discussed in Section 3, generalizations restricted to a particular column, which comprise statements about replications obtained from the same context, do not require parameterization. Generalizations across columns, which comprise statements about data obtained in different contexts, whether within or between replications, require parameterization to express assumptions about properties that substantively different superpopulation distributions have in common.

the contextual superpopulation $\mathbb{P}_{0,W}$ is a probability distribution on $\mathcal{Y}_W = \mathbb{R}^N$. In this case, an investigator might compute the linear regression estimator $\hat{\theta}(Y_W, W) = \left(X^\top X\right)^{-1} X^\top Y_W$

Example 2 (Spatiotemporal Events)**.** In problems commonly analyzed as point processes, where outcomes are discrete events in continuous space-time, the context is the observation window defined on a spatial area A and a time window T, so $W \in \{(A,T) : A \in \mathcal{L}(\mathbb{R}^2), T \in \mathcal{L}(\mathbb{R}^+)\}$, where $\mathcal{L}(\cdot)$ denotes Lebesgue measure. Here, the outcome Y_W can be represented as a set of points in $A \times T$, so $\mathcal{Y}_W = \{(N,Y) : N \in \mathbb{N}, Y \in (A \times T)^N\}$. In this case, for a given context $W = (A,T)$, the contextual superpopulation $\mathbb{P}_{0,W}$ is the distribution of a point process restricted on a state space $A \times T$, which is a probability distribution on the set of locally finite counting measures on $A \times T$. In this case, an investigator might compute a rate estimator $\hat{\theta}(Y_W, W) = \frac{\sum Y_W}{|A||T|}$.

Example 3 (Correlated Samples)**.** In problems that are commonly analyzed with Gaussian processes, where the outcome Y_W is a set of measurements associated with locations in a two-dimensional space, the context of the observed data is the number and spatial indices of the observed outcomes, so $W \in \{(N,L) : N \in \mathbb{N}, L \in \mathbb{R}^{N \times 2}\}$. In this case, the investigator may be interested in generalizing from $Y_W(\omega)$ to other contexts $Y_{W'}(\omega)$ from the same realization of an underlying stochastic process, indicated by the shared sample element ω. To represent this, the contexts in the range of validity include the values $Y_{W'}(\omega)$ that are conditioned upon, so $W' \in \{(N', L', Y_W) : N' \in \mathbb{N}, L' \in \mathbb{R}^{N' \times 2}, Y_W \in \mathcal{Y}_W\}$.

Example 4 (Restricted Range of Validity)**.** Most commonly, the range of validity $\mathcal{W}$ is defined as the set of all theoretically obtainable contexts, but it is also common for the range to be restricted. For example, in using linear models to approximate response surfaces in industrial experiments, Box & Draper (1959) specify a feasible operating region that restricts the range of validity to a compact subset of the covariate space $\mathcal{X}$, so $\mathcal{W} = \{(N, X) : X \in R \subset \mathcal{X}^N\}$. For highly regular prediction problems where the data being predicted are always observed in the same context, the range of validity can be a singleton, with $\mathcal{W} = \{W\}$.

2.2 Parameterization

In general, the distributions $\{\mathbb{P}_{0,W'} : W' \in \mathcal{W}\}$ are not assumed to be identical; in many cases, as when the range of validity $\mathcal{W}$ includes the sample size of the observed data Y_W, these superpopulations are not even defined on the same support. To structure the problem of characterizing the set of distributions $\{\mathbb{P}_{0,W'} : W' \in \mathcal{W}\}$, properties that are shared between these distributions are encoded as parameters, where parameters are defined in the sens of the nonparametric, semiparametric, and robust estimation literatures (Huber & Ronchetti, 2009; Bickel et al., 1998; van der Laan & Rubin, 2006).

A parameter θ in a parameter space Θ is the output of a statistical functional $\Phi(\cdot, W)$ that maps probability distributions defined on a particular sample space $\mathcal{Y}_W$ and their contexts W to points in Θ, so that $\theta = \Phi(\mathbb{P}_{0,W}, W)$. This construction is distinct from the model-based specification of parameters, which requires the specification of a model family. In this section, we highlight the distinction between functional and model-based parameters.

This functional view of parameters is a generalization of the model-based view of parameters. In the model-based view, the investigator chooses a set of distributional properties that the parameter θ encodes and defines a model family by allowing the parameter θ to vary within a parameter space Θ. Formally, a parametric model family is a set of probability distributions $\mathcal{P}_{\Theta,\mathcal{W}} \equiv \{\mathbb{P}_{\theta,W} : \theta \in \Theta, W \in \mathcal{W}\}$, indexed by both a parameter θ in a parameter space Θ and a context W in a set of relevant contexts $\mathcal{W}$. This view is restrictive because θ is an index within a model family, and is thus only well-defined within that model family.

On the other hand, for every parametric model family $\mathcal{P}_{\Theta,\mathcal{W}}$, one can define a functional $\Phi(\cdot, W)$ that returns the index of any model in that family, so that $\Phi(\mathbb{P}_{\theta,W}, W) = \theta$, for all $W \in \mathcal{W}$; meanwhile, this specification also returns a well-defined quantity for probability distributions $\mathbb{P}_W$ that are not contained in the model family $\mathcal{P}_{\Theta,\mathcal{W}}$.

This generality comes at a cost. Because the functional view does not reference a model-based hypothesis, the parametric summary $\Phi(\cdot, W)$ is not in general sufficient for specifying the contextual superpopulation $\mathbb{P}_{0,W}$.

Remark 1. With the functional view of parameters, it is not necessary to make a distinction between parameters and other superpopulation quantities of interest; this distinction is only necessary when using the model-based construction of parameters where a full specification of a model may be better expressed in terms of a parameter vector θ, but the quantity of interest $Q(\theta)$ is better expressed as a function of the family index. In the functional view, because the quantity of interest is itself a parameter, in that the composition of the function $Q(\cdot)$ and the parameter-defining functional $\Phi(\cdot, W)$ defines a new functional $\Phi'(\cdot, W)$, so that $Q(\theta) = Q(\Phi(\mathbb{P}, W)) = \Phi'(\mathbb{P}, W) = \theta'$.

Example 5 (Functional and Model-Based Parameters)**.** Suppose we have a sample Y_W observed in a context $W = (N, X)$, where N is the size of the sample, and X is a covariate vector in $\mathbb{R}^N$. In this case, suppose that the investigator's range of validity is $\mathcal{W} = \{(N, X) : N \in \mathbb{N}, X \in \mathbb{R}^N\}$, meaning that the investigator hopes to be able to generalize to all sample sizes with all possible scalar covariates. Suppose the contextual superpopulation $\mathbb{P}_{0,W}$ has the following definition:

$$Y_i \sim \text{Normal}(\theta_0 X_i, 1) \qquad\qquad \text{for each} i \in 1, \cdots, N. \tag{1}$$

so that, in vector notation, $\mathbb{P}_{0,W} = \text{MVNormal}(\theta_0 X, I_N)$.

A well-specified model of this generating process could parameterize the coefficient of X in the conditional mean of Y, and define a family

$$\mathcal{P}_{\Theta,W} = \{\text{MVNormal}(\theta X, I_N) : \theta \in \Theta\} \tag{2}$$

where $\Theta = \mathbb{R}$. Here, θ is a model-based parameter.

θ can also be represented as a the output of a functional $\Phi(\cdot, W)$ that extracts the linear dependence of the conditional mean of Y_W on X:

$$\theta = \Phi(\mathbb{P}_{0,W}, W) = \arg\min_{\theta \in \Theta} E_{\mathbb{P}_{0,W}} \left[\sum_i (Y_i - X_i \theta)^2 \right]. \tag{3}$$

Under the generating process defined in Equation 1, $\theta(\mathbb{P}_{0,W}) = \theta_0$.

The investigator could also specify a functional $\Phi(\cdot, W)$ using the mean functional, even though this summary is not sufficient to characterize the generating process $\mathbb{P}_{0,W}$:

$$\theta = \Phi(\mathbb{P}_{0,W}, W) = \arg\min_{\theta \in \Theta} E_{\mathbb{P}_{0,W}} \left[\sum_i (Y_i - \theta)^2 \right]. \tag{4}$$

For each $W \in \mathcal{W}$, $\Phi(\mathbb{P}_{0,W}, W)$ is well-defined, but it gives a different summary for each W with different covariate values X. Under the generating process defined in Equation 1, $\Phi(\mathbb{P}_{0,W}, W) = N^{-1} \sum_i \theta_0 X_i$.

3 Basis of Generalization

Recall that the investigator's goal is to draw conclusions from the observed sample Y_W and generalize these conclusions to data generated in a set of contexts given by the investigator's range of validity $\mathcal{W}$. In addition to information contained in the sample Y_W, generalization requires assumptions. If an investigator's range of validity contains only the observed context, so that $\mathcal{W} = \{W\}$, very few assumptions are necessary to generalize to new replications $Y_W(\omega)$, beyond the assumption that contextual superpopulation $\mathbb{P}_{0,W}$ remains the same. On the other hand, if this investigator wishes to generalize across distinct contexts, so that $\mathcal{W}$ is not a singleton, the investigator must assume that certain properties are shared among the distinct contextual superpopulations in the range of validity $\{\mathbb{P}_{0,W'} : W' \in \mathcal{W}\}$. We call such a property, which can be encoded as a functional parameter, a basis of generalization.

Definition 1 (Parameter Basis of Generalization)**.** A parameter $\Phi(\cdot, W)$ is a valid basis of generalization for a range of validity $\mathcal{W}$ if and only if $\Phi(\mathbb{P}_{0,W}, W) = \Phi(\mathbb{P}_{0,W'}, W')$ for all $W' \in \mathcal{W}$.

Requirements that a parameter satisfy Definition 1 are common in the applied statistics literature when advice is being given about the construction of a statistical model (McCullagh, 2002; Cox, 1990; Gelman, 2004; Cox & Donnelly, 2011). The assumption, following Fisher's formulation, is that an empirical scientific question can be neatly broken into a parameterization stage, where the investigator chooses an appropriate parametric summary $\theta = \Phi(\mathbb{P}_{0,W}, W)$ of the data-generating process, and an estimation and inference stage, where an estimator $\hat{\theta}(Y_W, W)$ is constructed to approximate this parameter θ. The scientific validity of this procedure only depends on the parameterization; once this is fixed, the statistician can focus on estimating this parameter as efficiently as possible.

Unfortunately, specifying conditions on parameterization is insufficient to appropriately guide statistical practice. Defining a parametric summary $\Phi(\mathbb{P}_{0,W}, W)$ that satisfies Definition 1 requires an intimate knowledge of the data-generating process and the relationships between contextual superpopulations. Particularly as data-intensive scientific investigations become more complex, it is uncommon for such knowledge to be available at the outset. More often, the investigator has a partial hypothesis about the data-generating process, selects a parametric summary $\Phi(\mathbb{P}_{0,W}, W)$ that would satisfy Definition 1 if the simplified model were well-specified, and applies an estimator derived from this model to estimate this parameter. In other cases, investigators choose a standard data analysis procedure first, and allow the range of this data summary to dictate their parameterization. In these realistic scenarios, an estimation procedure is constructed without reference to the true data-generating process, so the scientific relevance of the resulting estimate cannot be determined using Definition 1.

