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## One-sample location tests for multilevel data

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#### Abstract

In this paper, we consider testing the location parameter with multilevel (or hierarchical) data. A general family of weighted test statistics is introduced. This family includes extensions to the case of multilevel data of familiar procedures like the *t*, the sign and the Wilcoxon signed-rank tests. Under mild assumptions, the test statistics have a null limiting normal distribution which facilitates their use. An investigation of the relative merits of selected members of the family of tests is achieved theoretically by deriving their asymptotic relative efficiency (ARE) and empirically via a simulation study. It is shown that the performance of a test depends on the clusters configurations and on the intracluster correlations. Explicit formulas for optimal weights and a discussion of the impact of omitting a level are provided for 2 and 3-level data. It is shown that using appropriate weights can greatly improve the performance of the tests. Finally, the use of the new tests is illustrated with a real data example.

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#### 1. Introduction

#### 1.1. Motivation and previous work

Situations involving cluster correlated data are often encountered in practice. Typical examples of clusters are a family, a litter, a laboratory, and a region or a strata (in survey samples). It is usually assumed that observations are possibly correlated within clusters and independent across clusters. A single subject with repeated measurements over time can also be viewed as a cluster and this case is usually referred to as longitudinal data. As such, longitudinal data are a special case of clustered data. An example of 2-level data would be students clustered within schools where the students would be the level 1 units and the schools would be the level 2 units. In that case, it could be reasonable to assume that the measurements on students from different schools are independent while the measurements on students from the same school are possibly correlated. When many levels of hierarchy are present, the terms *multilevel* of *hierarchical* data are commonly used. Adding the class level to the example provides 3-level data. This time students represent the level 1 units, the classes (within a school) are the level 2 units and the schools are the level 3 units. Measurements would again be assumed independent if coming from students from different schools but possibly correlated otherwise. Moreover, the correlation itself could be different whether the students are within the same class or in different classes

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within the same school. Once we are aware of that possibility, we can easily imagine situations involving more and more levels but there are rarely more than four levels of hierarchy typically in practice. Some monographs are entirely devoted to models for multilevel data, Goldstein (2003), Raudenbush and Bryk (2002) and Kreft and de Leeuw (1998) and other monographs on longitudinal data analysis also discuss about them; Fitzmaurice et al. (2004), Hedeker and Gibbons (2006), Singer and Willett (2003) and Aerts et al. (2002).

Likelihood-based inference for models involving multilevel data is fairly well developed both for continuous and discrete responses and the users can rely on general purposes softwares (like SAS and SPSS) that can usually handle only two levels of hierarchy or on more specialized softwares (like MLwiN or HLM) that can handle more levels of hierarchy. However, the literature on nonparametric (rank- and sign-based) methods for such data is very sparse and treats only models with two levels of hierarchy. For the two-sample problem with univariate data, Rosner and Grove (1999), Rosner et al. (2003) and Datta and Satten (2005) have proposed generalizations of the Wilcoxon rank sum test for 2-level data. For the one-sample problem with univariate data, Larocque (2005) and Rosner et al. (2006) have extended the Wilcoxon signed-rank test to the case of 2-level data while Gerard and Schucany (2007) proposed a sign test for dependent binary data. Still for the one-sample problem but more generally for multivariate data, Larocque (2003), Larocque et al. (2007) and Nevalainen et al. (2007a,b) have studied weighted multivariate spatial sign tests and weighted multivariate spatial medians. So far, the literature on nonparametric methods has been silent for situations involving more than two levels of hierarchy. This paper aims at filling this gap and one of its goal is to introduce a new direction for research on nonparametric methods or on multilevel data depending on the point of view. The univariate one-sample case testing problem with multilevel data is the focus of this first effort and more precisely, general weighted test statistics will be introduced and studied with an emphasis on the *t*, sign and Wilcoxon signed-rank tests.

#### 1.2. Model description

The general multilevel (K-level) location model considered is

$$Y_{i_1 i_2 \cdots i_K} = \theta + \varepsilon_{i_1 i_2 \cdots i_K},$$

$$i_1 = 1, \dots, m_{(1) i_2 i_3 \cdots i_K}, \quad i_2 = 1, \dots, m_{(2) i_3 i_4 \cdots i_K}, \dots,$$

$$i_{K-1} = 1, \dots, m_{(K-1) i_K}, \quad i_K = 1, \dots, n,$$
(1)

where  $\theta$  is the fixed location parameter and where the  $\varepsilon$ 's are identically distributed random errors. The total sample size is denoted by N. Level 1, with the index  $i_1$ , denotes the deepest level (usually the individual) while level K, with the index  $i_K$ , denotes the outermost level. Pursuing the example above with three levels, level 1 is the student level, level 2 is the class level and level 3 is the school level. We are assuming independence between observations from different K-level clusters. Observations from a same K-level cluster are possibly correlated.

We wish to confront the hypotheses

$$H_0: \theta = 0 \text{ and } H_1: \theta \neq (>, <)0.$$
 (2)

This problem may arise in the context of pre-test post-test or matched-pairs designs with multilevel data. Section 6 will present a real data example where measurements of cardial output (CO) for subjects that are in a horizontal or an upright position are compared.

Although the use of multiple indexing as in (1) can be practical when the number of levels is small (say 2 or 3), it is clearly too burdensome in the general case and this is why we will avoid it by introducing the following notation where each observation is identified by a single index.

For  $a=1,\ldots,N$ , let  $Y_a$  be the ath observation. Let d(a,b) ( $\in \{0,1,\ldots,K\}$ ) give the closeness of individuals a and b in the hierarchy (i.e., the number of level(s) they have in common). If individuals a and b are in different K-level clusters, then d(a,b)=0. At the other extreme, d(a,a)=K. Continuing the example above with three levels, d(a,b)=0 if the students are in different schools, 1 if they are in the same school but in different classes, 2 if they are in the same school and in the same class, and 3 if a=b (i.e., they are the same student). Note that the  $N\times N$  symmetric matrix with elements d(i,j) yields a complete description of the hierarchy.

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