Introduction

Efficient analysis with biased samples

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Biased sampling can be found in most data collection processes and is quite common in medical studies. It may arise by design for example, oversampling specific subgroups in order to gain efficiency or inadvertently if someone refuses to respond. The best-known example occurs in a simple case-control study. The disease status is obtained from a random population, and potential covariates are observed for independent samples taken from each group (case or control).

Note that the sampling is stratified on the response variable. The likelihood depends on \( g(x) \), the distribution of the covariate \( x \), which is of no interest and usually too complicated for modeling. On the other hand, if the sampling were performed unconditionally or conditionally on \( x \), the likelihood would be given by

\[
L(\theta) = \prod_{i=1}^{N} f(y_i, x_i | \theta) \pi(x_i) \]

where, \( f(y|x; \theta) \) is the regression model. Note that it is independent of \( g(x) \) and so maximization is straightforward.

Anderson [1] and Prentice and Pike [4] have shown that for the binary logistic regression model with an intercept, maximum likelihood estimates from all regression coefficients except for the constant term can be obtained by ignoring the case-control scheme; i.e., the case-control problem can be treated as a prospective one.

Scott and Wild [6] have shown how to adjust the intercept. They have also extended that the second phase. The likelihood can be written as

\[
L(\theta) = \prod_{i=1}^{N} f(y_i, x_i | \theta) \pi(x_i) \]

The most basic approach is to ignore the sampling scheme and consider only the complete-observed units. In general, this will not lead to an efficient analysis of the data. With the purpose of improving it, several methods have been proposed.

Weighted likelihood

The weighted approach considers only the observed data, ignoring the incomplete ones. Here, each unit is weighted by the inverse of its probability of being selected for full observation. The likelihood is

\[
L(\theta) = \prod_{i=1}^{N} \frac{f(y_i, x_i | \theta)}{\pi(x_i)} \pi(x_i) \]

It is known to be robust, but inefficient.

Conditional likelihood

Conditional likelihood is an alternative approach used to increase efficiency. Lawless et al. [2] have shown that this approach is more efficient than the weighted method for different situations. The likelihood is given by

\[
L(\theta, G) = \prod_{i=1}^{N} f(y_i, x_i | \theta) \pi(x_i) \]

It leads to a dependency on \( G \), because

\[
P(R_i = 1) = \frac{\prod_{i=1}^{N} f(y_i, x_i | \theta)}{\prod_{i=1}^{N} \pi(x_i)} \]

In order to avoid this dependency we can condition the likelihood on \( x \) as well

\[
L(\theta) = \prod_{i=1}^{N} f(y_i, x_i | \theta) \pi(x_i) \]

and maximization is now straightforward. Note that both methods depend on \( G \). Lee et al. [3] have shown that by estimating that probability, there will be a gain in efficiency even if it is known.

Maximum likelihood method

The most efficient methods can be found here. They use complete likelihood to make inferences on \( y \), achieving full efficiency in special cases. The most basic methods are the estimated likelihood and the EM algorithm.

- **EM algorithm**: Bolly and Pepi [5] applied the EM algorithm to maximize the likelihood (2). Although it uses the complete-data set, the mean score, as it is called, can be shown to be related to the weighted method.

- **Estimated likelihood**: Since \( G_i(x) = P(y_i = x | x_i) \) for complete data, we can maximize (2) by replacing \( G_i(x) \) with \( \hat{G}_i(x) \), where

\[
\hat{G}_i(x) = \frac{N_i}{N} \hat{G}(x), \quad \text{where } \hat{G}(x) \text{ is the ECDF.} \]

Wasser and Zhou have also used \( \hat{G}_i(x) \), but for continuous \( y \) with no stratum information for the missing data.

Full efficient estimators can be obtained by profiling the full likelihood \( L(\theta) \) for \( \theta \) and distributions of \( G \). However, Zeng and Rockette [9] suggested working with a simpler maximization that is asymptotically equivalent. Instead of using the global MLE, full likelihood can be maximized under the restriction that \( G \) is supported by the observed values of \( x \).

Fully efficient methods have been developed for the following special cases:

- The multivariate case-control problem for discrete \( y \) and \( x \): an algorithm for obtaining the MLE was developed by Scott and Wild [7] for the multivariate case-control problem for discrete \( y \) and \( x \).

- Song et al. [8] obtained fully efficient estimator with a continuous response, but discrete covariates.

- Zhou et al. [9] worked with three distinct problems: a MAR, y MAR and both variables MAR, when both variables are discrete.

We are interested in the following objectives:

- To unify the work that has been done on conditional likelihood.
- To develop asymptotic expressions for the efficiency of weighted, conditional and full likelihood methods.
- To describe situations where the conditional approach is substantially better than weighting, when both methods achieve full efficiency and when the loss is negligible.

References