We aim to close this theoretical gap by formalizing the idea that when an investigator chooses a data analysis procedure, they are implicitly choosing a parameterization; namely, the quantity being targeted by the estimator is the the parameter that will be used as a basis of generalization. In particular, we define a canonical map of an estimator to the functional parameter that it estimates. We denote this parameter by $\Phi_{\hat{\theta}}(\cdot, W)$, and call the effective estimand. We will spend the remainder of the paper defining $\Phi_{\hat{\theta}}(\cdot, W)$.

With the notion of the effective estimand of a procedure, we can extend Definition 1 so that it classifies estimation procedures, rather than parameterizations, according to their scientific usefulness.

Definition 2 (Estimator Basis of Generalization)**.** An estimation procedure $\hat{\theta}(Y_W, W)$ is a valid basis of generalization for a range of validity $\mathcal{W}$ if and only if its target of estimation $\Phi_{\hat{\theta}}(\mathbb{P}_{0,W}, W)$ is a valid basis of generalization.

Remark 2. An estimator's being a valid basis of generalization is only a necessary condition for scientific usefulness. If the basis of generalization is trivial, so that it does not help in predicting a quantity of interest, it may still be useless.

Remark 3. The strict invariance requirement specified in Definition 1 and Definition 2 may be stronger than is necessary in a particular statistical investigation, for which a weaker form of the criterion could be applied. For example, if variation in the target of estimation $\Phi_{\hat{\theta}}(\mathbb{P}_{0,W}, W)$ is nonzero but small compared to sampling variability, then the investigator could determine that Definition 2 is approximately satisfied, particularly if the variation in the effective estimand $\Phi_{\hat{\theta}}(\mathbb{P}_{0,W}, W)$ is designed to trade off with variance.

Remark 4. In cases where a parameter $\Phi(\mathbb{P}_{0,W}, W)$ can be well-specified in the standard Fisherian workflow, checking Definition 2 can still be useful for identifying systematic components of generalization error.

4 The Effective Estimand

4.1 Requirements of a Canonical Estimand

To complete the statement of Definition 2, we require a canonical definition of the parametric summary being estimated by a given estimation procedure $\hat{\theta}(Y_W, W)$. This inverts the usual argument made in the statistical estimation literature, which fixes a parameter of interest is and derive an estimation procedure to estimate it. In this section, we invert this argument; we fix an estimation procedure and derive the parameter being estimated.

In principle, this inversion is an ill-posed problem. The target of estimation in any statistical problem is arbitrary; any function of the observed data can be framed as an arbitrarily poor estimate of any arbitrary quantity. Nonetheless, it is useful to define a canonical target of estimation for an estimation procedure. Here, we specify several requirements for such a canonical estimation target:

(R1) The estimand should not be defined in terms of a model family.

(R2) In cases where the estimation procedure is derived from a well-specified model, the target of estimation should reduce to the true parameter.

(R3) The target should be a function of the specific context W.

(R4) The relationship between estimator and estimand should be invariant to injective transformations.

Requirement (R1) ensures that the target of estimation is well-defined even when the investigator is not able to specify a model family that contains the true superpopulation $\mathbb{P}_{0,W}$. Requirement (R2) ensures that the target of estimation is a generalization of a model parameter in the familiar parametric setting. Requirement (R3) makes the target a useful tool for determining whether the procedure targets a quantity that can be used to generalize between different finite sample contexts W. Finally, requirement (R4) ensures that the target remains coherent between equivalent representations of the same estimation problem.

Requirement (R2) motivates a particular approach to defining a canonical estimand, namely by inverting criteria that are commonly applied to estimation procedures; put simply, whatever an estimator estimates, it should estimate it well. Requirements (R3) and (R4) eliminate two obvious criteria that could be inverted, namely large-sample consistency and unbiasedness.

To invert large-sample consistency, we would define the canonical estimand as the value $\theta \in \Theta$ for which the estimation procedure is large-sample consistent, equating the estimand to the large-sample limit of the estimator. This definition violates (R3) because the limit $\lim_{D(W)\to\infty} \hat{\theta}(Y_W)$ is defined over a particular dimension $D(W)$ of the context W, for example sample size or total exposure. Based on this criterion, estimators calculated from samples that differ in the dimension $D(W)$ along which the limit is taken are indistinguishable. In Section 7.5 we highlight a case where this can mask difficulties in the analysis of dependent network datasets of varying size.

To invert unbiasedness, we would define the canonical estimand as the expectation of the estimator $E_{\mathbb{P}_{0,W}} \hat{\theta}(Y_W)$, or the value $\theta \in \Theta$ for which the estimator is unbiased. While this summary does satisfy (R3), it violates (R4) because nonlinear transformations of the estimator result in non-equivalent definitions of the target of estimation. In addition to incoherence within a given problem, this definition has the opposite problem as the large sample limit: when estimation procedures exhibit finite-sample bias but are asymptotically unbiased, the expectation introduces spurious variation in the target of estimation as a function of the sample size.

4.2 Empirical Distributions

In the discussion that follows, it will be useful to consider an observed sample $Y_W(\omega)$ and a superpopulation $\mathbb{P}_{0,W}$ as elements in a common space of probability distributions. We can represent an observed sample in this way using its empirical distribution. See Van Der Vaart & Wellner (2000) for more extensive discussion of empirical distributions and empirical processes.

Definition 3. The empirical distribution $\hat{\mathbb{P}}^M_{Y_W}$ of a set of replicated samples $\{Y_W(\omega^{(i)}) : i \in 1, \cdots, M\}$ is a random probability measure on the outcome space $\mathcal{Y}_W$ given by $\frac{1}{M}\sum_{m=1}^{M} \delta_{Y_W(\omega^{(i)})}$.

In most applied cases, there is only one observed replication of a given sample, so $M = 1$, and we write the empirical distribution as $\hat{\mathbb{P}}_{Y_W}$. In this case $\hat{\mathbb{P}}_{Y_W}$ is a point mass at the observed value $Y_W(\omega)$ in $\mathcal{Y}_W$. We denote by Σ the sampling operator that maps $\mathbb{P}_{0,W}$ to $\hat{\mathbb{P}}_{Y_W}$ as Σ, so that $\hat{\mathbb{P}}_{Y_W} = \Sigma\mathbb{P}_{0,W}$.

Theoretical replications of the sample Y_W are a useful tool for connecting the observed sample $Y_W(\omega)$ to the contextual superpopulation $\mathbb{P}_{0,W}$ by a limiting argument. In particular, the Glivenko-Cantelli theorem and its extensions establish that expectations of random functions taken with respect to a sequence of empirical distributions converge to the expectation of the function taken with respect to the superpopulation distribution.

Definition 4 ($\mathbb{P}$–Glivenko-Cantelli Class)**.** A set of random functions $\mathcal{F} = \{f(x) : X \sim \mathbb{P}\}$ is in the $\mathbb{P}$–Glivenko-Cantelli class iff

$$\|E_{\hat{\mathbb{P}}^M} f - E_{\mathbb{P}} f\|_\infty \to 0. \tag{5}$$

This uniform convergence property is important for the theory of estimation because it implies that functionals, such as extrema, of the random function f also converge to the result of the functional evaluated on the superpopulation expectation of f. In particular, the famous Glivenko-Cantelli theorem establishes that the class of indicator functions defined on half-intervals is in the Glivenko-Cantelli class, so the empirical cumulative distribution function converges uniformly to the superpopulation cumulative distribution function.

Theorem 1 (Glivenko-Cantelli Theorem)**.** *Let $Y_1, \cdots, Y_n$ be a set of independent and identically distributed random variables distributed according to the cumulative distribution function F. Let F_n be the corresponding empirical cumulative distribution function*

$$F_n(y) = \sum_i \mathbf{1}_{Y_i \leq y}. \tag{6}$$

Then,

$$\|F_n - F\|_\infty \to 0 \text{ almost surely.} \tag{7}$$

We will make use of uniform convergence of empirical expectations of random functions to their superpopulation expectations in Section 4.3.

4.3 Derivation of the Effective Estimand

In this section, we define the effective estimand, which satisfies criteria ($R2$)–($R4$) for a large class of estimation procedures. The estimand is defined by plug-in: we rewrite the estimator $\hat{\theta}(Y_W)$ as a functional $\Phi_{\hat{\theta}}$, defined below, that operates on the empirical distribution of the data $\hat{\mathbb{P}}_{Y_W}$. We then apply this functional $\Phi_{\hat{\theta}}$ on the contextual superpopulation distribution $\mathbb{P}_{0,W}$ to define the parameter that the estimator is effectively estimating. Figure 1 summarizes this approach. To ensure that $\Phi_{\hat{\theta}}(\mathbb{P}_{0,W}, W)$ defines a statistically meaningful summary of $\mathbb{P}_{0,W}$, we include a limiting argument that shows that the operation of plugging in $\mathbb{P}_{0,W}$ into $\Phi_{\hat{\theta}}$ mirrors a particular hypothetical statistical operation performed on an infinitely large set of replications of the sample $Y_W(\omega)$.

The effective estimand is well-defined for a class of estimators $\hat{\theta}(Y_W, W)$ with following properties. The first two properties are fundamental and define how the estimator incorporates information from replications of the sample Y_W; the final two properties are technical.

(A1) $\mathcal{F}(\Theta)$ the space of functions defined on the parameter space Θ. Then $\hat{\theta}(Y_W, W)$ can be written as a two-part procedure:

$$\hat{\theta}(Y_W, W) = g\left(f(Y_W, \theta)\right), \tag{8}$$

where $f(\cdot, \theta) : \mathcal{Y}_W \mapsto \mathcal{F}(\Theta)$, so that for a given realization of the sample $Y_W(\omega)$, $f(Y_W(\omega), \theta) = f_{Y_W}(\theta)$ is a function on the parameter space Θ, and $g : \mathcal{F}(\Theta) \mapsto \Theta$ is a summarizing functional that maps a realized function $f(Y_W(\omega), \theta)$ into the parameter space. We call a realization $f(Y_W(\omega), \theta)$ a criterion function.

(A2) Let $Y_W^M = \{Y_W(\omega^{(m)}) : m = 1, \cdots, M\}$ be a set of m independent replications of a sample Y_W. The estimation procedure is extensible such that

$$f(Y_W, \theta) = f_0(\theta, W) + f_\omega(Y_W, \theta, W) \tag{9}$$

and

$$\hat{\theta}(Y_W^M, W) = g\left(f_0(\theta, W) + \sum_m f_\omega\left(Y_W(\omega^{(m)}), \theta, W\right)\right). \tag{10}$$

(A3) The family of functions $\{f(Y_W, \theta) : \theta \in \Theta\}$ is in the Glivenko-Cantelli class for the contextual superpopulations $\{\mathbb{P}_{0,W} : W \in \mathcal{W}\}$.

Table 1: Components of estimator functionals $\Phi_{\hat{\theta}}(\mathbb{P}, W) = g(E_{\mathbb{P}} f(Y_W, \theta))$.

	Method of Moments	Maximum Likelihood	Bayes Rule
$\hat{\theta}(Y_W)$	$M(Y_W)$	$\arg\max_{\Theta} \log \mathbb{P}_{\theta,W}(Y_W)$	$\arg\min_{\Theta} \int_{\Theta} L(\theta,\theta')\pi(\theta \mid Y_W)d\theta'$
$f(Y_W, \theta)$	$M(Y_W) - \theta$	$\log \mathbb{P}_{\theta,W}(Y_W)$	$\log \mathbb{P}_{\theta,W}(Y_W) + \log \pi(\theta)$
$g(h(\theta))$	$\text{root}_{\Theta} h(\theta)$	$\arg\max_{\Theta} h(\theta)$	$\arg\min_{\Theta} \int_{\Theta} L(\theta,\theta') \exp(h(\theta'))d\theta'$

$M(Y_W)$ is a moment vector of Y_W; $L(\theta, \theta')$ is a loss function giving a discrepancy between θ and θ'.

(A4) There is a continuous mapping theorem for $g(f_{Y_W}(\theta))$ in the infinity norm on the set of criterion functions $\{f(Y_W, \theta) : \theta \in \Theta\}$ for each $Y_W \in \mathcal{Y}_W$.

Requirement (A2) states that the estimator must be representable so that independent identical replications of the sample enter into the procedure's criterion function additively and symmetrically. We give several examples of estimators that satisfy this criterion and their corresponding decompositions in Table 1. Requirements (A3) and (A4) are necessary for the limiting argument we make below that justifies our interpretation of the effective estimand.

Finally, for each estimator $\hat{\theta}(Y_W)$ that satisfies *(A2)*, define the functional $\Phi_{\hat{\theta}}(\mathbb{P}, W)$ to be an operator that maps probability distributions on $\mathcal{Y}_W$ to the parameter space Θ as follows:

$$\Phi_{\hat{\theta}}(\mathbb{P}, W) = g\left(E_{\mathbb{P}} f(Y_W, \theta)\right). \tag{11}$$

Note that the estimator as evaluated on a single sample can be rewritten $\hat{\theta}(Y_W) = \Phi_{\hat{\theta}}(\hat{\mathbb{P}}_{Y_W}, W)$, where $E_{\hat{\mathbb{P}}_{Y_W}}$ is an expectation taken over a point mass at the observed sample Y_W. We define the effective estimand as the quantity obtained by substituting the contextual superpopulation $\mathbb{P}_{0,W}$ for $\hat{\mathbb{P}}_{Y_W}$ in this expression.

Definition 5. Let $\hat{\theta}(Y_W)$ be an estimation procedure that satisfies (A1)–(A4). Then the effective estimand $\bar{\theta}_W$ is defined as a statistical functional evaluated on the contextual superpopulation $\mathbb{P}_{0,W}$:

$$\bar{\theta}_W = \Phi_{\hat{\theta}}(\mathbb{P}_{0,W}, W) = g\left(E_{\mathbb{P}_{0,W}} f(Y_W, \theta)\right). \tag{12}$$

Because the estimator satisfies (A2), the effective estimand can be understood as the limit of an estimation procedure as it is applied to an increasingly large set of independent identical replications of the sample $Y_W^M = \{Y_W(\omega^{(m)}) : m = 1, \cdots, M\}$, with each sample reweighted so that their total influence is equivalent to the influence of a single sample $Y_W(\omega)$. We call this asymptotic frame the superpopulation plug-in asymptotic, because the limit parallels the convergence of empirical distributions to the superpopulation from which they were drawn.

Theorem 2. *Let $\hat{\theta}(Y_W)$ be an estimation procedure that satisfies (A1)–(A4). Then the effective* *estimand is equivalent to the limit*

$$\bar{\theta}_W = \lim_{M\to\infty} g\left(\frac{1}{M}\sum_m f\left(Y_W(\omega^{(m)}),\theta\right)\right). \tag{13}$$

or equivalently,

$$\Phi_{\hat{\theta}}(\hat{\mathbb{P}}_{0,W}, W) = \lim_{M\to\infty} \Phi_{\hat{\theta}}(\hat{\mathbb{P}}^M_{Y_W}, W) \tag{14}$$

Proof. For each M, the following three representations of an estimator computed from a weighted set of M replicated samples are equivalent

$$g\left(\frac{1}{M}\sum_m f\left(Y_W(\omega^{(m)}),\theta\right)\right) = g\left(E_{\hat{\mathbb{P}}^M_{Y_W}} f(Y_W,\theta)\right) = \Phi_{\hat{\theta}}(\hat{\mathbb{P}}_{Y^M_W}, W). \tag{15}$$

By (A3) f is in a $\mathbb{P}_{0,W}$–Glivenko-Cantelli class, so by definition $E_{\hat{\mathbb{P}}^M_{Y_W}} f(Y_W,\theta) \to E_{\mathbb{P}_{0,W}} f(Y_W,\theta)$ uniformly. By (A4), g preserves the limit, so $g\left(E_{\hat{\mathbb{P}}^M_{Y_W}} f(Y_W,\theta)\right) \to g\left(E_{\mathbb{P}_{0,W}} f(Y_W,\theta)\right) = \bar{\theta}_W$, or equivalently, $\Phi_{\hat{\theta}}(\hat{\mathbb{P}}^M_{Y_W}, W) \to \Phi_{\hat{\theta}}(\mathbb{P}_{0,W}, W)$. □

Remark 5. The purpose of this theorem is to connect the effective estimand to a hypothetical statistical operation, not to characterize the variability of an estimator $\hat{\theta}(Y_W)$ in terms of its effective estimand $\bar{\theta}_W$. Such arguments are available, and we will quote a powerful result of this type in the context of maximum likelihood estimation in Section 6.

Remark 6. Although not mentioned in the proof, (A2) establishes the scale on which the contextual superpopulation $\mathbb{P}_{0,W}$ is plugged in in place of the empirical distribution $\hat{\mathbb{P}}_{Y_W}$. This allows us to interpret the contextual superpopulation as the limit of a large, down-weighted sample, ensuring that $\Phi_{\hat{\theta}}(\mathbb{P}_{0,W}, W)$ corresponds to the output of a hypothetical statistical operation. In the case of maximum likelihood estimation, for example, this implies that $\Phi_{\hat{\theta}}(\mathbb{P}, W)$ takes an expectation of the log-likelihood $E_{\mathbb{P}} \log \mathbb{P}_{\theta,W}$ instead of, for instance, the expectation of the likelihood itself $E_{\mathbb{P}}\mathbb{P}_{\theta,W}$. We discuss this distinction in Example 6 at the end of this section.

The effective estimand satisfies requirements (R1)–(R4). Only (R2) requires detailed explanation; we will treat this below. (R1) is satisfied trivially because the definition of $\bar{\theta}_W$ makes no reference to a model family. Likewise, (R3) is satisfied trivially because $\bar{\theta}_W$ is defined as a functional of the contextual superpopulation $\mathbb{P}_{0,W}$. (R4) is satisfied because one-to-one transformations of the parameter $T : \Theta \mapsto \Gamma$ can be composed with the functional $\Phi_{\hat{\theta}}$ to form a new functional $\Phi'_\Gamma = T \circ \Phi_{\hat{\theta}}$ that transforms both the estimator $T(\Phi_{\hat{\theta}}(\hat{\mathbb{P}}_{Y_W}, W)) = \Phi'_\Gamma(\hat{\mathbb{P}}_{Y_W}, W)$ and the estimand $T(\Phi_{\hat{\theta}}(\mathbb{P}_{0,W}, W)) = \Phi'_\Gamma(\mathbb{P}_{0,W}, W)$.

(R2) is satisfied because the effective estimand is a direct inversion of a generalized notion of Fisher consistency (Fisher, 1922). An estimator is Fisher consistent with respect to a particular model

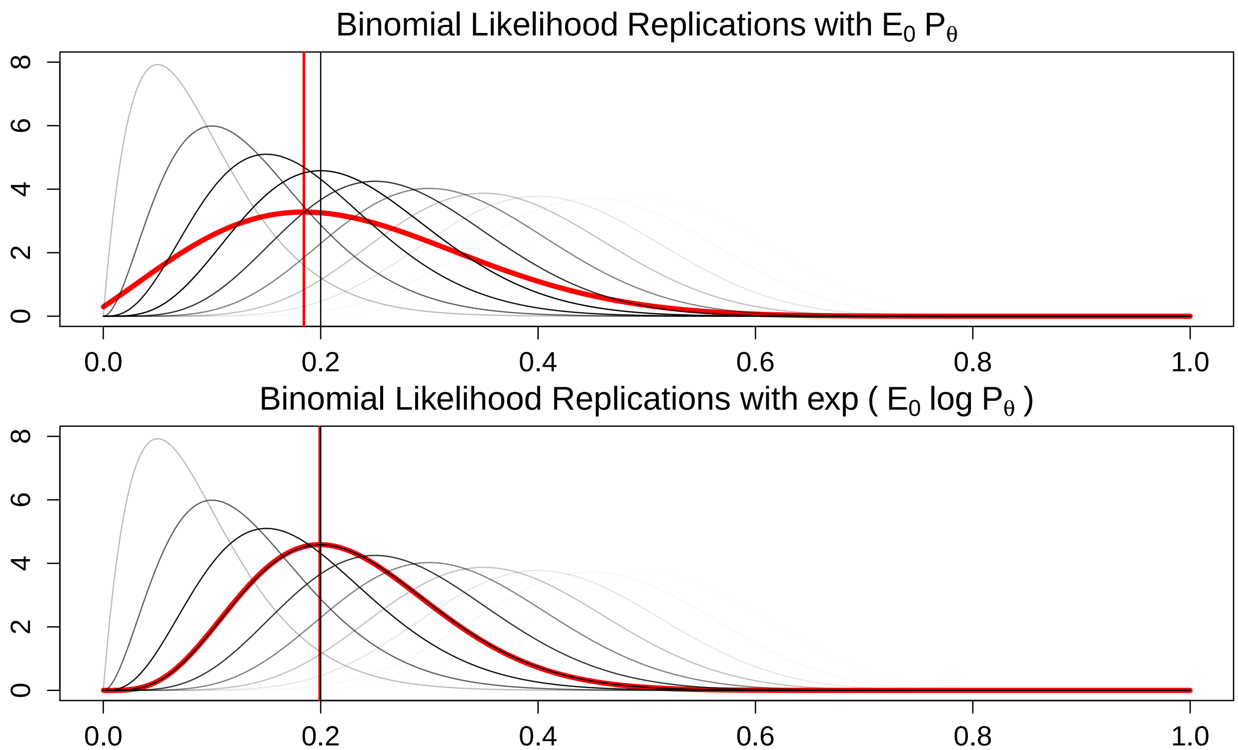

Figure 3: Comparison of expected criterion functions $f_{Y_W}(\theta)$ for a simple maximum likelihood estimation problem. The top frame defines the likelihood as the criterion function, while the bottom frame defines the log-likelihood as the criterion function. In this case, the effective estimand $\bar{\theta}(W)$ is defined by taking the maximizer of the expected criterion function $f_{Y_W}(\theta)$; defining the log-likelihood as the criterion function implies an effective estimand definition that satisfies (R2).

family if and only if the model is well-specified, so that $\mathbb{P}_{\theta_0,W} = \mathbb{P}_{0,W}$ for some $\theta_0 \in \Theta$, and the estimator $\hat{\theta}(Y_W)$, when rewritten as a functional $\Phi_{\hat{\theta}}$, yields the true parameter when it is applied to the superpopulation distribution, so that $\Phi_{\hat{\theta}}(\mathbb{P}_{0,W}, W) = \theta_0$. Succinctly, the effective estimand is, by definition, the value in the parameter space Θ for which an estimation procedure $\hat{\theta}(Y_W)$ is Fisher consistent, if the requirement for a well-specified model is relaxed. Thus, for estimators that are known to be Fisher consistent, most notably the maximum likelihood estimator, the effective esitmand yields the true parameter θ_0 when the model corresponding to the estimator is well-specified.

Example 6 (Binomial Probability Parameter)**.** This example demonstrates that Theorem 2 establishes the correct scale to plug in the expectation $E_{\mathbb{P}_{0,W}}$. Let Y_W be a sample distributed as a binomial random variable with $N_W = 20$ and success probability $\theta = 0.2$, and let the estimator $\hat{\theta}(Y_W, W)$ be the maximum likelihood estimator of a binomial model. $\hat{\theta}(Y_W, W)$ maximizes both $\mathbb{P}_{\theta,W}(Y_W)$ and $\log \mathbb{P}_{\theta,W}(Y_W)$, but the argument from Theorem 2 indicates that the effective estimand $\bar{\theta}(W)$ is defined by the maximizer of $E_{\mathbb{P}_{0,W}} \log \mathbb{P}_{\theta,W}(Y_W)$. Figure 3 shows that maximizing the expected log-likelihood recovers the true parameter $\theta = 0.2$, satisfying (R2), while maximizing the expected likelihood does not.

Remark 7. To our knowledge, Fisher consistency is rarely, if ever, referenced outside of the context of samples composed of independent identically distributed outcomes. By applying the notion of Fisher consistency to independent replications of whole samples, with arbitrary internal dependence structure, specifying a construction of the functional $\Phi_{\hat{\theta}}$, and tying it to a particular asymptotic frame, we have greatly expanded the set of situations in which this notion can be applied.

5 Maximum Likelihood Estimation

A particularly common class of estimators $\hat{\theta}(Y_W, W)$ that satisfy (A2) are estimators based on extrema, where the summarization function $g(\cdot)$ returns an extreme point of the criterion function $f(Y_W)$, the most common being maximum likelihood estimation. This class also includes M-estimators, empirical risk minimization estimators, and maximum a posteriori estimators. In this section, we summarize some results relating the behavior of the estimator $\hat{\theta}(Y_W)$ to the effective estimand $\bar{\theta}_W$ when the estimator is an extremum-based estimator. For concreteness, we will focus on the maximum likelihood estimator.

Let $\hat{\theta}(Y_W)$ be the maximum likelihood esitmator for a model family $\mathcal{P}_{\Theta,\mathcal{W}}$. This is formally defined as

$$\hat{\theta}(Y_W) = \arg\max_{\theta\in\Theta} \log \mathbb{P}_{\theta,W}(Y_W), \tag{16}$$

or the maximizer of the log-likelihood for Y_W defined by the model family $\mathcal{P}_{\Theta,\mathcal{W}}$. We derive the effective estimand for the maximum likelihood estimator here.

Following the functional representation presented in Table 1, the effective estimand of the maximum likelihood estimator can be written:

$$\bar{\theta}_W = \Phi_{\hat{\theta}}(\mathbb{P}_{0,W}, W) = \arg\max_{\theta\in\Theta} E_{\mathbb{P}_{0,W}}[\log \mathbb{P}_{\theta,W}(Y_W)]. \tag{17}$$

The form of Equation 17 has appeared in several parts of the literature before. Sawa (1978) called $\bar{\theta}_W$ the pseudo-true parameter, and pointed out that the estimating functional $\Phi_{\hat{\theta}}(\mathbb{P}, W)$ has a convenient interpretation as a Kullback-Liebler projection of a distribution $\mathbb{P}$ into the model family $\mathcal{P}_{\Theta,W}$; that is, the expression in Equation 17 is equivalent to the minimizer of the Kullback-Liebler divergence $KL(\mathbb{P}_{\theta,W} \| \mathbb{P})$. Earlier, Huber (1967) showed that in the case of independent and identically distributed data, the maximum likelihood estimator converges to a non-context-specific value similar to Equation 17 in large samples, even if the model is misspecified. More recently, Buja et al used this construction to define population coefficients in the case of misspecified linear regression where the regressors are treated as random, and noted the potential for generalization. Similar references to the pseudo-true parameter idea have appeared in the Bayesian modeling literature as well; see Walker (2013) and discussion. However, in all of these cases, the pseudo-true parameter has been treated as a large-sample asymptotic quantity. Spokoiny (2012) does discuss the pseudo-true parameter in a finite-sample context; we will discuss connections to this work in Section 6.

5.1 Examples

Here we provide several examples of the form of the effective estimand $\bar{\theta}(W)$ in cases where the estimator $\hat{\theta}(Y_W, W)$ is derived from maximum likelihood estimation. In these simple cases, the effective estimand often corresponds to an intuitive summary of the contextual superpopulation $\mathbb{P}_{0,W}$. However, the effective estimand is also useful tool for expressing the targets of estimators, even when that summary cannot be written in closed form. We give examples of this latter type in more detail in Section 7.

Example 7 (Linear regression effective estimand)**.** The bias of the maximum likelihood estimator is a particularly common summary for discussion model misspecification in terms of so-called omitted variable bias. In the case of linear regression, it turns out that the effective estimand and the expectation of the maximum likelihood estimator are equivalent:

$$\bar{\theta}_W = E_{\mathbb{P}_{0,W}}(\hat{\theta}(Y_W)) = (X^\top X)^{-1} X^\top E_{\mathbb{P}_{0,W}}(Y_W). \tag{18}$$

This equivalence only holds when the maximum likelihood estimator is linear in the outcome.

Example 8 (Poisson regression effective estimand)**.** Let Y_W be a collection of count-valued variables, and $W = (N, X_W)$, where N is the sample size Y_W, and X_W is a collection of binary covariates, each associated with one observation in Y_W. The investigator proposes a Poisson regression model with canonical link given the binary covariates X_W:

$$Y_i \sim \text{Poisson}(\exp(\theta^{(0} + \theta^{(1)} X_i)) \qquad \text{independently, for each } i = 1, \cdots, N, \tag{19}$$

where $\theta = (\theta^{(0)}, \theta^{(1)})$. In this case, the effective estimand has a closed form:

$$\bar{\theta}^{(0)}_W = \log\left(\frac{\sum_i E_{\mathbb{P}_{0,W}} Y_i (1 - X_i)}{\sum_i (1 - X_i)}\right) \tag{20}$$

$$\bar{\theta}^{(1)}_W = \log\left(\frac{\sum_i E_{\mathbb{P}_{0,W}} Y_i X_i}{\sum_i X_i} \bigg/ \frac{\sum_i E_{\mathbb{P}_{0,W}} Y_i (1 - X_i)}{\sum_i (1 - X_i)}\right) \tag{21}$$

Here, the effective estimand is the log of expected counts within the group of units for which $X_i = 0$, and the log of the ratio of expected counts between the $X_i = 1$ and $X_i = 0$ groups. When the Poisson regression model is well-specified, these are the true population coefficients. However, note that because of the nonlinearity introduced by the log, the expectation of the maximum likelihood estimator, which would move the expectations $E_{\mathbb{P}_{0,W}}$ outside of the log, does not yield the same result.

This non-equivalence holds in all cases where the maximum likelihood estimator is non-linear in Y_W, e.g., generalized linear models with likelihoods of the form

$$\bar{\theta}_W = \arg\max E_{\mathbb{P}_0,W}\left[\sum_i Y_i' X_i\theta - A(X_i\theta)\right] \tag{22}$$

where the log-partition function $A(\cdot)$ has a non-linear first derivative, the estimator is biased even if the model is well-specified, but because the maximum likelihood estimator is Fisher consistent, the effective estimand yields the true parameter, satisfying $(R2)$.

Example 9 (Maximum likelihood estimator for upper bound of uniform random variable)**.** The statistical operation presented in Theorem 2 satisfies (R2) by reducing to the true parameter value even in estimation situations that are considered non-standard. Here, we consider the canonical example of a non-standard the maximum likelihood estimator for the upper bound of a uniform random variable. Suppose that we have a sample Y_W distributed according to

$$Y_i \sim \text{Uniform}(0, \theta_0) \text{ independently, for all } i \in 1, \cdots, N_W \tag{23}$$

and that the investigator estimates θ_0 using the maximum likelihood estimator form a correctly specified model. The estimator has the following form

$$\hat{\theta}(Y_W, W) = \arg\max_\Theta \sum_i [-\log(\theta) + \log(1_{0 \le Y_i \le \theta})] \tag{24}$$

In this case, the effective estimand behaves as expected,

$$\begin{aligned}
\bar{\theta}(W) &= \lim_{M\to\infty} \arg\max_\Theta M^{-1} \sum_{m=1}^{M} \sum_i [-\log(\theta) + \log(1_{0\le Y_i \le \theta})] &(25)\\
&= \lim_{M\to\infty} \arg\max_\Theta -\log(\theta) + M^{-1} \sum_{m=1}^{M} \sum_i \log(1_{0\le Y_i\le\theta}) &(26)\\
&= \arg\max_\Theta -\log(\theta) + E_{\mathbb{P}_{0,W}} \log(1_{0\le Y_i\le\theta}) &(27)\\
&= \arg\max_\Theta -\log(\theta) + \min\left(1, \frac{\theta}{\theta_0}\right) 0 - \max\left(0, \frac{\theta_0 - \theta}{\theta_0}\right)\infty &(28)\\
&= \theta_0, &(29)
\end{aligned}$$

reducing to the true parameter θ_0 and satisfying $(R2)$.

6 Concentration of Estimators about the Effective Estimand

So far, we have established that the effective estimand $\bar{\theta}(W)$ is the quantity for which the estimator $\hat{\theta}(Y_W, W)$ is a plug-in estimator, a relationship illustrated in Figure 1. In this section, we gather several results showing that this relationship is not only symbolic; we can also characterize the sampling distribution of the estimator $\hat{\theta}(Y_W, W)$ in terms of its effective estimand $\bar{\theta}(W)$.

When the effective estimand has a closed-form representation in terms of the moments of the contextual superpopulation $\mathbb{P}_{0,W}$, simple but weak concentration results are available, for example, by application of Chebyshev or similar concentration bounds. We give one example in Example 10.

For extremum-based estimators with more complicated forms, Spokoiny (2012) presented results that characterize the variation of the estimator $\hat{\theta}(Y_W)$ in terms of the effective estimand $\bar{\theta}_W$, by directly bounding the extremum of log-likelihood process. These results are extensions of results from empirical process theory that characterize the behavior of the maximizer of a random function in terms of the maximizer of that function's expectation. In particular, Spokoiny established large deviation bounds for the extremum-based estimators, and finite-sample versions of the locally asymptotically normal theory for maximum likelihood estimation in the medium- and small-deviation regimes. We reproduce the large-deviation result here, writing the final result in our notation.

Theorem 3 (Spokoiny (2012) Theorem 4.3)**.** *Suppose (Er) and (Lr). Let R_k be such that $b(r_k) \equiv b$. Let, for $r \geq r_0$,*

$$1 + \sqrt{x + \mathbb{Q}} \leq 3\nu_0^2 g(r)/b, \tag{30}$$

$$6\nu_0\sqrt{x + \mathbb{Q}} \leq rb, \tag{31}$$

$$\tag{32}$$

with $x + \mathbb{Q} \geq 2.5$ and $\mathbb{Q} = c_1 p$. Then

$$\mathbb{P}\left(\hat{\theta}(Y_W, W) \notin \Theta_{\bar{\theta}(W)}(r_0)\right) \leq \exp(-x) \tag{33}$$

Example 10 (Concentration around effective estimand)**.** In the problem set up from Example 8, we can specify relatively simple concentration bounds for the maximum likelihood estimator $\hat{\theta}(Y_W, W)$ about its effective estimand $\bar{\theta}(W)$.

Proposition 4. *The maximum likelihood estimator $\hat{\theta}(Y_W, W)$ of the model described in Equation 19 concentrates around its effective estimand $\bar{\theta}(W)$ for all contexts W, with probability bounds given* *by*

$$\mathbb{P}(|\hat{\theta}(Y_W, W)^{(1)} - \bar{\theta}(W)^{(1)}| \le \log(1+\delta)) \ge 1 - \frac{\mathrm{Var}_{\mathbb{P}_{0,W}}\left(\sum Y_i(1-X_i)\right)}{\delta^2\left(E_{\mathbb{P}_{0,W}}\sum Y_i(1-X_i)\right)^2}$$

$$\mathbb{P}(|\hat{\theta}(Y_W, W)^{(2)} - \bar{\theta}(W)^{(2)}| \le \log(1+\delta)) \ge 1 - \frac{4\mathrm{Var}_{\mathbb{P}_{0,W}}\left(\sum Y_i(1-X_i)\right)}{\delta^2\left(E_{\mathbb{P}_{0,W}}\sum Y_i(1-X_i)\right)^2} - \frac{4\mathrm{Var}_{\mathbb{P}_{0,W}}\left(\sum Y_iX_i\right)}{\delta^2\left(E_{\mathbb{P}_{0,W}}\sum Y_iX_i\right)^2}$$

Proof. We derive the probability bound for $\hat{\theta}_0$ explicitly. The same formulation can be followed for $\hat{\theta}_1$.

$$\mathbb{P}\left(|\hat{\theta}(Y_W, W)^{(1)} - \bar{\theta}(W)^{(1)}| \le \log(1+\delta)\right) \tag{34}$$

$$\ge \mathbb{P}\left((1-\delta) \le \left(\frac{\sum Y_i(1-X_i)}{\sum E_{\mathbb{P}_{0,W}} Y_i(1-X_i)}\right) \le (1+\delta)\right) \tag{35}$$

$$= \mathbb{P}\left(\left|\sum Y_i(1-X_i) - \sum E_{\mathbb{P}_{0,W}} Y_i(1-X_i)\right| \le \delta \sum E_{\mathbb{P}_{0,W}} Y_i(1-X_i)\right) \tag{36}$$

$$\ge 1 - \frac{\mathrm{Var}_{\mathbb{P}_{0,W}}\left(\sum Y_i(1-X_i)\right)}{\delta^2\left(E_{\mathbb{P}_{0,W}}\sum Y_i(1-X_i)\right)^2}, \tag{37}$$

where the final step is an application of the Chebyshev inequality.

For the other coefficient, we bound a similar deviation for the quantity

$$\hat{\theta}(Y_W, W)^{(1)} + \hat{\theta}(Y_W, W)^{(2)} = \log\left(\frac{\sum E_{\mathbb{P}_{0,W}} Y_iX_i}{\sum X_i}\right)$$

separately.

$$\mathbb{P}(|(\hat{\theta}(Y_W, W)^{(1)} + \hat{\theta}(Y_W, W)^{(2)})) - (\bar{\theta}(W)^{(1)} + \bar{\theta}(W)^{(2)})| \le \log(1+\delta)) \le 1 - \frac{d}{\delta^2 E_{\mathbb{P}_{0,W}}\sum Y_iX_i}$$

Combining these bounds, we obtain a deviation bound for $|\hat{\theta}_1 - \bar{\theta}_1|$

$$\begin{aligned}
&\mathbb{P}\left(|\hat{\theta}(Y_W, W)^{(2)} - \bar{\theta}(W)^{(2)}| \le \delta\right) \\
&\quad \ge 1 - \mathbb{P}(|\hat{\theta}(Y_W, W)^{(1)} - \bar{\theta}(W)^{(1)}| \ge \delta/2) \\
&\qquad - \mathbb{P}(|(\hat{\theta}(Y_W, W)^{(1)} + \hat{\theta}(Y_W, W)^{(2)}) - (\bar{\theta}(W)^{(1)} + \bar{\theta}(W)^{(2)})| \ge \delta/2) \\
&\quad \ge 1 - \frac{4\mathrm{Var}_{\mathbb{P}_{0,W}}\sum Y_i(1-X_i)}{\delta^2\left(E_{\mathbb{P}_{0,W}}\sum Y_i(1-X_i)\right)^2} - \frac{4\mathrm{Var}_{\mathbb{P}_{0,W}}\sum Y_i(1-X_i)}{\delta^2\left(E_{\mathbb{P}_{0,W}}\sum Y_iX_i\right)^2}
\end{aligned}$$

□

7 Examples

7.1 Template

In this section, we present a series of example investigations. Each example includes a definition of the context W, outcome Y_W, and range of validity $\mathcal{W}$; an estimation procedure $\hat{\theta}(Y_W, W)$ with corresponding parameter space Θ; and a description of a set of contextual superpopulations $\{\mathbb{P}_{0,W'} : W' \in \mathcal{W}\}$ for which the estimator' $\hat{\theta}(Y_W, W)$ effective estimand $\bar{\theta}(W)$ is not invariant to the context W'.

The key idea that these examples highlight is that when an estimator $\hat{\theta}(Y_W, W)$ is not a valid basis of generalization per Definition 2, an investigator employing $\hat{\theta}(Y_W, W)$ will be estimating a different quantity in different contexts W'. This goes a step beyond the conventional view that misspecified models simply estimate a "best approximation" to the contextual superpopulation process $\mathbb{P}_{0,W}$; here we show that what constitutes the "best approximation" is a function of context that the investigator would rather treat as ancillary.

The instability of the effective estimand $\bar{\theta}(W)$ in these examples does not indicate that the estimator $\hat{\theta}(Y_W, W)$ is generating a mistaken or spurious summary of the data. Rather, it indicates that the $\hat{\theta}(Y_W, W)$ approximates a summary of the contextual superpopulation $\mathbb{P}_{0,W}$ that is incomparable across contexts. In each of the examples below, it could be argued that the estimator $\hat{\theta}(Y_W, W)$ is an informative summary of the data, e.g., for characterizing replications within the observed context W, even if it does not constitute a valid basis for generalization.

7.2 Linear Regression

Linear regression is one of the most-used statistical estimation procedures, and is often applied in contexts where the investigator acknowledges that the generative model underlying the linear regression procedure is misspecified. In fact, In fact, in some parts of the literature geared toward industrial applications, for example, Box & Draper (1959), applying a regression procedure $\hat{\theta}(Y_W, W)$ is referred to as "graduating" rather than "estimating" a conditional response function. The standard advice to exercise caution when extrapolating from linear regression fits can be restated clearly in terms of the effective estimand. The discussion in this section closely parallels Buja et al.

For linear regression, the context is composed of a sample size and a covariate vector for each sampled unit, $W = (N_W, X_W)$ for some $N_W \in \mathbb{N}$ and some $X_W \in \mathbb{R}^{N\times p}$, and a real-valued outcome $Y_W \in \mathbb{R}^N$. We consider the range of validity $\mathcal{W}$ to be the set of all contexts of any sample size, for which the covariates X_W all lie within a ball in the covariate space $B_X(r)$ centered, without loss of generality, at 0 with radius r, so $\mathcal{W} = \{(N_{W'}, X_{W'}) : N_{W'} \in \mathbb{N}, X_i \in B_X(r) \text{ for each } X_i \in X_W\}$.

The estimator $\hat{\theta}(Y_W, W)$ and its effective estimand $\bar{\theta}(W)$ are given by:

$$\hat{\theta}(Y_W, W) = \left(X_W' X_W\right)^{-1} X_W' Y_W; \qquad \bar{\theta}(W) = \left(X_W' X_W\right)^{-1} X_W' E_{\mathbb{P}_{0,W}} Y_W. \tag{38}$$

In this case, the effective estimand and the expectation of the estimator are equivalent, so $\bar{\theta}(W) = E_{\mathbb{P}_{0,W}} \hat{\theta}(Y_W, W)$.

We will assume that the contextual superpopulation distribution of any sample Y_W is independent with additive, mean-zero errors ϵ_i, so that

$$Y_i \sim E_{\mathbb{P}_{0,W}}[Y_i \mid X_i] + \epsilon_i \text{ independently, for each } i = 1, \cdots, N_W. \tag{39}$$

Under this independence assumption, the effective estimand is also equivalent to a large-sample limit of the estimator, similar to the limit presented by Buja et al. Let $W_n(W)$ be a context parameterized by sample size, where $N_{W_n(W)} = n$ and $X_{W_n(W)}$ be a set of covariates of size n drawn from the empirical distribution of the original covariate set X_W, i.e., by bootstrap of the covariates X_W. Then $\lim_{n\to\infty} \hat{\theta}(Y_{W_n(W)}, W_n(W)) = \bar{\theta}(W)$.

Example 11 (Simple Linear Regression with Nonlinear Superpopulation)**.** Suppose that an investigator defines the context such that the covariates X_W have a 2-vector $X_i = (1, X_i^{(1)})$ associated with each outcome Y_i. Suppose that all contextual superpopulations of interest $\{\mathbb{P}_{0,W'} : W' \in \mathcal{W}\}$ actually have conditional expectation functions that are quadratic in $X_i^{(1)}$:

$$E_{\mathbb{P}_{0,W}}[Y_i \mid X_i] = \theta_0^{(0)} + \theta_0^{(1)} X_i^{(1)} + \theta_0^{(2)} {X_i^{(1)}}^2 \text{ for each } i \in 1, \cdots, N_W. \tag{40}$$

We will denote the components of the estimator as $\hat{\theta}(Y_W, W) = (\hat{\theta}(Y_W, W)^{(0)}, \hat{\theta}(Y_W, W)^{(1)})$, where $\hat{\theta}(Y_W, W)^{(0)}$ is the estimated intercept coefficient, and $\hat{\theta}(Y_W, W)^{(1)}$ is the linear coefficient on X_i. Likewise, we define the components of the effective estimand $\bar{\theta}(W) = (\bar{\theta}(W)^{(0)}, \bar{\theta}(W)^{(1)})$.

In this case, the effective estimand $\bar{\theta}(W)$ has the following general form, similar to a result discussed in Box & Draper (1959). Letting $\bar{X}_W = N_W^{-1} \sum_i X_i^{(1)}$, $S_{X_W^2} = N_W^{-1} \sum_i {X_i^{(1)}}^2$ and $S_{X_W^3} = N_W^{-1} \sum_i {X_i^{(1)}}^3$ be the second and third empirical moments of the covariate set X_W,

$$\bar{\theta}(W)^{(0)} = E_{\mathbb{P}_{0,W}} \hat{\theta}(Y_W, W)^{(0)} = \theta_0^{(0)} + \left(S_{X_W^2} - \frac{S_{X_W^3} - \bar{X}_W S_{X_W^2}}{S_{X_W^2} - \bar{X}_W^2} \bar{X}_W \right) \theta_0^{(2)} \tag{41}$$

$$\bar{\theta}(W)^{(1)} = E_{\mathbb{P}_{0,W}} \hat{\theta}(Y_W, W)^{(1)} = \theta_0^{(1)} + \frac{S_{X_W^3} - \bar{X}_W S_{X_W^2}}{S_{X_W^2} - \bar{X}_W^2} \theta_0^{(2)}. \tag{42}$$

The effective estimand here depends strongly on the first three sample moments of the covariate set X_W, so misspecified linear regression estimator $\hat{\theta}(Y_W, W)$ is not a valid basis of generalization per Definition 2. Figure 4 illustrates a particular case of this example, with two contexts W and W', with $N_W = 4, X_W = \{-1.2, -0.5, -0.1, 0.1\}$ in one context, and $N_{W'} = 3, X_{W'} = \{-0.25, 0.5, 1\}$.

In this case, the effective estimand corresponds to a regression surface with a slope of a different sign depending on the chosen context.

In general, the effective estimand $\bar{\theta}(W)$ of a linear regression depends on the context W whenever the conditional expectation function $E_{\mathbb{P}_{0,W}}[Y_i \mid X_i]$ is nonlinear in the covariates X_i, a point that Buja et al. (2016) discuss in great detail. In these cases, $\hat{\theta}(Y_W, W)$ implies a summary of the contextual superpopulation $\bar{\theta}(W) = \Phi_{\hat{\theta}}$ that depends on the analyzed context W, which has major implications for generalization. In particular if the investigator wishes to build theory on the basis of the parameter estimate $\hat{\theta}(Y_W, W)$, they much acknowledge that $\hat{\theta}(Y_W, W)$ is a summary of both the system of interest and the mechanism used to choose the analytical context W. Likewise, if the investigator wishes to make predictions in other contexts, they must exercise caution when the predictive context W' differs too much from the analytical context W.

7.3 Generalized Linear Models

Generalized linear models are a class of models that generalize linear regression, and are often applied in similar ways (McCullagh & Nelder, 1989). The context W and range of validity $\mathcal{W}$ are the same as in the linear regression case, but the outcome space of Y_W differs. In addition, for generalized linear models, the effective estimand $\bar{\theta}(W)$ is not, in general, equivalent to the expected estimator $E_{\mathbb{P}_{0,W}}\hat{\theta}(Y_W, W)$.

The estimator is given by

$$\hat{\theta}(Y_W, W) = \arg\max_{\Theta} \left[\sum_i Y_i' X_i \theta - A(X_i \theta) \right] \tag{43}$$

$$= \text{root}_{\Theta} \left[\sum_i (Y_i - A'(X_i \theta)) X_i \right] \tag{44}$$

and the effective estimand is given by

$$\bar{\theta}(W) = \arg\max_{\Theta} E_{\mathbb{P}_{0,W}} \left[\sum_i Y_i' X_i \theta - A(X_i \theta) \right] \tag{45}$$

$$= \text{root}_{\Theta} \left[\sum_i (E_{\mathbb{P}_{0,W}} Y_i - A'(X_i \theta)) X_i \right]. \tag{46}$$

Here, if the derivative $A'(X_i\theta)$ is not linear in $X_i\theta$, the solution to $\bar{\theta}(W)$ is not linear in $E_{\mathbb{P}_{0,W}}$, so the $\bar{\theta}(W) \neq E_{\mathbb{P}_{0,W}}\hat{\theta}(Y_W, W)$, unlike the linear regression case.

Again, here we assume that the contextual superpopulation distribution of any sample Y_W is independent, and distributed as a random variable $F(\cdot)$ parameterized by its expectation

$$Y_i \sim F(E_{\mathbb{P}_{0,W}}[Y_i \mid X_i]) \text{ independently, for each } i = 1, \cdots, N_W. \tag{47}$$

Like the linear regression case, this independence assumption induces an equivalence between the effective estimand and the same large-sample limit of the estimator presented in Section 7.2, so that $\lim_{n\to\infty} \hat{\theta}(Y_{W_n(W)}, W_n(W)) = \bar{\theta}(W)$ under an asymptotic that bootstraps the covariates X_W presented in Section 7.2.

Example 12 (Simple Poisson Regression with Nonlinear Superpopulation)**.** Assume the same context and parameter definitions as Example 11, but with a count-valued outcome $Y_W \in \mathbb{N}^N$. Assume that the log-expectation of each unit in Y_W is given by a quadratic function in $X_i^{(1)}$:

$$\log E_{\mathbb{P}_{0,W}}[Y_i \mid X_i] = \theta_0^{(0)} + \theta_0^{(1)} X_i^{(1)} + \theta_0^{(2)} {X_i^{(1)}}^2 \text{ for each } i \in 1, \cdots, N_W. \tag{48}$$

The effective estimand $\bar{\theta}(W)$ here has the general form:

$$\bar{\theta}(W) = \text{root}_\Theta \left[\sum_i \exp\left(\theta_0^{(0)} + \theta_0^{(1)} X_i^{(1)} + \theta_0^{(2)} {X_i^{(1)}}^2\right) X_i - \right. \tag{49}$$
$$\left. \sum_i \exp\left(\bar{\theta}(W)^{(0)} + \bar{\theta}(W)^{(1)} X_i^{(1)}\right) X_i \right]$$

Given that the exponentiated expressions in Equation 49 contain polynomials of X_i of different orders when $\theta_0^{(2)}$ is nonzero, no single value $\bar{\theta}(W)$ can satisfy Equation 49 for all covariate sets X_W. Thus, an investigator can deduce that under this type of misspecification, $\hat{\theta}(Y_W, W)$ is not a valid basis of generalization. Figure 4 illustrates a particular case of this example, with two contexts W and W', with $N_W = 4, X_W = \{-1.2, -0.5, -0.1, 0.1\}$ in one context, and $N_{W'} = 3, X_{W'} = \{-0.25, 0.5, 1\}$. In this case, the effective estimand corresponds to a regression surface with a slope of a different sign depending on the chosen context.

The consequences of an effective estimand $\bar{\theta}(W)$ that depends on the specific context W for generalized linear models are analogous to the consequences for linear regression models, presented in Section 7.2.

7.4 Process Models

Process models are popular tools for designing analyses of dependent data. In the analysis of timeseries and spatial data, a common modeling assumption is that the observed data are drawn from a stationary process. Translating into our notation, in a minimal example $W = (N_W, T_W)$ where T_W is a set of points at which the process is observed, and $Y_W = (Y_t : t \in T_W)$ are the

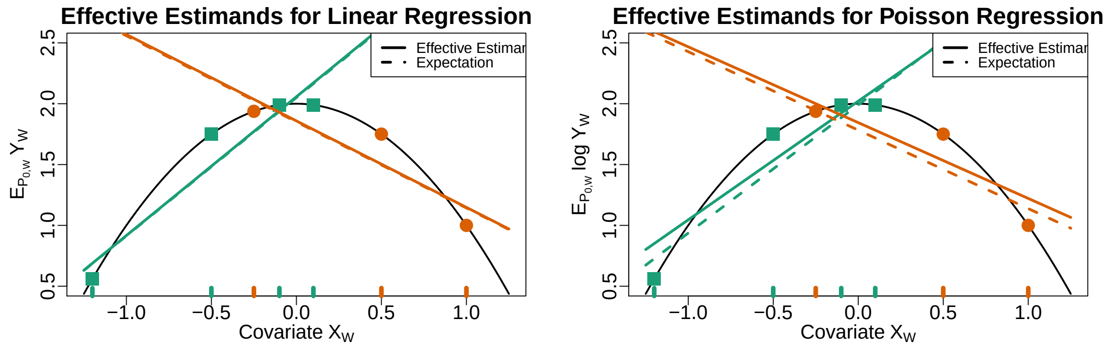

Figure 4: Illustrations for Examples 11 and 12. Response surfaces (solid lines) corresponding to the effective estimand for ordinary least squares (left) and Poisson regression (right) with a linear specification when the true response surface is quadratic (solid curve) in two distinct contexts: in the green context, $N_W = 4, X_W = \{-1.2, -0.5, -0.1, 0.1\}$, while in the orange context, $N_{W'} = 3, X_{W'} = \{-0.25, 0.5, 1\}$. In both cases, the effective esitmand varies as a function of the covariate values X_W and $X_{W'}$. In the ordinary least squares case, the effective estimand surface corresponds to the “zero noise” regression surface suggested by Buja et al. In the Poisson regression case, the effective estimand surface is not the same as the surface implied by the expected poisson regression coefficients (dashed lines). The effective estimand defines the analog of the “zero noise” regression surface for generalized regression methodologies for which sampling error is not additive.

outcome as these temporal or spatial locations. The stationarity assumption is that,

$$(Y_{t^{(0)}}, \cdots, Y_{t^{(N_W)}}) \sim (Y_{t^{(0)}+\tau}, \cdots, Y_{t^{(N_W)}+\tau}) \tag{50}$$

or that the distribution of outcomes Y_W is invariant to shifts in observed locations. This assumption can be weakened to specify invariance in the moments of Y_W up to a particular order. Stationary processes have fixed marginal moments for each observation Y_t that do not depend on t.

In this example, we use the effective estimand $\bar{\theta}(W)$ to understand how an estimator $\hat{\theta}(Y_W, W)$ derived from a stationary model behaves when it is applied to non-stationary data. Here, we consider the simplest possible stationary process, a white-noise process composed of independent and identically distributed normal random variables, and a simple non-stationary process given by Brownian motion. We will define the range of validity $\mathcal{W}$ to be restricted to contexts W where $t^{(0)} = 0$ for all T_W.

Example 13 (White Noise Model and Brownian Motion Superpopulation)**.** Suppose that Y is a one-dimensional standard Brownian motion with $Y_0 = 0$. An investigator samples observations from this process by choosing a sampling context $W = (N_W, T_W)$. For any W, the contextual superpopulation defines the distribution of Y_W as

$$Y_{t^{(k)}} \sim \begin{cases} 0 & \text{for} \quad k = 0, \\ \text{Normal}\left(Y_{t^{(k-1)}}, t^{(k)} - t^{(k-1)}\right) & \text{for} \quad k > 0 \end{cases} \tag{51}$$

Suppose that the investigator is interested in the variation of samples Y_W drawn from the process Y.

The investigator specifies the sample variance as the estimator $\hat{\theta}(Y_W, W)$. The estimator $\hat{\theta}(Y_W, W)$ and effective estimand $\bar{\theta}(W)$ are

$$\hat{\theta}(Y_W, W) = \sum_k (Y_{t^{(k)}} - \bar{Y}_W)^2/(N_W - 1); \quad \bar{\theta}(W) = E_{\mathbb{P}_{0,W}}\left[\sum_k (Y_{t^{(k)}} - \bar{Y}_W)^2\right]/(N_W - 1). \quad (52)$$

This choice of estimator could be justified as being the restricted maximum likelihood estimator for the variance parameter σ^2 from a white-noise model for Y_W defined as

$$Y_{t^{(k)}} \sim \text{Normal}(0, \sigma^2) \text{ independently for } k = 1, \cdots, N_W, \quad (53)$$

but the investigator could also use this estimator as part of a moment-matching estimation procedure for any stationary process model, e.g., an $AR(p)$ process.

Defining the time increments $\delta^{(k)} = t^{(k)} - t^{(k-1)}$ for each $t^{(k)}$ in T_W, we can represent Y_W as follows

$$Z_W \sim \text{MVNormal}_N(0, I_{N_W}) \quad (54)$$

$$S_W = \begin{pmatrix} {\delta^{(1)}}^{1/2} & & & \\ {\delta^{(1)}}^{1/2} & {\delta^{(2)}}^{1/2} & & \\ & \vdots & \ddots & \\ {\delta^{(1)}}^{1/2} & {\delta^{(2)}}^{1/2} & \cdots & {\delta^{(N_W)}}^{1/2} \end{pmatrix} \quad (55)$$

$$Y_W \sim S_W Z_W. \quad (56)$$

If we define the idempotent linear operator that subtracts the mean from each element of a vector as

$$D_W = I - \frac{1}{N_W}\mathbf{1}_{N_W}\mathbf{1}'_{N_W}, \quad (57)$$

where $\mathbf{1}_{N_W}$ is a column vector of N_W ones, then the effective estimand can be computed as the expectation of a quadratic form

$$\bar{\theta}(W) = (N_W - 1)^{-1} E_{\mathbb{P}_{0,W}}[Z'_W(S'_W D'_W D_W S_W) Z_W] \quad (58)$$

$$= (N_W - 1)^{-1}\,\text{trace}(S'_W D'_W D_W S_W) = (N_W - 1)^{-1}\,\text{trace}(D_W S_W S'_W) \quad (59)$$

$$= (N_W - 1)^{-1} \sum_{k=0}^{N_W - 1} k \frac{N_W - k}{N_W} \delta^{(k)}. \quad (60)$$

In a simple case, where the increments are equal, $\delta^{(k)} = \delta$ for each k, this simplifies further to

$$\bar{\theta}(W) = \delta \frac{N_W + 1}{6} \quad (61)$$

The effective estimand is a function of the placement of the set of observation points $T_W = (t^{(k)} : k = 0, \cdots, N_W)$ specified by the context W. Thus, for a Brownian motion process, the sample variance estimator $\hat{\theta}(Y_W, W)$ is not a valid basis of generalization per Definition 2. Figure 5 illustrates this example.

7.5 Social Network Analysis

Social networks have a sparsity property, where the number of pairwise relationships among a set of actors tends to represent a smaller and smaller proportion of the total possible pairwise interactions the larger the sample of actors becomes (Orbanz & Roy, 2013). One difficulty in network modeling is that many network models are built on a conditional independence or exchangeability assumption about the data generating process that cannot represent this scaling behavior. This mismatch an present difficulties for generalization between network contexts. This example reproduces an example in D'Amour & Airoldi (2016).

Represented in our notation, social network data are defined with respect to a set of actors V_W of size N_W drawn from an actor population $\mathbb{V}$, with $\binom{N_W}{2}$ pairwise outcomes recorded as Y_W and pairwise covariates recorded as X_W. Formally, the context W is (N_W, V_W, X_W), where $N_W \in \mathbb{N}, V_W \subset \mathbb{V}, X_W \in \mathbb{R}^{\binom{N}{2} \times p}\}$. The pairwise outcomes $Y_W \in \mathcal{Y}^{\binom{N}{2}}$, where the outcome space for each pair $\mathcal{Y}$ can have a variety of supports, for example, it can be binary, count-valued, real-valued, or point process–valued.

Example 14 (Poisson Regression Model for Sparse Social Network)**.** Consider a simple example where the outcomes are count-valued, so $\mathcal{Y} = \mathbb{N}$. Let each $Y_{ij} \in Y_W$ represent the number of coauthorships between two actors i and j for each ij, and let $X_{ij} \in X_W$ be a binary covariate indicating whether actors i and j work at the same institution. Suppose the investigator uses a Poisson regression model with an intercept and covariate X_W to explain the coauthorship counts Y_W, defining the estimator $\hat{\theta}(Y_W, W)$ as the maximum likelihood estimator for the Poisson regression. Then the effective estimand $\bar{\theta}(W)$ has the same form as the effective estimand presented in Example 8:

$$\bar{\theta}_W^{(0)} = \log\left(\frac{\sum_{ij} E_{\mathbb{P}_{0,W}} Y_{ij}(1 - X_{ij})}{\sum_{ij}(1 - X_{ij})}\right) \tag{62}$$

$$\bar{\theta}_W^{(1)} = \log\left(\frac{\sum_{ij} E_{\mathbb{P}_{0,W}} Y_{ij} X_{ij}}{\sum_{ij} X_{ij}} \Bigg/ \frac{\sum_{ij} E_{\mathbb{P}_{0,W}} Y_{ij}(1 - X_{ij})}{\sum_{ij}(1 - X_{ij})}\right) \tag{63}$$

However, in this case the expectation $E_{\mathbb{P}_{0,W}}$ has additional structure. The sparsity property of a network process can be written as follows

Definition 6 (Sparse Graph Process)**.** Let $Y_{\mathbb{V}}$ be a random graph process on $\mathbb{V}$. $Y_{\mathbb{V}}$ is *sparse* if and

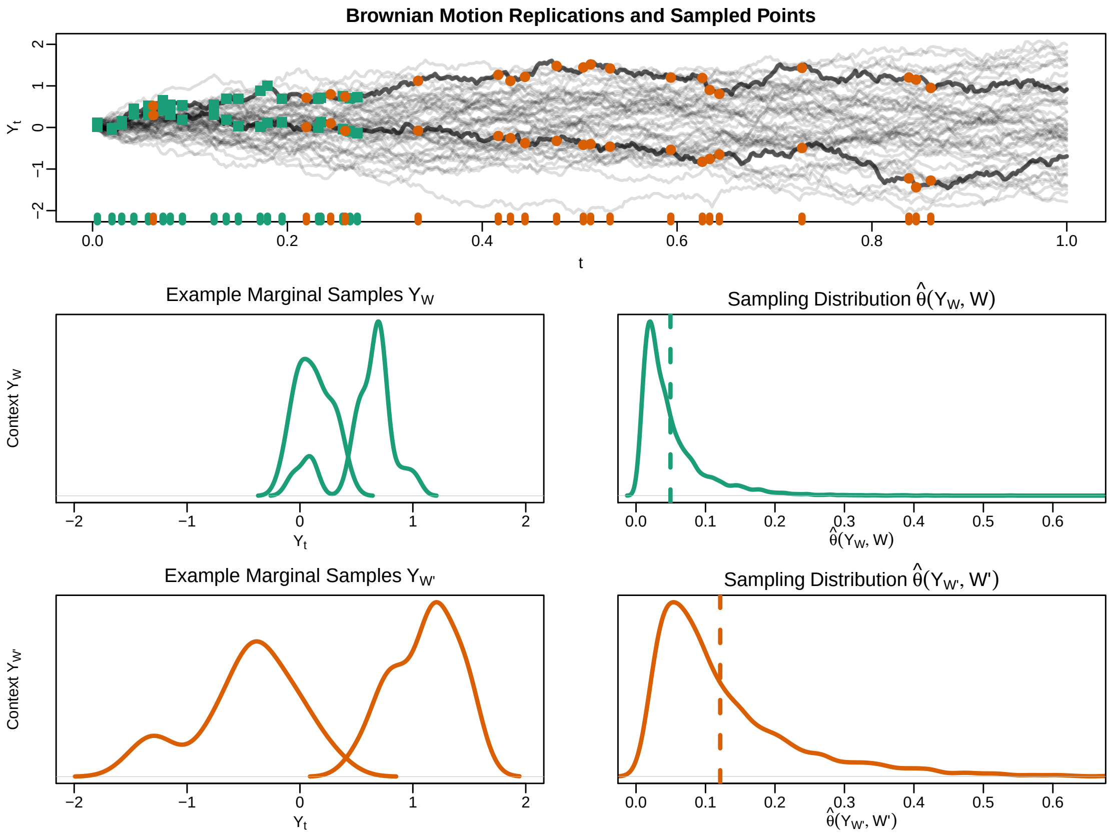

Figure 5: Non-stationary process illustration from Example 13, demonstrating that an estimator that computes marginal sample moments is not a valid basis for generalization here. The top panel shows a set of replicated sample paths from a standard Brownian motion on the interval $[0, 1]$, with two paths highlighted for illustration. In this example, the context specifies the points at which the investigator observes the sample path. We compare the effective estimand $\bar{\theta}(W)$ of the sample variance estimator $\hat{\theta}(Y_W, W)$ when applied in two sampling contexts W and W'. The context W, represented in green, has 20 observation points in the interval $[0, 3]$. The context W', represented in orange, has 20 observation points in the inveral $[0, 1]$. The investigator computes the sample variance estimator $\hat{\theta}(Y_W, W)$, treating the mean as known. The left panels show sample distributions of the points observed on the two example sample paths under each context W and W'. The right panels show the sampling distribution of $\hat{\theta}(Y_W, W)$, with the vertical line drawn at the value of the effective estimand $\bar{\theta}(W)$. The choice of context has a clear effect on the value of the effective estimand $\bar{\theta}(W)$, so $\hat{\theta}(Y_W, W)$ does not satisfy Definition 2.

only if for any $\epsilon > 0$ there exists an n such that for any context W with $N_W > n$ the corresponding finite dimensional random graph Y_W has the property $E_{\mathbb{P}_{0,W}}(\sum_{ij} \mathbf{1}_{Y_{ij} \neq 0}) < \epsilon$.

Succinctly, for contexts with larger vertex sets V_W, we expect a smaller proportion of the $\binom{N_W}{2}$ pairs of actors to have nonzero outcomes.

Suppose that the investigator's range of validity $\mathcal{W}$ includes contexts of varying size N'_W, but for which the following assumptions hold for all W':

(X1) All institutions have finite size.

(X2) A non-vanishing fraction of institutions have a positive number of expected within-institution coauthorships.

Suppose that the following facts are also true about each contextual superpopulation

(Y1) Each sample $Y_{W'}$ is a finite-dimensional sample drawn from the same sparse graph process.

(Y2) Each pairwise outcome Y_{ij} as a finite expectation.

(Y3) Each contextual superpopulation satisfies identification conditions given in D'Amour and Airoldi 2016.

Under these assumptions, D'Amour & Airoldi (2016) show that $\bar{\theta}^{(0)}$ can be made arbitrarily negative, and $\bar{\theta}^{(1)}$ can be made arbitrarily large by choosing an ever-larger sample size N_W. This is because the sparsity property $(Y1)$ and the finite expectation property $(Y2)$ combine to ensure that the ratio in Equation 62 approaches 0, while the contextual properties $(X1)$ and $(X2)$ ensure that the ratio in the numerator of Equation 63 remains positive. Thus, the poisson regression maximum likelihood estimator $\hat{\theta}(Y_W, W)$ is not a valid basis for generalization per Definition 2.

Example 15 (Large-Scale Point Process Regression on Inventor Network)**.** Figure 6, reproduced from D'Amour & Airoldi (2016), demonstrates the consequences of a shifting effective estimand in large-scale social network analysis with an example using the United States patent record (Li et al., 2014). They employ an estimator $\hat{\theta}(Y_W, W)$ based on a point process model that generalizes the poisson regression above. They model the patent coauthorship timeseries Y_W in terms of pairwise covariates X_W that encode, for each inventor pair ij in the inventor set V_W, and each time t in the observation period, whether the inventors i and j work for the same firm at time t, and whether the inventors i and j collaborated previously to time t. They applied this estimator $\hat{\theta}(Y_W, W)$ to inventor networks in metropolitan areas of differing size and showed a clear relationship between the estimates and the size N_W of the inventor set V_W in each region.

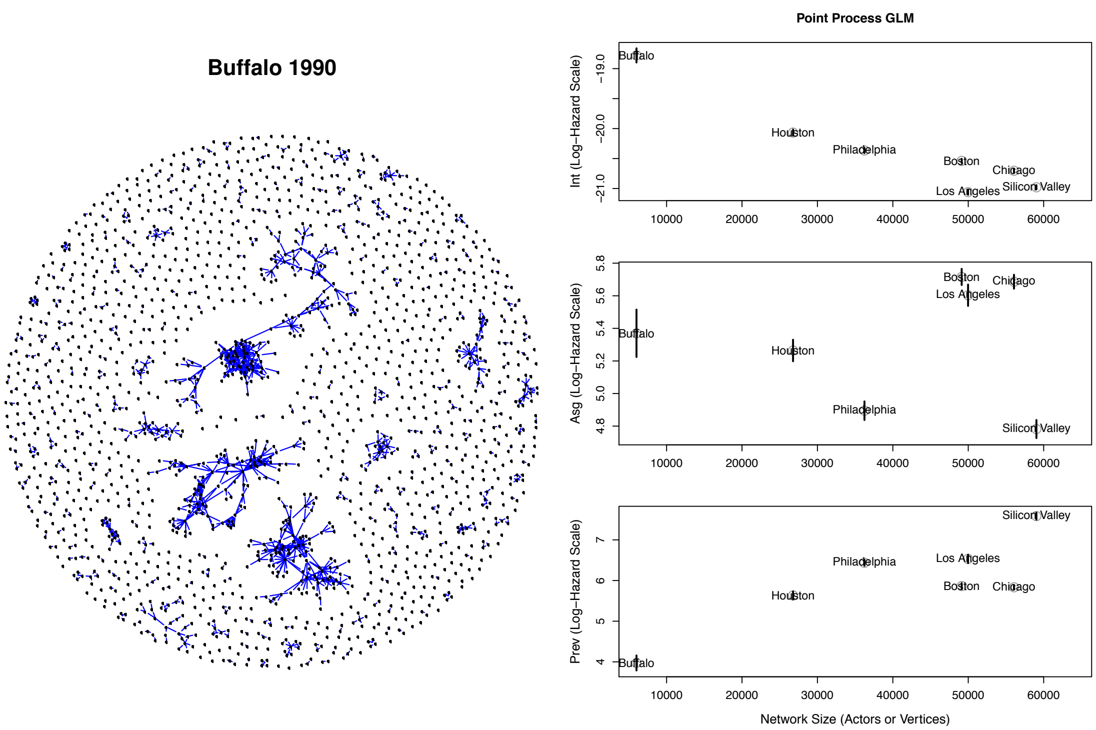

Figure 6: Left: An example network of an inventor collaboration network from the Buffalo, New York metropolitan area in a three-year window ending in 1990, a small subgraph taken from Li et al. (2014). Nodes are inventors and ties between nodes indicate a coauthorship between the connected inventors on a patent. Right: Estimated coefficients from a point process generalized linear model to describe inventor collaboration frequencies in a sample of large metropolitan areas in the United States. From top to bottom, the coefficients are for the intercept term `Int`, a binary covariate `Asg` indicating whether two inventors work at the same firm, and a binary covariate `Prev` indicating whether two inventors had coauthored a patent previously. As expected, there is a strong dependence of the `Int` and `Prev` coefficients on sample size that is well-explained by the sparsity property of social networks.

8 Discussion

8.1 The Effective Estimand in Practice

In the paper, we have outlined the effective estimand $\bar{\theta}(W)$ as a theoretical construct that can be used to describe common problems that can undermine investigators' attempts to generalize conclusions drawn on the basis of an estimator $\hat{\theta}(Y_W, W)$. In this section, we give advice for how the effective estimand idea can be used to detect such problems in practice. More formal treatment of these procedures is left to future work.

The difficulty in applying the effective estimand is that $\bar{\theta}(W)$ is defined in terms of expectations $E_{\mathbb{P}_{0,W}}$ taken with respect to an unknown contextual superpopulation distributions. In practice, we see methods by which investigators can test whether their estimator $\hat{\theta}(Y_W, W)$ is a valid basis for generalization across their range of validity $\mathcal{W}$. From the methods requiring the strongest assumptions to the weakest: investigators can fully specify a contextual superpopulation distributions $\mathbb{P}_{0,W'}$ for each $W' \in \mathcal{W}$ and compute $\bar{\theta}(W)$ directly or through simulation; investigators can assume constraints on the contextual superpopulation distributions $\mathbb{P}_{0,W'}$ for each $W' \in \mathcal{W}$ without fully specifying each distribution and check deductively whether the constraints these induce on the effective estimand $\bar{\theta}(W)$ invalidate it as a basis of generalization; or investigators can subsample or resample from an observed context W and use the estimator $\hat{\theta}(Y_W, W)$ as a test statistic to determine wither the effective estimand $\bar{\theta}(W)$ is invariant across these derived contexts W'.

The examples in the previous section demonstrate the use of the first two strategies that require direct assumptions to be made about the contextual superpopulations $\mathbb{P}_{0,W'}$ for $W' \in \mathcal{W}$. These approaches are useful in situations where the investigator is attempting to understand a complex process where, for the sake of parsimony, not all known properties of the system under study have been incorporated into the design of the estimator $\hat{\theta}(Y_W, W)$. In these cases, these left-over properties can be used to constrain contextual superpopulations $\mathbb{P}_{0,W'}$ for checking for instability in the effective estimand $\bar{\theta}(W)$.

The final subsampling or resampling strategy has significant overlap with the existing literatures on the bootstrap, jackknife, cross-validation, and other data perturbation schemes (Yu, 2013; Bickel & Freedman, 1981). We offer two suggestions in light of the effective estimand theory developed here. First, investigators should attempt to understand variance estimates obtained from these methods in terms of within-context sampling variability in $\hat{\theta}(Y_W, W)$ and between-context variation in the effective estimand $\bar{\theta}(W)$. Buja et al. (2016) perform this decomposition elegantly in the linear regression context, and similar developments would be useful for contexts where the conditional mean and conditional variance have a more complex relationship.

Secondly, and more importantly, investigators should explore non-uniform subsampling and resampling schemes to test the stability of the effective estimand. As opposed to more traditional applications of these perturbation methods that are meant to simulate identical replications of the observed data Y_W, the purpose of these tests is to identify how the effective estimand $\bar{\theta}(W)$ potentially varies across non-identical contexts. Deliberate splits of the data according to experimental design principles that maximize the power of a test for misspecification along the lines of Box & Draper (1959) could be useful here. The analysis in Example 15 and Figure 6, where the data were split across metropolitan areas of different size and estimates were compared across these subsets, is an example of an informal implementation of this approach.

8.2 Extensions

The theory presented here can be extended in numerous ways, but we highlight one particular extension here. The requirement of strict invariance in the effective estimand in order to be a valid basis of generalization, implied by Definition 2, can be loosened to incorporate, for example, a comparison between the variation of the effective estimand $\bar{\theta}(W)$ across contexts in the range of validity $\mathcal{W}$ and the variation of $\hat{\theta}(Y_W, W)$ across replications. In this light, Definition 2 is similar to a requirement for unbiasedness; an analogue to the bias-variance tradeoff could therefore be a useful notion.

For example, shrinkage estimators (James & Stein, 1961; Efron & Morris, 1975) deliberately incorporate an aspect of the context W, namely the sample size N_W, in determining how to trade off bias and variance. The resulting effective estimand does not satisfy Definition 2, but the procedure improves total mean-squared prediction error. This being said, the shrinkage scheme can also lead to suspect conclusions when, say, an investigator wishes to compare the means of groups of significantly different size. In these cases, the effective estimand can be a useful tool for understanding the influence of a particular shrinkage scheme on substantive conclusions, but the overly strict criterion in Definition 2 will need to be replaced. A similar analysis could be done to characterize the influence of prior distributions in Bayesian data analysis.

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